THE COALITION FOR HEMOPHILIA B

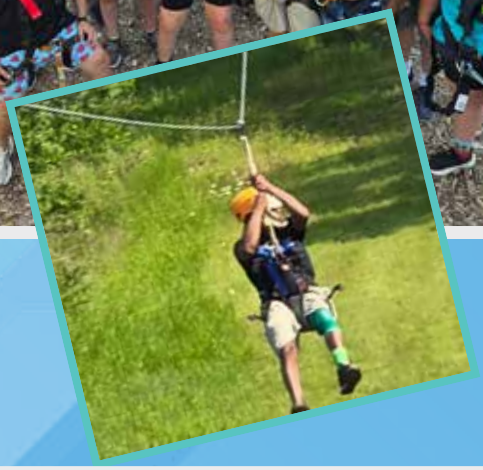
HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

SUMMER 2025

BEATS MUSIC PROGRAM

MEN'S EDUCATION & EMPOWERMENT PROGRAM



WOMEN'S EDUCATION & EMPOWERMENT 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.

BY RENAE BAKER

MUSIC: A HEALING ART

THE BEATS MUSIC PROGRAM HOLDS ITS SEVENTH ANNUAL SESSION

"The BEATS sets the stage for you to build your courage and confidence. What you see is some of the most courageous people, young and old alike—getting up and performing for the love of it, with no pretense of needing to be perfect." — Mike, returning parent participant

If you've never experienced the BEATS Music Program, imagine a weekend filled with laughter, rhythm, and connection. Now in its seventh year, this unforgettable event—created and hosted by The Coalition for Hemophilia B (CHB) brings together singers, musicians, and families from across the hemophilia B community to celebrate the power of music as a healing art. This year's BEATS was held July 16–20 in Nashville, Tennessee, setting the perfect backdrop for another inspiring chapter in this one-of-a-kind program.

The BEATS program was the vision of Kim Phelan, CEO of The Coalition for Hemophilia B, and Wayne Cook, CHB President. After speaking with parents who said, "Our kids aren't into sports, what else can they do?" Kim and Wayne realized there was a need for something more. They wanted to create a program that gave musically inclined children and adults a place to shine, connect, and express themselves creatively. That spark of an idea grew into BEATS, now one of the most beloved and inspiring programs in the bleeding disorders community.

Each year, BEATS welcomes participants of all ages and skill levels, from first-time singers to seasoned performers. Everyone learns, grows, and finds their voice in an atmosphere that's supportive, creative, and full of heart. "The BEATS keeps getting better and better," said Adam Smith, a professional music producer and longtime instructor. "You can actually see people's confidence grow from the first day to the final concert. It's amazing."

This year's theme song, "Keep Your Head Up," was reimagined with a reggae twist and recorded by participants in a professional Nashville studio. For many, stepping up to the microphone was both exciting and nerve-wracking. "It takes real courage to record your voice or instrument," said Tara Smith, a longtime caregiver and volunteer who helps coordinate












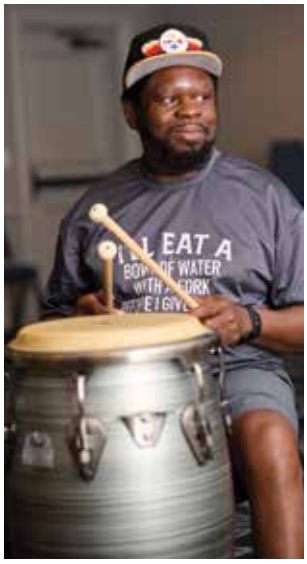
EAT A
WATER






the studio sessions. “But seeing everyone push past the nerves and light up when they realize what they can do, that’s the magic of BEATS.”

Between studio time and rehearsals, the weekend pulsed with creativity. Mornings kicked off with energetic icebreakers led by Rocky Williams and Erica Garber, setting a positive tone for the day. The hotel buzzed with the sounds of laughter, impromptu jam sessions, and shared encouragement proof that when people come together through music, something special happens.

A BEATS favorite, Elec Simon, returned with his high-energy bucket drumming jam, joined by Ben Satterlee on the drum kit. Within minutes, the room was filled with rhythm and excitement as everyone joined in, showing once again how music has the power to unite people from every background and experience level.

Throughout the weekend, participants worked closely with talented instructors including Renae Baker, Joe Turley, Rich Adams, Tiger Fitzhugh, Hope Hamby, Sam Blizzard, and A.J. Huang, who covered everything from vocals and guitar to piano, strings, and drums. “I love what I’ve learned here, the techniques, the people, the atmosphere, it’s all amazing,” said Keith, a first-time participant.

Guest presenters brought an extra layer of inspiration. Musician and advocate Max Feinstein spoke about how songwriting became a tool for healing, describing music as “a way to process pain, channel joy, and rediscover yourself.” Later, Nashville singer-songwriter Trevor Martin performed his original songs and shared behind-the-scenes stories of his creative journey, reminding everyone that being vulnerable through art is one of the greatest strengths we have.

Another highlight was *The Beat of Life* session led by CHB members Rick Starks and Domenic Catrine, who shared their personal experiences living with Hemophilia B and how music has helped them cope and connect. “Watching shy kids discover their voice, and seeing that transformation, is the most rewarding experience ever,” said Rick. “It’s the greatest program in the community.”

The weekend reached its crescendo with the BEATS Finale Concert, where participants took the stage to perform for a live and live-streamed audience. This year’s MC, Renae Baker, kept the energy high and the crowd engaged as participants
















showcased everything they'd learned. Whether they sang, played guitar, or shared reflections, every person radiated pride and confidence. "You could feel the excitement in the room," said Leisa, a returning parent. "Watching everyone come alive through music, it's unforgettable."

To top it all off, attendees also visited the Grand Ole Opry, soaking up the spirit and sound of Nashville's legendary music scene. It was the perfect encore to a weekend filled with harmony, friendship, and joy.

From the first drumbeat to the last standing ovation, BEATS once again proved that music is a healing art, a universal language that connects, uplifts, and inspires. "I came because music is my passion," said Danny, a returning participant. "What I love best about BEATS is the community. I love every second of it."

Whether you sing, play, or simply love music, there's a place for you at BEATS. It's not about being perfect, it's about expressing yourself, finding courage, and discovering the beauty of what happens when a community comes together in song.

The BEATS program continues to thrive thanks to the generosity of its sponsors: CSL Behring and Novo Nordisk at Producer Level, Sanofi at Conductor Level, and CVS Specialty and Medexus Pharma at Performer Level. Their representatives didn't just attend—they participated, laughed, and joined the fun, helping make BEATS feel like one big, creative family.






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‡ The median AsBR for people who started on 7- or 14-day prophylaxis was 0. For people who switched to prophylaxis from on-demand, the median AsBR was 0.7. AsBR=annualized spontaneous bleed rate.

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FINDING MY RHYTHM: A FIRST-TIMER'S JOURNEY AT THE BEATS MUSIC PROGRAM

BY PAM WILLIAMS

I'd heard about the BEATS Music Program for years but never quite found the courage to apply. Every year, I'd think about it, and then talk myself out of it. What if I wasn't good enough? What if everyone else was more talented?

Then, at this year's Coalition for Hemophilia B Annual Symposium, I was chatting with my cousin, Ben, who mentioned he wanted to apply too, but didn't want to go alone. I laughed and said, "Okay, I'll apply, but no promises that we both get in!" It turns out we were both thinking the same thing. Even though we already knew many people in the Coalition community, BEATS was something new, something a little intimidating, a space that would challenge us to open up, take risks, and maybe rediscover parts of ourselves we'd forgotten.

A few weeks later, I opened my email and saw the word "Congratulations." I'd been accepted. I stared at the screen in disbelief. Fifteen years had passed since I last touched a piano. Singing? That hadn't really happened since high school and college, unless you count the occasional karaoke night. I texted Ben immediately, and when he said he'd been accepted too, it suddenly felt real. We were doing this.

Choosing the Song That Chose Me

When I found out the solo vocal group was already full, I had no choice but to join the piano group. That news sent a jolt of nerves through me. My first instinct was panic, but then I decided to face the challenge head-on. I picked a song that's always meant something special to me: "The Sound of Silence."

Back in my hometown of Woodstock, Vermont, there was a legend that my high school's ancient history teacher, Mr. Robert Yoh, was actually the real author of the song's lyrics. According to him, he had submitted

the poem to a contest, only for it to later appear, slightly altered, under someone else's name. Whether the story was true or not, it was clear the loss hurt him deeply. When our concert choir performed the song one year, he had to leave the auditorium, it was just too painful. I chose the song in his memory, hoping I could do it justice.

Still, doubt crept in. Would I even remember how to play? Could I get my hands to cooperate after so long? I borrowed a songbook from the library and began practicing on a grand piano at my aunt's church. Slowly, the muscle memory returned, the notes, the flats, the feel of the keys. It really was like riding a bike. As the days counted down to Nashville, my excitement began to replace my fear.

First Day Jitters

When I arrived at BEATS, the butterflies came back in full force. I kept thinking, What if I freeze when I sit down at the piano? What if I forget everything?

Our piano group was a mix of talent and experience two accomplished pianists, one beginner, and me somewhere in between. Our instructor, Sam, was patient, encouraging, and endlessly kind. He had a gift for meeting each of us exactly where we were.

For the first time, I learned to play on an electric keyboard, something entirely new for me. While Sam coached one student, the rest of us practiced quietly on our own keyboards. Bit by bit, my confidence grew. Each of us supported one another, offering cheers, smiles, and little words of encouragement that made the room feel like home.

Lights, Camera, Studio!

On day two, our group visited the recording studio, where each instrument section recorded their part for the group song. The process was fascinating, and a little overwhelming at first. I remember sitting there like a deer in headlights, unsure of what to do. Thankfully, Adam, our music producer, quickly realized I needed a bit of guidance and came up with a simple right-hand chord pattern that I could play at intervals throughout the song.

What struck me most about BEATS was how inclusive it was. No matter your experience level, everyone was encouraged to participate in everything. It wasn't about perfection, it was about courage, growth, and joy.

The Magic of BEATS

The rest of the week was filled with a perfect mix of learning and laughter. We attended educational sessions, played games, and gathered at night for jam sessions that stretched late into the evening. Some nights, I'd sneak into the main room and quietly practice on the grand piano, hoping no one could hear me. But gradually, I stopped caring who might be listening. The more I played, the more I felt like myself again.

By the time Saturday rolled around, it was showtime. The BEATS concert was live-streamed, and I was listed as the third performer. My hands were shaking as I sat down at the piano, but once the music started, everything else faded away. I played like no one was watching.

Were there a few mistakes? Probably. But I didn't stop. When I finished, a wave of relief and pride washed over me. I had done it, I'd stepped way outside my comfort zone, and I had survived.

Lessons Beyond the Music

Looking back, BEATS was more than a music program. It was a reminder of what it means to be brave, to take risks, and to rediscover parts of yourself you'd let go of long ago.

What impressed me most was how supportive and genuine everyone was. Whether they were first-timers or returning participants, everyone encouraged one another. You could feel the sense of belonging in every session, every performance, every conversation in the hallway.

The beauty of BEATS is that it brings everyone in the hemophilia B community together through the shared language of music.

One of my favorite moments came when another participant told me, "Your piano group became so cohesive, it was obvious how much you all supported one another. It was so wonderful to watch." That comment meant everything. It summed up exactly what BEATS is about: connection, confidence, and community.

Looking Ahead

As I packed my bags to head home, I knew one thing for sure: this wouldn't be my last time at BEATS. I'm already planning to apply again next year, and this time, I'll be practicing well in advance!

BEATS reminded me that it's never too late to start again, to pick up an instrument, to sing, to express yourself, or to share your story. Music heals, connects, and empowers, and that's exactly what this program does for every person who walks through its doors.

For me, that one week in Nashville changed everything. What began as a leap of faith turned into one of the most rewarding experiences of my life.

I'm so grateful to The Coalition for Hemophilia B for creating this space, for believing in the power of music, and for giving people like me a place to rediscover their rhythm.

Because sometimes, all it takes is one brave "yes" to change your tune, and your life.

Stronger Together:
Men's Retreat 2025 Brings Brotherhood, Courage, Growth, and Renewal

BY MATT STONE

There's something special about summer, it invites us to slow down, reconnect, and spend time with people who truly understand us. For the men of the hemophilia B community, this summer brought an unforgettable opportunity to do just that.

From May 29 to June 1, men and fathers gathered at Post Oak Lodge in Tulsa, Oklahoma, for a weekend filled with wisdom, laughter, and genuine connection.

Hosted by The Coalition for Hemophilia B (CHB) and generously sponsored by Pfizer, this retreat offered more than just education, it offered belonging. It was a space to recharge, share experiences, and strengthen the brotherhood that defines our community.

A Warm Welcome and Powerful Conversations

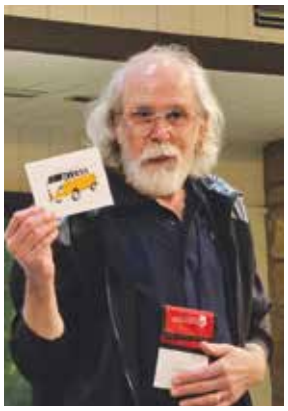
The weekend began with a warm welcome from the Coalition team, setting the tone for a retreat built on trust and camaraderie. Early icebreakers encouraged conversation and laughter, helping attendees bond quickly and find common ground.

Soon after, rap sessions, a CHB retreat favorite, brought authenticity and connection to the forefront. These small-group discussions gave men the chance to share personal stories about life with hemophilia B, fatherhood, and perseverance. What began as structured dialogue soon became heartfelt exchanges about resilience, relationships, and hope.

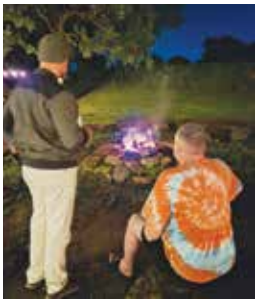
Learning, Leadership, and Shared Purpose

As the weekend unfolded, education and inspiration flowed side by side. Lee Hall shared how mentorship and storytelling helped him grow into his advocacy voice, inspiring others to see advocacy not just as a responsibility, but as a reflection of lived experience.









Taking Ownership of Your Health







for our community


Financial expert Donnie Akers offered clear and practical guidance on planning for the future, helping participants feel more confident about managing finances and preparing for life's unexpected turns. His approachable style made a complex topic easy to understand and empowering to act on.

Exploring the Future of Care

Education continued with Dr. David Clark, CHB's Chairman, who delivered a clear, engaging overview of the latest treatment advances and innovations shaping the future of hemophilia B care. His presentation helped participants better understand the evolving landscape of therapies, empowering them to have more informed discussions with their healthcare providers.

Pfizer also shared educational updates about ongoing developments in hemophilia B care, reinforcing the importance of staying informed and engaged in shared decision-making.

Facing Challenges and Embracing Growth

Following the educational sessions, Thomas from GutMonkey brought his trademark energy and insight to the "Leading Edge" program, also supported by Pfizer. Through interactive challenges and thoughtful reflection, participants explored what it means to take ownership of their health and life choices.

The program centered around three key themes: knowledge, curiosity, and advocacy. In the Be Brave Lab, attendees were reminded to choose their own challenges, stay present in the moment, and find joy in the process of growth. It was a powerful reminder that courage isn't about fearlessness—it's about showing up, again and again, with heart.

Teamwork, Trust, and Adventure

Team-building activities and outdoor challenges gave everyone a chance to connect beyond the classroom. The treetop zipline and aerial obstacle course became instant highlights, pushing participants to step outside their comfort zones while cheering each other on through every leap and climb.

These moments of shared laughter and encouragement deepened bonds and reminded everyone that strength doesn't come from standing alone, it's built together.

Fun, Friendship, and the Bleeder Olympics

The retreat's signature Bleeder Olympics brought out everyone's playful spirit, combining friendly competition
















with community pride. Human foosball and outdoor games kept the energy high, with plenty of laughs and lighthearted rivalry.

As the day wound down, campfires offered space for reflection and connection. Stories, laughter, and quiet moments of understanding filled the night air. For many, these conversations—real, raw, and supportive, were the heart of the weekend.

A Celebration of Culture and Connection

The final evening offered a stunning celebration of culture and unity. The Dancing Eagles brought Indigenous traditions to life through breathtaking music, storytelling, and movement. From the rhythmic Fancy Dance to the intricate Hoop Dance, each performance embodied resilience, spirit, and strength.

The night concluded with the Friendship Dance, where participants joined hands in a circle of connection. Accompanied by live flute music and traditional song, it was a moment of reflection and renewal, a celebration of community that transcended words.

Reflections Among the Pines

The mix of peaceful surroundings and adventure created a space where men could breathe deeply, think clearly, and rediscover balance.

By the weekend's close, what began as a group of individuals had become something greater, a community of brothers united by understanding, compassion, and purpose.

The Coalition for Hemophilia B extends heartfelt thanks to Pfizer for their generous support, which helped make this meaningful and memorable weekend possible.

Participant Reflections

"I like Coalition events because I'm able to have conversations about my son's condition in a forum where everybody understands, and nothing feels hidden."

"I loved getting to know the guys and being able to connect with them."

"It was great seeing old friends while making new ones. I loved learning about new treatments and hearing everyone's stories."

"There are no words to explain—it changed my life."

Final Thoughts

The Men's Retreat Summer 2025 was a powerful reminder of what happens when men come together with open hearts and shared purpose. It was about learning, challenging ourselves, laughing freely, and realizing that growth and healing are always stronger in community.

At its core, this retreat celebrated everything that makes our community extraordinary: the courage to grow, the wisdom to listen, and the connection that reminds us we are never alone.

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It is not known if HYMPAVZI is safe and effective in children younger than 12 years old.

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

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HYMPAVZI may cause serious side effects, including:

- **blood clots (thromboembolic events).** HYMPAVZI may increase the risk for your blood to clot. Blood clots may form in blood vessels in your arm, leg, lung, or head and can be life-threatening. Get medical help right away if you develop any of these signs or symptoms of blood clots: swelling or pain in arms or legs; redness or discoloration in your arms or legs; shortness of breath; pain in chest or upper back; fast heart rate; cough up blood; feel faint; headache; numbness in your face; eye pain or swelling; trouble seeing
- **allergic reactions.** Allergic reactions, including rash and itching have happened in people treated with HYMPAVZI. Stop using HYMPAVZI and get medical help right away if you develop any of the following symptoms of a severe allergic reaction: swelling of your face, lips, mouth, or tongue; trouble breathing; wheezing; dizziness or fainting; fast heartbeat or pounding in your chest; sweating

The most common side effects of HYMPAVZI are injection site reactions, headache, and itching.

These are not all the possible side effects of HYMPAVZI. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.

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What should I tell my healthcare provider before using HYMPAVZI?

Tell your healthcare provider about all your medical conditions, including if you:

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- have a severe short-term (acute) illness such as an infection or injury.
- are pregnant or plan to become pregnant. HYMPAVZI may harm your unborn baby.
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  - You should use effective birth control (contraception) during treatment with HYMPAVZI and for 2 months after the last dose of HYMPAVZI.
  - Tell your healthcare provider right away if you become pregnant or think that you may be pregnant during treatment with HYMPAVZI.
- are breastfeeding or plan to breastfeed. It is not known if HYMPAVZI passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, and herbal supplements.

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See the detailed "Instructions for Use" that comes with your HYMPAVZI for information on how to inject a dose of HYMPAVZI, and how to properly throw away (dispose of) used HYMPAVZI prefilled syringe or HYMPAVZI prefilled pen.

- Use HYMPAVZI exactly as prescribed by your healthcare provider.
- Your healthcare provider will provide information on the treatment of breakthrough bleeding during your treatment with HYMPAVZI. **Do not** use HYMPAVZI to treat breakthrough bleeding.

What warnings should I know about HYMPAVZI?

HYMPAVZI may cause serious side effects, including:

- **blood clots (thromboembolic events).** HYMPAVZI may increase the risk for your blood to clot in blood vessels in your arm, leg, lung, or head and can be life-threatening. Get medical help right away if you develop any of these signs or symptoms of blood clots:
  - swelling or pain in arms or legs
  - redness or discoloration in your arms or legs
  - shortness of breath
  - pain in chest or upper back
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing
- **allergic reactions.** Allergic reactions, including rash and itching have happened in people treated with HYMPAVZI. Stop using HYMPAVZI and get medical help right away if you develop any of the following symptoms of a severe allergic reaction:
  - swelling of your face, lips, mouth, or tongue
  - trouble breathing
  - wheezing
  - dizziness or fainting
  - fast heartbeat or pounding in your chest
  - sweating

The most common side effects of HYMPAVZI are injection site reactions, including:

- itching
- swelling
- hardening
- redness
- bruising
- pain

Headache and itching were also common side effects. A serious side effect of swelling in the legs happened in one patient in the clinical trial.

These are not all of the possible side effects of HYMPAVZI. Call your doctor for medical advice about side effects. For more information, ask your doctor.

This information is not comprehensive. How to get more information:

- Talk to your health care provider or pharmacist
- Visit www.HYMPAVZI.com to obtain the FDA-approved product labeling
- Call 1-888-HYMPAV-Z

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

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HEALING THROUGH LEARNING, LAUGHTER, AND SHARED STRENGTH – WOMEN'S EMPOWERMENT IN ACTION RETREAT 2025

BY ERICA GARBER

Amid gentle lakeside breezes and the whispering pines surrounding the Atlanta Evergreen Resort, a deep sense of calm settled over the group of women who came together for the 2025 Women's Empowerment in Action Retreat, held June 26–29, 2025.

The weekend was a celebration of joy, reflection, and renewal, drawing women from across the hemophilia B community for a few precious days devoted to connection and self-care.

What unfolded was more than a weekend retreat, it was a collective exhale, a moment to release burdens, reclaim energy, and rediscover strength in shared experience. Participants described it best: "It wasn't just a program. It felt like coming home."

A Warm Welcome and a Shared Beginning

From the very first moments, the stress of daily life began to melt away. Laughter echoed through the lobby as women reconnected with old friends and greeted new faces. Gentle massages, shared meals, and spontaneous conversations set the tone for the weekend ahead.

CHB Executive Director, Erica Garber, opened the retreat with heartfelt remarks, inviting everyone to "say yes to themselves", to be fully present, to breathe, and to allow joy back in. Her message resonated: "You do so much for others every day. This weekend is for you."

What began as polite introductions quickly became heartfelt conversations. In the opening icebreaker, Erica guided participants beyond small talk into meaningful dialogue. Laughter mixed with tears as stories unfolded, stories of resilience, caregiving, motherhood, and advocacy. By the end of the session, strangers had become sisters.

The Art of Gratitude and Creative Healing

That spirit of connection flowed into *The Art of Gratitude*, led by Ashley Smith, Sanofi's Community Relations and Education Manager. Blending creativity with emotional wellness, Ashley encouraged participants to express gratitude through painting. Each woman created a canvas symbolizing what she cherished most, family, freedom, faith, or healing and in doing so, honored the quiet resilience they carry every day.

Many returned to their paintings throughout the weekend, adding colors and symbols as new insights emerged. The canvases became living reflections of gratitude and growth, a reminder that healing can be both a process and a masterpiece in progress.

















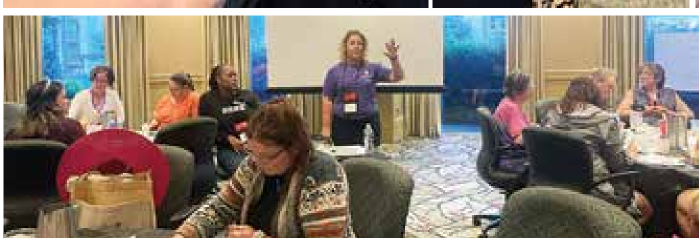


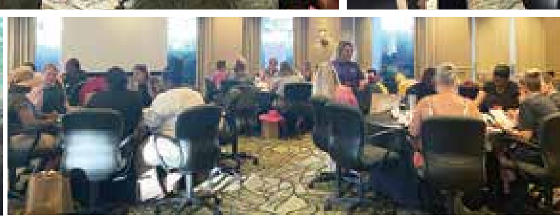



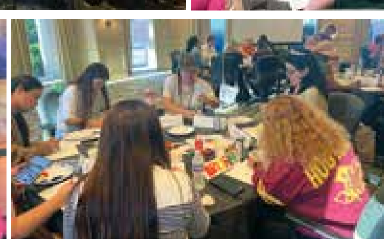
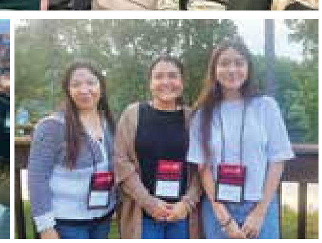



Rhinestones, Laughter, and the Power of Play

Not all healing happens in silence. Sometimes, it bursts forth through laughter and movement. Erica led the now-legendary *Rhinestone Relay*, a playful team challenge filled with sparkle, cheering, and joyful chaos. Women teamed up to complete creative tasks that brought out their inner child, and their unstoppable competitive spirit!

The laughter was contagious. "We laughed so hard our faces hurt," one participant shared. "It reminded us that healing lives in joy, too." The activity captured the weekend's theme perfectly, when women come together, strength multiplies, and the sparkle of community shines brightest.

Finding Courage in Nature

Early Friday morning, the group ventured outdoors for an excursion to Stone Mountain Park. Under the open sky, women climbed, hiked, and reflected. Some took to the SkyHike ropes course, pushing past fear and rediscovering confidence, while others enjoyed quiet walks and lakeside journaling.

Nature became both a teacher and a companion reminding participants that healing is not linear, but alive and ever-changing. The stillness of the morning made way for clarity and courage, fueling deeper conversations in the sessions that followed.

Real Stories, Real Strength

That afternoon, two roundtable discussions created space for shared truth and lived experience.

In "Women Bleeders Roundtable," Jennifer DeGlopper and Nelly Miranda guided an honest dialogue about misdiagnosis, delayed care, and the unique challenges women face in getting their voices heard. Attendees found both validation and solidarity, realizing they were far from alone in their experiences.

Meanwhile, caregivers gathered for *Holding Space for Caregivers*, led by Tara Blakely and Laura Echandi. This session was filled with compassion and laughter as women shared the realities of caring for loved ones with hemophilia B, the exhaustion, the fear, and the fierce love that keeps them going. One caregiver summed it up beautifully: "We hold so much for others. Today, we got to be held."

Family, Communication, and Everyday Advocacy

The next session, *Family Connection*, led by Linda Pollhammer,RN, Pfizer Hemophilia Patient Navigator, turned attention toward relationships and communication. Using real-world scenarios and role-play, Linda helped participants explore how to talk about bleeding disorders at home, at work, and in medical settings while protecting personal energy and privacy.

Her interactive approach encouraged women to find their voice and to name what they need — from clearer communication with doctors to more understanding from family members. The takeaway was powerful: advocacy begins with honesty and boundaries.

Chit-Chat, Chocolate, and Compassion

As the sun set, women gathered for *Chit-Chat and Chocolate*, a session led by Karen Boyd, LMSW, that blended sweetness with sincerity. The room glowed with soft laughter, heartfelt stories, and the comforting aroma of cocoa. It was a space where tears and joy coexisted freely a space where every woman could simply be.

By night's end, the walls between participants had dissolved completely. As one attendee shared, "This was the first time I've talked openly about my fears and I didn't feel judged. I felt understood."


Walking Toward Renewal

Saturday morning began with a lakeside hike led by CHB's Patient Liaison, Farrah Muratovic. The forested paths offered quiet reflection and gentle movement. Surrounded by nature, women shared silent gratitude and

newfound peace.

Back indoors, the sessions turned inward. In *Relationships 101*, emotional wellness coach Latisha Russell guided participants to examine boundaries, resilience, and the stories we tell ourselves about love and worth. Her empowering reminder, "You are the author of your own story" — resonated deeply, inspiring many to recommit to self-trust and self-care.

Later, Lee Hall returned with *Telling Your Story*, an advocacy workshop that helped participants shape their experiences into powerful narratives. Whether speaking to policymakers or simply sharing with a friend, women learned how their voices can build bridges of understanding and spark lasting change.

What We Carry, What We Release

One of the most moving moments of the weekend came in the session *What We Carry*, co-led by Karen Boyd, LMSW, and Dave Rushlow, LMSW. In a circle of honesty, women shared the invisible weights they bear from caregiving and fatigue to self-doubt and fear of the future.

Each voice added a thread to the collective story of strength. Some cried softly; others simply nodded, understanding without words. By the end, something had shifted. The heaviness had lifted, replaced by a shared lightness proof that healing happens in community.

The session flowed naturally into the weekend's closing reflection, *The Anatomy of Hope and Connection*. Erica invited participants to reflect on the body as a metaphor for resilience, hands for what we hold, hearts for what we long for, feet for the paths ahead. The stories shared in that closing circle will echo for years to come, carried forward through new friendships and renewed purpose.

Sparkle and Sass: Celebrating Sisterhood

The final night was pure joy. The celebration, *Sparkle and Sass: A Night of Country Queens*, paid tribute to fierce friendship and fearless fun. Inspired by Dolly Parton and other trailblazing women, participants arrived dressed in fringe, glitter, sequins, and rhinestones, the brighter, the better!

Laughter filled the air as the group danced, sang, and celebrated everything they had experienced together. From soulful karaoke ballads to line dancing under twinkling lights, the evening captured the retreat's spirit perfectly: strong, joyful, unapologetic women shining together.

As one participant said, "I came here feeling tired. I'm leaving with light in my heart."

A Weekend That Lasts Beyond Goodbye

When Sunday morning came, goodbyes were bittersweet. Hugs lingered, and promises to stay connected filled the room. Many participants reflected on how the weekend had changed them, not through lectures or schedules, but through the simple power of presence.

"This retreat reminded me that I'm not alone. I have a whole network of women who understand exactly what I'm going through."

"After attending these sessions, I feel renewed and ready to face what comes next."

"It was life-changing. I will forever be grateful to The Coalition for Hemophilia B for creating this space."

The Coalition for Hemophilia B extends heartfelt thanks to Pfizer and Sanofi for their generous support in making this unforgettable retreat possible. Their partnership ensures that women affected by hemophilia B have access to programs that nurture the mind, body, and spirit empowering them to lead with strength, laughter, and compassion.

The 2025 Women's Empowerment in Action Retreat was a reminder that healing takes many forms: sometimes through learning, sometimes through laughter, and always through connection. Together, we are redefining what empowerment looks like, one story, one smile, and one shared moment at a time.

Pfizer sanofi

HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

US Physicians' Satisfaction with Current Treatments and Joint Health

6/23/25 Novo Nordisk and Adelphi Real World surveyed US hematologists who treat five or more hemophilia patients per month to determine their (the physicians) satisfaction with current prophylactic treatments and their patients' joint health. They received responses from 63 physicians who provided information on 355 patients with hemophilia A and 66 patients with hemophilia B.

For hemophilia B, as for hemophilia A, the results were underwhelming. Overall, only 37.9% of physicians had complete satisfaction with their patients' current treatments. They further broke down the results by product type (that is, standard half-life (SHL) and extended half-life (EHL) products) and for patients with or without inhibitors. All of the results were still in the 30–40% range, except for inhibitor patients, for whom the physicians had a satisfaction rating of 42.9%. The top two reasons physicians were not satisfied with the products available for their hemophilia B patients were the lack of availability of more efficacious products and the lack of effectiveness for specific bleed types.

There is always the question of whether the lack of good treatment outcomes is because of the products themselves or because of a lack of adherence by patients. Adherence questions are difficult to answer, but the physicians believed that 66.7% of their patients on SHL products and 70.4% on EHL products were compliant. Compliance is defined here as taking more than 80% of the prescribed dose.

The physicians also reported that 27.3% of their hemophilia B patients have joint problems, and that of those patients 72.2% have target joints and 76.5% experienced joint pain. Thus, in spite of all the products available, there still seems to be a long way to go. Flipping the results over suggests that 60–70% of physicians are not satisfied with current treatments. The authors conclude: "This highlights that patients with hemophilia may still need to make compromises in their current hemophilia management." [ISTH abstract PB1485]

Different Immune Responses in Hemophilia A and B Inhibitor Patients

7/31/25 Because of the relatively small number of hemophilia B patients and the similarities between the disorders, information from studies of hemophilia A is often assumed to also apply to hemophilia B. However, one place that doesn't work is in treatment of patients who develop inhibitors. Far fewer hemophilia B patients develop inhibitors (typically 5% or fewer) than do patients with hemophilia A (about 30%).

While inhibitors are less frequent in hemophilia B patients, the symptoms can be worse. Bs with inhibitors can also develop allergic reactions to factor IX and have anaphylactic reactions, severe allergic reactions that can be fatal. Bs with inhibitors can also develop a kidney disorder called nephrotic syndrome. Immune tolerance induction (ITI) can eliminate inhibitors for about 70% of As, but only works in less than about 30% of Bs.

Researchers have puzzled about these differences for several decades, but now a Chinese group has shown that there are actually different types of immune responses involved in inhibitor development for As and Bs. The researchers sequenced the genomes of individual peripheral blood mononuclear cells (PBMCs) from hemophilia A and B inhibitor patients. PBMCs are a group of white blood cells, part of the immune system, that includes B cells, T cells, and monocytes. T cells of various types can kill individual cells that are infected or damaged. They also work with B cells to produce antibodies.

The results show that hemophilia A patients with inhibitors have a higher proportion of B cells and more active B cells. Those B cells produce the antibodies that bind to factor VIII and inhibit its action. In contrast, hemophilia B patients with inhibitors have a higher proportion of T cells and more active helper-T cells. This difference helps explain some of the characteristics seen in A and B inhibitor patients.

That B cells are more involved in hemophilia A inhibitors may be the reason that the drug rituximab, which suppresses B cells, is more effective in hemophilia A inhibitor patients, and also the reason that ITI works better. In hemophilia B inhibitor patients, who have more T cell involvement, even when inhibitor

activity has been temporarily reduced by rituximab and ITI, memory T cells can continue to stimulate newly generated B cells to resume production of the inhibitor antibodies.

There is much more that needs to be learned. The immune system is amazingly complex, but each little step, each new piece of information, helps us put together a picture of how it works. [Li E et al., Blood Adv, online ahead of print 7/31/25]

Osteopenia and Osteoporosis in Hemophilia

6/22/25 It is well known that people with hemophilia are more likely to have poor bone health compared to people without hemophilia. Researchers in Europe looked at 130 patients with severe hemophilia A and 94 patients with severe hemophilia B along with an equal-size control group of people with similar age and body mass index (BMI). They measured bone mineral density (BMD) by DEXA scans, dividing the participants into three sub-groups: 1) those with normal BMD, 2) those with osteopenia, a somewhat lower than normal BMD, and 3) those with osteoporosis, a much lower than normal BMD.

The worst case was in patients not on prophylaxis who had a 2.20 times greater prevalence of osteopenia and a 13.8 times greater prevalence of osteoporosis. The best case was in patients on prophylaxis who started treatment at age 10 or younger. They had a 1.8 times greater prevalence of osteopenia, but only 0.22 times the prevalence of osteoporosis. That suggests that they have a 5 times lower risk of osteoporosis than people in the general population.

They also looked at patients who started prophylaxis later in life. Those who started between ages 11 and 20 had a prevalence of osteoporosis 1.5 times higher than the patients who started earlier, and those who started after age 20 had a 3.75 times higher prevalence. This is more evidence that prophylaxis, started early in life, is very beneficial for hemophilia treatment. The cause of BMD decline in hemophilia is controversial. It may have a biochemical cause due to the lack of factors VIII or IX; it may be due to reduced mobility and sedentary lifestyles; or it might even be due to HIV infection. [ISTH abstract PB1481]

6/22/25 A group from Austria has done some studies to try to understand the effects of the various clotting factors on bone metabolism. Like many things in the body, bone is constantly being made and destroyed at the same time. This helps to ensure that everything in the body remains fresh and in good working order. There are two main types of cells that do this work. Osteoblasts form bone and osteoclasts destroy bone. The researchers looked at the effects of a number of clotting factors on both types of cells in the laboratory. They found that factor X reduced mineralization (bone formation) in osteoblasts, an effect that was not seen with activated factor X. Factor X is activated in the coagulation cascade by factors VIII and IX, so a deficiency of activated factor X caused by hemophilia could lead to reduced bone formation. This is new information. Scientists have been trying to determine whether bone health is affected by individual clotting factors or just by some of the products of the clotting cascade, especially thrombin, which is activated factor II. These results suggest that activated factor X may also have an effect. [ISTH abstract PB1367]

Sarcopenia in Hemophilia

4/24/25 Just as osteopenia is a loss of bone mass, sarcopenia is a loss of muscle mass. This is another symptom of hemophilia that may be due to reduced mobility and sedentary lifestyles, although a biochemical cause hasn't been ruled out. A group in Japan looked at 66 patients (58 As and 8 Bs) over 40 years of age. They found severe sarcopenia in 15.1% of the group. Compared to another study looking at sarcopenia in Asian men without hemophilia, the group tended to have a younger age of incidence of sarcopenia. [WFH 2025 Critical Care Summit abstract PP-084]

Intracranial Hemorrhage in Hemophilia

6/23/25 Intracranial Hemorrhage (ICH), commonly called a "brain bleed," is a severe, debilitating, and potentially fatal complication in hemophilia, yet it hasn't been studied much. An Italian group surveyed hemophilia A and B patients from a number of treatment centers for ICH over the 10-year period from 2012–2022. They found 26 hemophilia A patients and 6 Bs who had suffered ICH in that period, 11 milds and 21 severes. Three-quarters of the patients were adults, with an average age of 45.5 years.

The most important finding is that patients on prophylaxis had a significantly lower incidence of ICH, 18.8% for the prophy patients compared with 81.2% for those treated on-demand. That's a sobering statistic about the importance of prophylaxis. High blood pressure, a risk factor for ICH, was present in 14 of the 24 adult patients, and especially in 90% of the mild patients. Both severe and mild patients had a high survival probability of over 75% up to 5 years after ICH. There was no significant difference in ICH incidence between those with hemophilia A or B. [ISTH abstract PB0267]

Hospitalization Outcomes in Hemophilia A and B

6/23/25 A group of researchers looked at hospitalization outcomes in patients with hemophilia A or B across the entire US in 2021. This included 7530 hospitalizations for hemophilia A patients and 1745 for Bs. The Bs had a median age of 44.9 years and were 80.8% male. The mortality (death) rate was 2.66% for the As and 2.01% for the Bs, with an odds ratio suggesting that the As had a 1.35 times higher risk of death than Bs. The average length of stay in the hospital was significantly longer for the As, at 6.3 days than for the Bs at 5.3 days. In spite of the shorter hospitalizations, the average total hospitalization charges were higher for Bs at $196,315, than for the As at $162,567. The data also allowed them to look at racial and economic disparities. Low-income patients had higher mortality rates and longer hospital stays. Black and Hispanic patients had significantly higher hospitalization costs, $198,750 versus $165,300. [ISTH abstract PB0273]

New Estimate of De Novo Cases of Hemophilia

4/24/25 It is usually estimated that about 30–40% of hemophilia cases (A and B) are new (de novo) mutations. That is, the mother is not a carrier and there is no family history of hemophilia. That may be an overestimate based on people not knowing whether their ancestors had bleeding issues. A group in India did a genetic study that should give a more precise answer to the question. Out of 617 hemophilia patients, they found 104 (16.9%) who had no family history of hemophilia—or so they thought. The researchers did genetic testing on all of those patients and came up with some surprising results.

Out of the 104, it turned out that 88 (85%) had mothers who did have hemophilia gene mutations, and thus were carriers, but they didn't know it. Only 16 patients (2.6%) had mothers who did not have hemophilia gene mutations. Thus, this study suggests that only about 2.6% of hemophilia patients have true de novo mutations. Whenever a study shows this much of a change from accepted current knowledge, others will usually try to repeat the study to determine whether the new results are actually valid. We'll see whether these results hold up. [WFH 2025 Critical Care Summit abstract FP-014]

Thrombophilia in Hemophilia Patients

6/22/25 A group from Poland presented a study of thrombophilia in hemophilia A and B patients. Thrombophilia is the tendency to clot too much, which is dangerous and can be fatal. It can happen in patients with Factor V Leiden, who have a mutation in their factor V that keeps it from being inhibited by activated protein C. Another common cause is mutations in the prothrombin (factor II) gene.

The study included 102 adult patients (86 As and 16 Bs) with a median age of 43 years (range 21–88). Seventy-six percent of patients had severe hemophilia and three percent developed inhibitors. Seven percent had Factor V Leiden and one patient had a prothrombin mutation. Nine patients (8.8%) had thrombotic events while on factor therapy, including four heart attacks and three strokes. That group had significantly higher incidences of diabetes, smoking, and obesity. They were also estimated to have a 5.2 times higher risk of death.

The bottom line is that thrombotic mutations were found in 7.8% of Polish hemophilia patients, higher than in the general population. It's not clear why hemophilia patients would have a higher risk of also having thrombotic mutations, but this suggests that we need to be better aware of the possibility. This may be especially true for patients switching to rebalancing agents, which all carry warnings about thrombotic complications. In patients already more disposed to thrombosis, further reducing the anticoagulant controls on the clotting process could be risky. [ISTH abstract PB0859]

Methotrexate for Joint Protection?

6/23/25 Methotrexate is an immune system suppressant that is used in rheumatoid arthritis (RA) to treat synovitis (an inflammation of the lining of the joints) and preserve joint integrity. A group of investigators in Lyon, France and Chicago wondered whether it would be effective in treating hemophilic arthropathy. In hemophilia B mice, they looked at the combination of extended half-life factor IX (EHL-FIX) and methotrexate. The mice were divided into two groups: Group 1 received i.v. EHL-FIX at 50 IU/kg every 3 days, while Group 2 received the same EHL-FIX treatment supplemented by subcutaneous methotrexate once a week.

They found that the animals in Group 2 had significantly reduced cartilage damage and exhibited significantly reduced blood vessel density in the joint, which suggested a lower risk of secondary bleeding. They concluded: "Low dose methotrexate may be a promising therapy to protect against irreversible cartilage damage caused by blood-induced arthropathy when used in combination with regular prophylaxis, and this promising therapy deserves further investigation." [ISTH abstract OC 44.4]

Joint Bleeding in Women with Hemophilia

4/24/25 Some studies have found joint damage in women with hemophilia and carriers, in spite of no reports of joint bleeds. This suggests sub-clinical bleeding ("microbleeds") may be occurring, but there has been very little research on joint health in women with bleeding disorders. In a study conducted by a group in Argentina, 37 women who either have hemophilia or are carriers (11 symptomatic and 14 asymptomatic) were evaluated using the HEAD-US and HJHS scores. Only two women reported joint bleeds, but 14 (38%) reported chronic joint pain in their ankles or knees.

The HEAD-US and HJHS scores were revealing. Only 10 subjects (27% of subjects) had HEAD-US scores of zero, while 11 (29.8%) had HJHS scores of zero, both indicating healthy joints. Interestingly, one symptomatic and one asymptomatic carrier had high HEAD-US scores indicating elevated joint damage. Thus, over 70% of the women in the study had some joint damage, but most didn't know it. The authors conclude: "WBD [women with bleeding disorders] need a routine joint status evaluation to detect early signs of joint damage and receive an adequate treatment." [WFH 2025 Critical Care Summit abstract PP-185]

Factor IX Levels Do Increase During Pregnancy

6/23/25 It is well-known that factor VIII levels increase in women during pregnancy, but it has always been assumed that factor IX levels stay the same. It turns out that assumption was based on very little data. At ISTH, a British group presented data on factor IX levels in 39 hemophilia B carriers across 63 deliveries, the largest group ever tested for factor levels in pregnancy. The carriers' median baseline factor IX level before pregnancy was 56% (range 8–133%). One-third of the group had diagnoses of mild hemophilia B. Overall, their median factor IX levels increased by 0.30 IU/ml (30% of normal), a substantial increase. For instance, a woman with a baseline level of 20% would on average increase to 50% during pregnancy.

There was a wide range of responses, from a decrease of 0.11 IU/ml to an increase of 0.81 IU/ml. Only two women, of the 39, had decreases in their levels, and several had small increases, but the majority showed marked increases. The increases were most pronounced in women with baseline levels of 30% or more. Women with baseline levels less than 30% tended to not increase as much. [ISTH abstract PB0554]

Hemophilia Clinical Trials in Women

2/7/25 Now that women are finally being recognized as having hemophilia, we need to make sure we are prepared to treat them. We know that women bleed differently than men. For instance, the severity of bleeding in women does not seem to depend on factor levels, as shown by several recent studies. Fortunately, we know that most women respond well to infused factor, but is that the best treatment? Or is there something better we should be doing?

Another concern is that hemophilia treatment products have only been tested in men, so we have no real idea whether they might work differently in women. Industry and FDA expect them to work the same, but with no actual clinical data, how can we be sure? A recent Letter to the Editor in the journal Haemophilia, [O'Donnell MJ et al., Haemophilia, 31(3) 575-577, 2025] gives a startling statistic. In the 113 interventional hemophilia clinical studies conducted between 2014 and 2024, there were a total of 4503 subjects. Only five of those subjects were women, and one of the women might have been counted twice. We have to do better.

There are reasons for this. First, of course, is the fact that until recently, the medical profession has neglected to observe that women can have hemophilia. However, even since the 1950s, when we started to see reports of hemophilia in women, women have been ignored. I've often asked industry reps at conferences why they don't pay more attention to women. After all, we're talking business here, and including women could potentially double their market. I mostly get puzzled looks.

Another reason has to do with the pharmaceutical business. It is well known that the first company to get a product licensed in a new treatment area often gets the major share of the subsequent market. Therefore, they want the fastest path to licensure, which means the easiest and fastest clinical studies. To get their studies done with the least muss and fuss, they want "perfect patients" as study subjects. They don't want patients with other diseases or conditions that might influence the results. And, especially now that we know that women bleed differently, they don't want that potential complication.

Finally, most studies are done on subjects with severe and/or moderately severe (<2% factor level) hemophilia. Since women tend to have moderate or mild hemophilia, most of them are not eligible. However, those severity determinations are based on factor levels, and again, we know that women don't bleed in accordance with their factor levels. Until we know more about how women bleed, companies are going to be reluctant to include them. We need more research, but the key to getting more research may be that we need more advocacy.

FDA is supposed to ensure diversity in clinical studies, but even under more diversity-friendly administrations, they have not made a strong push in that direction. Instead, in my view, over the past few years, they have been giving broad indications for products that have only been tested in narrow groups of patients. Is that looking for trouble?

More on X-Chromosome Inactivation as a Cause of Bleeding in Women

Now that we know that women can have hemophilia, we need to understand why. Part of the reason that early researchers thought that women would naturally be immune from hemophilia was because of their simplistic understanding of genetics. We understand a lot more today, but our knowledge is still quite incomplete. One thing we have learned about is X-chromosome inactivation (XCI). We once thought that women wouldn't have hemophilia because they have two X-chromosomes. Even if one of the X-chromosomes had a mutated factor VIII or IX gene, it was assumed that the "good" gene on the other X-chromosome would still make enough factor.

Mary Lyon, an English geneticist, discovered in 1961 that in female mammals (including humans) one of the X-chromosomes in every cell is inactivated. The inactivation process is usually random, so in a woman with a "good" factor IX gene on one chromosome and a mutated factor IX gene on the other, in about half of her cells the X with the good gene will be the only one active and in the other half, the X with the mutated factor IX gene will be the only one active. With this 50:50 split, she should produce about 50% of normal factor IX and about 50% mutated factor IX. Since a 50% factor IX level is within the normal range, although at the bottom, she shouldn't have hemophilia.

However, Lyon and others soon found that in many women the inactivation pattern is skewed. Instead of a 50:50 split, the ratio can vary to as much as 80:20 or even 90:10. If the 80 or 90% represents the mutated gene, then most of the factor she makes will be defective and she should have hemophilia. This seemed to make so much sense, that people jumped on the idea that skewed XCI must be the reason that women have hemophilia. In fact, one of the presentations that we're going to talk about makes the statement, "Hemophilia A and B are X-linked genetic disorders predominantly affecting males, although female carriers may present with variable symptoms due to X-inactivation."

Not so fast! There are a number of complications to this story. There have been a number of studies showing that some carriers with highly skewed XCI don't have hemophilia and some women who have hemophilia don't have skewed XCI. However, the larger problem is that the XCI theory still works on the idea that bleeding in women is due to low factor levels, yet a number of recent studies show that bleeding in carriers does not depend on factor levels. That's not to say that skewed XCI and low factor levels are irrelevant. They are probably part of the explanation, but there is still a big gap. We have to accept that we really don't know why carriers bleed.

6/22/25 Three presentations at ISTH looked at XCI. The first one is a case study of a woman with Turner syndrome. Turner syndrome is a rare disorder in females in which part or all of an X-chromosome is missing. In this woman's case, she had a large deletion that included both the factor VIII and IX genes. Thus, she is a carrier of both hemophilia A and B, but her problem is thrombophilia—she clots too much. She actually has high factor VIII (146% of normal) and factor IX (121%) levels, but those are still in the normal range, which goes up to 150%.The authors attribute her high factor levels to skewed XCI which favors the "good" X-chromosome. [ISTH abstract PB1430]

6/23/25 A study from China looked at 20 women (15 As and 5 Bs) with hemophilia to see whether they could determine the causes of their bleeding. A little more than half of the patients had skewed XCI, which was thought to affect their factor levels. The authors did a correlation analysis between the amount of skewing and factor levels, which showed a significant correlation. The correlation coefficient shows that the degree of XCI skewing explains about 77% of the variation in factor levels, so skewing may be important in those patients. However, almost half of the women did not have skewing, so that could not explain their bleeding symptoms. [ISTH abstract PB1389]

6/22/25 Finally, a group from Italy looked at a mother and daughter who are both hemophilia B carriers but have different bleeding tendencies. The mother, 38 years old, has a factor IX level of 5% and has had a number of bleeding episodes including post-partum hemorrhage and spontaneous hematomas, including severe ones, for which she has received factor IX treatment. The daughter is 19 years old and has a factor IX level of 17% with no bleeding history. Both have the same genetic mutation. Both have skewed XCI patterns, 81:19 in the mother and 80:20 in the daughter. The authors assume that the 81% and 80% refer to the chromosome with the mutated gene, but apparently haven't tested that. Note that, although the women have almost the same skewing pattern, their factor levels are different. The authors conclude: "Skewed

methylation [XCI] may contribute to modifying the hemorrhagic phenotype [bleeding behavior] in related patients who have the same mutation, but it is not the only factor involved" [underlining added]. [ISTH abstract PB1420]

All of this suggests that skewed XCI does contribute to bleeding in women, but there are obviously other things that also play major roles. Biology seemed much simpler in the early 1800s when physicians decided that women couldn't have hemophilia. Hopefully now, we realize how complex it actually is and that we don't know as much as we thought we did. We don't understand why women bleed, and we've got 200 years of neglected research to catch up on.

Bleeding Behavior in Carriers and Women with Hemophilia

6/23/25 We've been saying that women don't bleed according to their factor levels, and a group from Spain has presented more evidence of that at ISTH. They looked at 138 women and girls belonging to 61 families with hemophilia A or B. They classified them according to the 2021 ISTH criteria into groups of asymptomatic carriers (factor level >40% with no bleeding symptoms), symptomatic carriers (factor >40% with bleeding symptoms) and those with hemophilia A or B (factor levels <40%). They found some interesting results.

Most strikingly, about half (52%) of the women with hemophilia A and one-third of the women with hemophilia B had no bleeding symptoms, even though their average factor levels were 34% (range 12–40%) for the As and 30% (28–33%) for the Bs. In contrast, the symptomatic carriers, who had an average factor level of 88% (45–135%), all had bleeding symptoms—even the woman with a 135% factor level. The factor levels in the asymptomatic carriers ranged from 41% to 145%, very comparable to the range for the symptomatic carriers. Then why the difference in bleeding?

These results just add to the growing body of information that shows that women bleed differently. Factor levels are probably still important in women, but there appear to be other things that significantly affect their bleeding behavior. [ISTH abstract PB0331]

6/23/25 A group of female researchers from Penn State is giving it a start. Thrombin (activated factor II) is the primary output of the clotting cascade. Thrombin converts fibrinogen to fibrin, which sticks to itself and to platelets to form the clot. Therefore, the more thrombin generated, the better the clot. The Penn State researchers looked at thrombin generation assays (TGAs) in 11 hemophilia B carriers to see whether TGA would show differences between carriers who bleed and those who don't. In four carriers with factor IX levels below 50% (who would be considered to have hemophilia in the U.S.), two had bleeding symptoms and two didn't, but there were no significant differences in their TGA results.

However, three other carriers with levels above 50% who had abnormal bleeding (and would be considered symptomatic carriers according to the ISTH criteria) did show significant differences in TGA compared to four others with >50% levels. Thus, the results are complicated, but as we continue to poke at the problem, more clarity may arise. [ISTH abstract PB0327]

6/23/25 A group from the Western Pennsylvania HTC looked at post-partum hemorrhage (PPH) in hemophilia carriers at their center. They looked at 36 hemophilia A and B carriers with a total of 67 deliveries (15 A and 52 B). Interestingly, low factor levels below 50% occurred in 54.1% of the hemophilia B carriers compared to only 25% of the As. This was a retrospective study, so it looked at what had already happened rather than looking at the effects of a specific intervention. Hemostatic therapy (factor or antifibrinolytics) was given to 68.7% of the patients but 80.7% of the Bs.

PPH occurred in 24% of the deliveries but only the hemophilia B carriers needed rehospitalization for bleeding issues. There was no significant difference in the hemostatic therapies given between the PPH and non-PPH cases. The authors conclude, "Factor activity may not predict bleeding risk, highlighting the need for individual management." [ISTH abstract PB0286]

Note that in the above, I'm using "carrier" in its strict scientific sense, as do the authors of the papers. A carrier is simply a person who carries a specific gene, in these cases mutated factor VIII or IX genes. The fact that they carry that gene, does not tell us their medical condition. Carriers of hemophilia genes (mutated factor VIII or IX genes) may or may not have hemophilia. What these studies are trying to determine is what else happens to give a carrier a bleeding problem or not.

Since it appears that it's not just genetics that causes hemophilia in women, there is also the question of whether a woman who is not a carrier could also have bleeding problems due to a cause we haven't identified yet. At the recent NBDF meeting there was a session on bleeding disorders of unknown cause (BDUC), which are becoming more prevalent. This raises the scientific question whether the "unknown cause(s)" have anything to do with why carriers bleed.

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HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Summer 2025

There is a significant amount of new product development going on in hemophilia B. The potential new 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 alphabetically by the name of the organization developing the product.

A number of the items below were presented at the International Society on Thrombosis and Haemostasis (ISTH) 2025 Congress, which was held June 21–25, 2025, in Washington, DC. Copies of the abstracts (summaries) for the presentations are available for free at https://www.isthcongress.org/abstracts.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using, including products for inhibitor treatment. This section also includes updates on some of the current products on the market.

CSL Publishes Comparison between IDELVION® and ALPROLIX®

7/23/25 CSL Behring markets IDELVION®, an extended half-life (EHL) recombinant factor IX concentrate. The factor IX in IDELVION® is fused to an albumin molecule to give it a longer half-life. ALPROLIX® is a competing product marketed by Sanofi in which factor IX is bound to the Fc-portion of an antibody molecule to also give it a longer half-life. Previous studies with the two products had only compared IDELVION® or ALPROLIX® to comparable standard half-life products, but not to each other. Therefore, a group of researchers from Germany and Italy looked at comparisons in the real-world outcomes for patients on the two therapies.

They looked at 194 male patients with severe or moderate hemophilia B who had been on prophylaxis with the products for at least 12 months, 107 subjects on IDELVION® and 87 subjects on ALPROLIX®. They found a significantly lower average factor IX consumption with IDELVION®: 42.4 IU/kg/week for IDELVION® and 65.2 IU/kg/week for ALPROLIX®. The subjects on IDELVION® had an average dosing interval of 9.5 days while those on ALPROLIX® had an average dosing interval of 7.9 days. However, the average annualized bleeding rates (ABRs) were not significantly different between the two products. Novo Nordisk's Rebinyn® (Refixia in Europe) was not included in the analysis since its use in Italy has been limited. A future comparison including Rebinyn® will be published later.

So, do these results suggest that IDELVION® is the better product? Not at all! The "best" product is the one that works best for you. First, note that there were no significant differences in bleeding rates between the two products. They are equivalent in average effectiveness. Also, note that the above results are all averages. If you look at individual patient results, there is quite a spread in the numbers. An important principle in medicine is that every patient is different. We individualize treatment because what works best for one patient might be a dud for another. In the U.S., we don't try to put everyone on the same treatment, even though that would be the most cost-effective approach. Instead, we try to give each patient the best outcome. The bottom line is to work with your physician to find the product that works the best for you. [Oldenburg J et al., Adv Ther, online ahead of print 7/23/25]

Staidson Reports on Clinical Studies in Inhibitor Patients

6/22/25 Staidson (Beijing) Biopharmaceuticals is developing bemiltenase alfa (BA) as a bypassing agent for treatment of hemophilia A and B patients with inhibitors. BA is an enzyme that activates factor X. It is purified from the venom of the Russell's Viper snake. Snake venoms often contain compounds that activate clotting. Unpurified venoms were used experimentally in the 1930s as treatments for hemophilia, and are still used in laboratory assays to activate certain clotting factors. Staidson is currently testing their purified product in Phase II clinical studies in China.

In 70 patients in their Phase Ib/IIa study, the researchers looked at various doses of BA, after which they selected a dose of 0.10 U/kg for their subsequent Phase IIb study in 25 patients. The results showed good control of bleeding with no serious adverse events. The average half-life of BA was 8.7 hours (range 7.1 to 11.1), which is longer than many of the current bypassing agents. [ISTH abstract PB0772]

Versiti Developing Longer-Acting Factor VII for Inhibitor Treatment

6/22/25 Researchers from Versiti Blood Research Institute and the University of British Colombia are developing a messenger RNA (mRNA) treatment to produce factor VII as a bypassing agent for treatment of hemophilia patients with inhibitors. Current factor VIIa products used as bypassing agents have a very short half-life of only a few hours. Therefore, inhibitor patients may require multiple infusions per day to treat a serious bleed. The Versiti group proposes instead using an mRNA encoding the instructions to produce factor VII on a longer-term basis.

The mRNA is encapsulated in a lipid nanoparticle (LNP), similar to the way the mRNA COVID vaccines encapsulate an mRNA that produces the COVID virus spike protein. After injection of the LNPs, they travel to cells and inject their mRNA contents into the cell. The cell then takes up the mRNA and produces the protein encoded by it. If the mRNA codes for a COVID virus spike protein, the cell will produce copies of that protein and secrete them into the bloodstream where the immune system will see them and produce antibodies against the spike protein, and thus against the virus. With the Versiti therapy, the mRNA codes for factor VII, which is then secreted into the bloodstream to help in clotting.

In a mouse model for hemophilia A and a rat model for von Willebrand disease, the researchers found that a single injection can double the normal factor VII level in the blood for up to 48 hours. This could provide additional clotting activity in inhibitor patients with less-frequent injections, as compared with a more frequent i.v. infusion of a bypassing agent. This work is in the early stages, but the results so far seem promising. [ISTH abstract OC 59.4]

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 system is a complex system of clotting factors that promote clotting plus anticoagulants that inhibit and control clotting. In a person without a bleeding disorder, the clotting and anticoagulant activities are in balance, so the system produces clots as needed. In hemophilia, with the loss of some clotting factor activity, the system is unbalanced; there is too high a level of anticoagulant activity keeping the blood from clotting. Rebalancing agents mainly reduce or inhibit the activity of anticoagulants in the system. Most of these agents work to help restore clotting in people with both hemophilia A or B, with or without inhibitors, and will probably find application for other bleeding disorders.

Protein S as a Target for Rebalancing Hemostasis

Protein S is an anticoagulant in the clotting system that has several roles. It acts as a co-factor for activated protein C (APC) in the inactivation of factors Va and VIIIa. A co-factor is a compound that binds to an enzyme to increase the activity of the enzyme. For instance, factor VIII is a co-factor for activated factor IX (factor IXa). It binds to factor IXa and increases factor IXa's ability to activate factor X. Protein S also directly inactivates factors Va, IXa and Xa. Because it has the ability to inactivate several of the activated clotting factors, inhibition of protein S is seen as an attractive target for rebalancing.

3/26/25 Silence Therapeutics is developing a small interfering RNA (siRNA) treatment to inhibit the body's production of protein S as a rebalancing therapy. Their siRNA is linked to another molecule that prevents it from entering any other cells except liver cells. Keeping protein S at normal levels in other body cells is expected to decrease the risk of thrombosis from the treatment. In hemophilic mice and in non-human primates, their treatment was shown to improve clotting without triggering widespread clot formation throughout the body. [Eladnani RP et al., J Thromb Haemost, online ahead of print 3/26/25]

6/22/25 A group of researchers from Duke and LSU is looking at aptamers against protein S. An aptamer is a small piece of DNA or RNA that can bind to a protein and inhibit its effect. Their goal is to infuse their aptamer along with an infusion of factor IX in order to increase the half-life of factor IXa. Laboratory studies so far have shown a decrease in clotting time and increases in thrombin generation and factor X activation. [ISTH abstract OC 20.2]

6/23/25 Star Therapeutics, through their Vega Program, is developing VGA039 as a rebalancing agent targeting protein S. As mentioned above, many of the rebalancing agents will probably work for other bleeding disorders, and Star is developing VGA039 specifically for patients with von Willebrand Disease (vWD). They had previously presented results for VGA039 in a non-human primate model of vWD, and at ISTH, they showed data from experiments with blood from vWD patients. They also showed data from non-vWD patients whose blood samples were treated with an antibody to inhibit factor VIII. In both cases, VGA039 significantly reduced clotting times. [ISTH abstract PB0111]

Novo's Alhemo® Approved for Non-Inhibitor Patients

7/31/25 Novo Nordisk has developed Alhemo® (concizumab) as a rebalancing agent for treatment of patients with hemophilia A or B. Alhemo® is a daily, subcutaneous monoclonal antibody product that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. In December 2024, the U.S. FDA approved Alhemo® for treatment of hemophilia A or B patients 12 years of age or older with inhibitors. On 7/31/25, FDA extended that indication to include A and B patients, 12 or older, without inhibitors. Thus, Alhemo® joins Pfizer's Hympavzi™ and Sanofi's Qfitlia™ as subcutaneously-administered rebalancing agents for non-inhibitor patients. Both Alhemo® and Qfitlia™ are also approved for use in inhibitor patients. [Novo Nordisk press release 7/31/25]

6/22/25 The results of the clinical studies for Alhemo® were presented in several papers at ISTH. In A and B patients without inhibitors and without target joints, Alhemo® treatment produced a median ABR of 0.4 (bleeds/year), compared with a pre-treatment ABR of 26.9 for patients using on-demand treatment. For patients without inhibitors, but with target joints, the results were a median ABR of 3.4 compared to a pre-treatment ABR of 14.9. [ISTH abstract OC 59.2]

Another paper presented the results for non-joint bleeds with Alhemo®. Most non-joint bleeds were muscle bleeds. The data showed an ABR of 0.1 for muscle bleeds with Alhemo® prophylaxis compared to an ABR of 1.5 for those patients using on-demand treatment with clotting factor. [ISTH abstract PB0851]

5/14/25 A group from The Maldives and Pakistan performed a summary analysis of all of the clinical studies that have been published on Alhemo®. They found a median ABR of 2.9 for hemophilia A patients and 1.6 in hemophilia B patients. These were significantly lower than the pre-treatment on-demand results of 19.6 and 14.9, respectively.

(Note that the manufacturers often compare results for their prophylactic treatment with results from patients using on-demand treatment with clotting factor. That's completely appropriate for inhibitor patients, who are mainly treated on-demand. However, most non-inhibitor patients are treated prophylactically [or should be, if possible] and have much lower ABRs with that therapy.)

Since the ABRs for on-demand treatment are usually much higher than ABRs for patients on prophylaxis, that makes the ABR for the new treatment look even better. However, in the studies for Pfizer's and CSL's hemophilia B gene therapies, they measured patient's ABRs during a 6-month lead-in study while they were still on prophylaxis with their previous factor treatment. From that data, we know that the "typical" ABRs for Bs on factor prophylaxis are about 4 bleeds/year, but with wide patient-to-patient variation. Therefore, the ABR of 1.6 for Bs on Alhemo® does represent a significant improvement.

The group also looked at thrombin generation and found a dose-dependent correlation. That means that as you increase the dose of Alhemo®, you get a corresponding increase in thrombin levels. Thrombin (factor IIa) is the factor that converts fibrinogen to fibrin to form a clot, so the more thrombin, the better the clot. They also found a corresponding decrease in TFPI activity, as expected.

In terms of safety, they found that up to 26% of patients developed low-titer antibodies against Alhemo®, but those antibodies appeared to have no effect on bleeding behavior. Most adverse events were mild or moderate and there were no serious adverse events attributed to Alhemo®. There were also no thrombotic episodes. The risks of injection site reactions, fever, and musculoskeletal pain were not significantly different from those for normal clotting factor.

One of the unusual adverse events in a number of patients was upper respiratory infection. However, the statistics showed no significant difference in incidence from the control groups that did not receive Alhemo®. The fact that the clinical studies were performed during the COVID pandemic may account for some of those infections. [Siddiqui E et al., Clin Appl Thromb Haemost, online ahead of print 5/14/25]

6/25/25 Another group looked at the pharmacokinetics of Alhemo®. They found a wide patient-to-patient range for half-life of 31 to 72 hours. Alhemo® is a daily subcutaneous injection. [Mahlangu J, Expert Opin Drug Metab Toxicol, online ahead of print 6/25/25]

Pfizer Announces Results for Hympavzi™ in Inhibitor Patients

6/26/25 Pfizer has developed 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™ was the first rebalancing agent licensed in the U.S. in October, 2024, but only for non-inhibitor patients. They have now reported Phase III results for Hympavzi™ in inhibitor patients.

In 48 inhibitor patients with hemophilia A or B treated for at least 12 months, they found an ABR of 1.39, compared to an ABR of 19.78 in the same patients for on-demand treatment with bypassing agents. Hympavzi™ was generally well-tolerated with side effects consistent with those seen in non-inhibitor patients treated with Hympavzi™. Pfizer continues to analyze the data and is expected to file an application to extend their indication to inhibitor patients next year. [Pfizer press release 6/26/25]

Sanofi Presents Additional Data on Qfitlia™

6/22/25 Sanofi has developed Qfitlia™ (fitusiran), a rebalancing agent for treatment of hemophilia A or B patients 12 years or older, with or without inhibitors. Qfitlia™ is a small interfering RNA (siRNA) treatment that inhibits the production of the anticoagulant antithrombin (AT). Fitusiran is a monthly or every-other-month subcutaneous injection. At ISTH, they presented additional data on the new product.

One paper presented the results of a study of the pharmacokinetics and pharmacodynamics of Qfitlia™. Using computer modeling, they predicted that regardless of dosage regimen (monthly or every two months) more than 99% of patients would reach a steady-state AT level after 19.9–22.7 weeks of use. That is consistent with the results from the clinical studies. After discontinuation of Qfitlia™, AT levels are predicted to remain at less than 60% for about 20 weeks.

AT levels in 213 subjects in the clinical studies were successfully maintained in the 15–35% range, with an average AT level of 23.5%. When starting Qfitlia™, patients have the opportunity to adjust their dose, based on AT measurements. Overall, 37.6% of clinical study subjects required no dose change, 56.3% needed one dose change and 6.1% required more than one dose change to stay within the 15–35% range. [ISTH abstract OC 59.1]

6/22/25 You never know what you're going to find with new products, and in some cases, that can give scientists clues to help understand what's going on in the human body. AT actually occurs in two forms (called isoforms), alpha-AT and beta-AT. The alpha form comprises about 90% of the AT found in the body. The beta form only comprises about 10% of the total AT, but it has twice as much anticoagulant activity as the alpha form.

A French group has shown that Qfitlia™ changes the ratio of alpha to beta in patients. It increases the amount of beta-AT in the bloodstream, thus giving the total AT (alpha plus beta) a higher anticoagulant activity. A similar increase in beta-AT was also found in hemophilia A mice. Interestingly, they found no change in the alpha/beta ratio in the liver cells that make AT. That suggests that the decrease in the amount of alpha-AT occurs after the proteins have been secreted from the liver cells and are in the circulation. Further research is needed to understand this, but it apparently has no bearing on the efficacy of Qfitlia™. [ISTH abstract OC 74.5]

Another group looked at the relationship between AT levels and the annualized bleeding rate (ABR). They found a direct relationship between AT level and ABR, based on data from 254 patients spanning 552.9 patient-years of observation. Although there is fairly wide patient-to-patient variation, their averages showed an ABR of 0.73 at an AT level of 10%, 2.31 at an AT level of 15% and 4.58 at an AT level of 35%. They point out that the ability to vary the AT level, and thus the ABR, by varying the Qfitlia™ dose is an advantage. [ISTH abstract OC 59.3]

GENE AND CELL THERAPY

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

Be Bio Doses First Patient in Clinical Study of BE-101

7/30/25 Be Biopharma is developing BE-101, a cell therapy for hemophilia B. BE-101 uses a patient's own B cells, genetically engineered to contain a normal factor IX gene. B cells are a type of white blood cell that are part of the immune system. B cells produce antibodies and thus are good at producing large amounts of protein. The patient's B cells are harvested from the bloodstream, transformed in the laboratory to contain a functional factor IX gene, and then transfused back into the body. B cells tend to sequester themselves in a niche in the bone marrow where they have lifetimes on the order of decades. Because BE-101 uses the patient's own cells, there is not expected to be any immune reaction against the transplanted cells. Therefore, the treatment can be repeated, for instance, to add more cells and thereby increase the production of factor IX.

Be Bio has now treated the first patient in their Phase I/II study in adult subjects with severe or moderately-severe (<2% factor IX) hemophilia B. Part 1 of the study will be a dose escalation, starting with a small dose of B cells and then gradually increasing the dose in subsequent patients to determine the dose required

to produce the desired factor IX activity level. Part 1 will include up to 18 patients, then the safety and effectiveness of the selected dose will be examined in up to six additional patients. [Be Biopharma press release 7/30/25]

Belief Biomed/Takeda Gene Therapy Update

4/10/25 Belief Biomed (BBM), a Chinese company, has developed BBM-H901, a gene therapy for hemophilia B that was licensed in China in April 2025. BBM-H901 uses a proprietary AAV vector to deliver a Padua-variant factor IX gene. BBM is working with Takeda China who will commercialize the product in China, Hong Kong, and Macau. BBM/Takeda may pursue licensure in the U.S. or Europe, having received Breakthrough Therapy and Orphan Drug designations from FDA and Advanced Therapy Medical Products designation from the EMA.

At ISTH, BBM reported on results from nine patients who had been on the therapy for 3.5 to 4.7 years. Their average factor IX level was 48.4% at the most recent follow-up. There were no deaths, severe adverse events, thrombotic events or inhibitors. One patient of the original ten dropped out of the study after 3 years because of a low factor level of 2%. [ISTH abstract OC 69.2]

CSL BEGINS LONG-TERM FOLLOW-UP STUDY OF HEMGENIX®

6/24/25 CSL Behring 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. At ISTH, CSL described their long-term follow-up study, called IX-TEND 3003, of the patients who have been in any of the Phase IIb or III studies of HEMGENIX®. Subjects will be monitored for long-term efficacy and safety for 15 years post-treatment. For the first 10 years post-treatment, subjects will be tested every 6 months and then annually for up to 15 years. [ISTH abstract PB0843]

7/11/25 CSL has also published the final results of their Phase IIb study of HEMGENIX®. This was a small study meant to show that changing the from the normal (called "wild-type") factor IX gene, which had been used in the previous studies, to the higher-activity Padua gene gave acceptable results. The three patients in the Phase IIb study are thus the patients who have had HEMGENIX® for the longest time.

The patient's average factor IX activity at 6-weeks post-treatment was 30.6%. This increased to 40.8% after 1 year and to 45.7% after 5 years. Thus, factor IX levels actually increased over the 5 years of the study. The average ABR over the 5 years was 0.14, with two of the patients having no bleeds. All of the patients were able to stop prophylaxis. All safety results were comparable with what was seen in the Phase III study—there were no late-emerging events. [von Drygalski A et al., Blood Adv, online ahead of print 7/11/25]

6/23/25 At ISTH, CSL also presented data from their previous studies of AMT-060, their treatment using the wild-type factor IX gene. Factor IX levels remained steady for up to 8 years and all patients remained off of prophylaxis. The treatment remained well-tolerated with no new adverse reactions. Thus, the results continue to look favorable. [ISTH abstract PB0780]

St. Jude/UCL Reports Gene Therapy Results for 13 Years

6/12/25 St. Jude Children's Research Hospital and University College London (UCL) performed one of the first successful gene therapy studies for hemophilia B, starting in March, 2010. This was mainly a research study to investigate the feasibility of gene therapy, rather than a study aimed at developing a specific product. This June, 2025, they published the results of a 13-year follow-up for the patients in the ongoing study.

Ten severe hemophilia B patients received one of three doses of their AAV vector containing a wild-type factor IX gene. Factor IX levels have remained steady across the three dose cohorts with average factor IX levels of 1.7%, 2.3% and 4.8%, respectively in the lowest to highest dose groups. The average ABR for the whole group fell from 14.0 pre-treatment to 1.5 after 13 years.

The main adverse events were temporary liver inflammation. No inhibitors, thrombotic events, or chronic liver injuries were seen. Two cancers were observed in the patients, but they were deemed unrelated to the gene therapy treatment by an expert team. These results suggest that AAV gene therapy can provide a sustained clinical benefit with no late-onset safety concerns for an extended period of time. [Reiss UM et al., N Engl J Med, 392:2226-2234, 2025]

Ten-Year Ban on Heritable Gene Editing

5/27/25 In discussions of gene therapy, I'm often asked whether the treatment changes the genes in the reproductive cells, so that a patient no longer transmits hemophilia to their children. The answer is "No," there is no change to those genes, and in fact, it would take quite a bit of new technology to make such a change.

However, that question is still a valid concern. Modifying genes in reproductive cells is called "germline editing." According to Dr. Bruce Levine, a professor studying cancer gene therapy at the University of Pennsylvania, "Germline editing has very serious safety concerns that could have irreversible consequences. We simply lack the tools to make it safe now and for at least the next 10 years." In other words, if we mess up, we potentially risk producing an actual human being who would have to suffer with that mess for their lifetime.

Because of these concerns, three major organizations, the Alliance for Regenerative Medicine (ARM), the International Society for Cell and Gene Therapy, and the American Society for Cell and Gene Therapy are calling for a 10-year moratorium on germline editing. The proposal came out of a 5/26/25 meeting in Washington DC that convened scientists, bioethicists, religious leaders, biotech executives, patients, and policy experts. The moratorium would not affect the current gene therapies, which are called somatic cell therapies. Somatic cells include all of the rest of the cells in the body except those involved in reproduction. [ARM press release 5/27/25]

Psychological Impact of Gene Therapy in Hemophilia

6/24/25 A group from Italy has presented preliminary results from an ongoing 15-year study of the psychological effects of gene therapy in hemophilia. At ISTH, they presented the results from their pre-treatment and 1- and 6-month post-treatment surveys. At 6 months post-treatment, patients reported fewer limitations in work and daily activities, less fatigue, an increased perception of enjoying excellent health, and greater confidence in the possibility of resolving potential problems. Interestingly, several of the participants viewed their satisfaction with their previous treatment more negatively at 6 months post-gene therapy compared to their evaluations just prior to the therapy. [ISTH abstract PB0863]

You know who she is. She's the hematologist, social worker, or nurse who cares about your total wellness, not just your treatment. She's your coworker, neighbor, or friend with hemophilia B who stands up for herself and other women like her. She's your sister, daughter, mom—the woman who is always there for you.

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Nelly Never Gives Up: Called to Serve and Impact Families in the Latino Community

BY SHELLY FISHER

Within minutes of speaking with Nelly Miranda, you feel as if you've known her for years. She radiates warmth, curiosity, and kindness, the kind that makes you feel instantly seen and valued.

During our interview, she was the first to ask a question: "Do you have a bleeding disorder?" It wasn't a formality; it was pure care. Had I answered yes, I'm certain she would have immediately shared resources, checked on me later, and maybe even set a lunch date.

That's just who Nelly is a helper by nature, an advocate by calling, and a mother whose personal journey with hemophilia B has inspired her to serve others. In addition to what she considers her most important role being mom to her son and daughter she serves as the Community Latino Coordinator for the New York City Hemophilia Chapter. Her passion for helping others shines through every conversation and every connection she makes.

A Mother's Instinct and a Life-Changing Diagnosis

Nelly earned her degree in accounting and finance in Mexico before moving to New York in 2001 with a passion for supporting nonprofit organizations. Her career path, however, took a dramatic turn in 2010 when her 10-month-old son had an accident that changed everything.

Her son had just learned to crawl and, like many curious babies, took a tumble from the bed. Concerned, Nelly rushed him to the emergency room. After a brief examination, the doctors reassured her that "these things happen" and sent her home. But Nelly's intuition told her something wasn't right.

Over the next few days, her baby's crying continued. At a follow-up visit, the pediatrician again dismissed her worries, saying, "Children fall off the bed. It's okay." But when swelling appeared on her son's head, Nelly's instincts kicked into overdrive. She went back to the hospital and this time refused to leave without answers.

Doctors proposed draining the blood that had accumulated, but Nelly pushed for further testing first. Her persistence paid off. The tests revealed that her son had been bleeding internally for a week due to mild hemophilia B. He was immediately treated with clotting factor and remained in intensive care for several days.

That moment forever changed Nelly's life. "I was scared, but I also knew I had to learn everything I could," she recalls. "You don't expect to face something like this, but

you have to be strong for your children."

Discovering Her Own Diagnosis — and Her Daughter's

At the time of her son's diagnosis, Nelly was told that she didn't need to be tested because hemophilia B is typically passed down through mothers to sons. "The doctor said that because my son had hemophilia, I must be a carrier, and that was that," she explains. "They said it was very rare for women to actually have hemophilia."

Years later, in 2022, Nelly's own experience challenged that assumption. After a routine dental cleaning, she began bleeding heavily for nearly five days. Alarmed, she went to her local Hemophilia Treatment Center (HTC), where tests confirmed what she had long suspected: she, too, had mild hemophilia B.

"I tell every mom and girl I meet, if you have symptoms, get tested," she says passionately. "It's very different to be a carrier than to have hemophilia B. You need to know."

When her daughter began complaining of joint pain, Nelly once again trusted her instincts and pushed for testing. The results confirmed that her daughter also had mild hemophilia B. "Now we know," she says proudly. "And knowledge is power."

Today, her daughter is thriving as a college student, managing her health with self-awareness and confidence. She's been granted accommodations such as a lower dorm floor and the use of a golf cart on campus when needed. Nelly's son, now a talented varsity baseball player, has transitioned from occasional factor use to a consistent prophylaxis routine that keeps him active and safe.

"As a parent, you have to advocate for early testing," Nelly emphasizes. "It makes all the difference when your children get the care they need early on."

Wisdom from the Women Before Her

Nelly credits her grandmother, now 100 years old, as a source of courage and guidance. Despite her age, her grandmother still calls Nelly regularly on her cell phone, offering wisdom that has shaped Nelly's perspective.

Nelly recalls that her grandmother's son, Nelly's uncle, suffered from frequent bleeds but was never formally diagnosed. Working as a ranch hand, he attributed his injuries to "something wrong with his platelets" and passed away in his 40s.

"It was my grandmother who told me, 'You need to find out what this is so you can help them,'" Nelly says. "She didn't want me to live in denial like so many families do. She wanted me to act."

That advice became a guiding principle. Nelly not only became an advocate for her family but also extended that compassion to countless others in the Latino community.

Serving Families, Breaking Barriers

In her role as Community Latino Coordinator for the New York City Hemophilia Chapter — and as a volunteer for both the New England Hemophilia Association and The Coalition for Hemophilia B (CHB) Nelly dedicates herself to helping families navigate the complex world of bleeding disorders.

"So many Latino families have a hard time understanding their diagnosis," she explains. "Add the language barrier, and it becomes even harder. My job

is to make sure they truly understand what's happening in their own language so they can take care of themselves and their children."

Her outreach includes translating medical information, guiding families through treatment options, connecting them with HTCs, and helping them access educational and financial resources. "When you see the relief on a parent's face because they finally understand it's the best feeling in the world," she says.

Paying It Forward Through Community and Compassion

Nelly describes her work with CHB as her way of "paying forward" the support her family once received. She has attended CHB's Annual Symposium for the past four years and calls it one of the most inspiring events of her life.

"It's like one big family reunion," she says with a smile. "I love working check-in and greeting everyone. There's always something special for the kids horseback riding, crafts, fun activities and every year I learn something new."

She recalls seeing a young girl confidently perform her own infusion and being deeply moved. "It made me think of how far we've come," she says. "And how strong these kids are."

Beyond education and bonding, Nelly admires how CHB programs encourage community service among youth. "I love that we teach our kids to give back like making soap for the homeless or learning about recycling," she says. "It's about building empathy and responsibility."

For Nelly, the symposium is more than an event it's a lifeline. "Meeting other families who share our experiences reminds me that we're not alone," she reflects. "It keeps me grounded and grateful."

Guidance for the Newly Diagnosed

When asked what advice she offers to families new to hemophilia B, Nelly doesn't hesitate. "I always share my story," she says. "It helps them see that they're not alone and that they can handle this."

Her approach is rooted in connection. She encourages new families to get involved right away to attend local chapter meetings, camps, and events where they can meet others who truly understand.

"The kids should meet other kids immediately," she says. "It helps them feel normal, confident, and accepted. Everyone processes differently, but connection is the key."

The Strength of Family and the Power of Hope

Nelly credits her strength to the women who came before her, her grandmother and mother — both of whom she now knows have lived with symptoms of hemophilia themselves. "They were so strong," she says. "They protected me growing up, guided me, and pushed me to find answers. Everything I do is because of them."

Even with her busy schedule, Nelly carves out time to unwind with her children. Their favorite tradition? Movie nights. She's a self-professed movie buff who loves stories that transport her to new places, but her heart belongs to romantic comedies like The Proposal. "I love movies that make me laugh and feel hopeful," she says.

Her personal mantra, however, is what truly defines her: "Do your best and never give up."

Those words guide her through every challenge and triumph, from advocating for her family's health to empowering others in the hemophilia community.

As she continues to build bridges, educate families, and uplift others, Nelly's story reminds us that one person's courage can ripple through generations. Her determination, compassion, and commitment to service prove that true strength comes not from what we endure, but from how we choose to rise, again and again, for the sake of others.

IMPORTANT SAFETY INFORMATION

What is HEMGENIX?

HEMGENIX®, etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

Please see full prescribing information for HEMGENIX at HEMGENIX.com.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use HEMGENIX safely and effectively. See full prescribing information for HEMGENIX.

HEMGENIX® (etranacogene dezaparvovec-drlb) suspension, for intravenous infusion
Initial U.S. Approval: 2022

----------INDICATIONS AND USAGE----------

HEMGENIX is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or
- Have repeated, serious spontaneous bleeding episodes.

----------CONTRAINDICATIONS----------

None.

----------WARNINGS AND PRECAUTIONS----------

- Infusion reactions: Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or interrupt administration. Re-start administration at a slower infusion once resolved.
- Hepatotoxicity: Closely monitor transaminase levels once per week for 3 months after HEMGENIX administration to mitigate the risk of potential hepatotoxicity. Continue to monitor transaminases in all patients who developed liver enzyme elevations until liver enzymes return to baseline. Consider corticosteroid treatment should elevations occur.
- Hepatocellular carcinogenicity: For patients with preexisting risk factors (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age), perform regular (e.g., annual) liver ultrasound and alpha-fetoprotein testing following administration.
- Monitoring Laboratory tests: Monitor for Factor IX activity and Factor IX inhibitors.

----------ADVERSE REACTIONS----------

The most common adverse reactions (incidence ≥5%) were elevated ALT, headache, blood creatine kinase elevations, flu-like symptoms, infusion-related reactions, fatigue, malaise and elevated AST.

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring at 1-866-915-6958 or FDA at 1-800-FDA-1088 or *www.fda.gov/medwatch*.

----------USE IN SPECIFIC POPULATIONS----------

No dose adjustment is required in geriatric, hepatic, or renal impaired patients.

Based on November 2022 version

HEMGENIX is manufactured by uniQure Inc. and distributed by CSL Behring LLC.
HEMGENIX® is a registered trademark of CSL Behring LLC.

www.CSLBehring.com www.HEMGENIX.com USA-HGX-0856-SEP24

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BEING A LATINO PARENT IN THE UNITED STATES: CONFLICTS, BELIEFS, TRADITIONS, FEARS, AND FAMILY DYNAMICS

BY LAURA ECHANDI

On February 25, The Coalition for Hemophilia B held a program in Spanish. It was part of their Foro Latino de Hemofilia B series. In this event, we hosted a powerful and insightful session exploring the unique experiences of Latino parents raising children in the United States.

The event, titled *Being a Latino Parent in the U.S.: Conflicts, Beliefs, Traditions, Fears*, and *Family Dynamics*, brought together families, caregivers, and community members affected by hemophilia B for an evening of connection, reflection, and emotional learning.

The discussion was led by guest speaker Carolina Castillo, a respected bilingual mental health professional with more than 17 years of experience. Licensed in both New Jersey (LPC) and New York (LMHC) and nationally certified as a Clinical Mental Health Counselor, Carolina has devoted her career to helping children, teens, and adults navigate mental health challenges and life transitions. Her compassionate, culturally informed approach created a safe space for honest conversation and mutual understanding.

Throughout her presentation, Carolina guided participants through meaningful discussions about balancing cultural traditions with U.S. parenting norms, managing the fears many immigrant parents face, and fostering healthy family communication. Using real-world examples and evidence-based tools, she encouraged families to honor their cultural roots while also adapting to new environments and expectations.

The event resonated deeply with participants, many of whom shared personal reflections and found comfort in realizing they were not alone in their struggles or hopes. Carolina's empathy and expertise helped families recognize that healing and growth often begin with open dialogue and shared experience.

The Latino Hemophilia B Forum continues to be a vital space for education, empowerment, and community-building. Events like this highlight that caring for emotional and cultural well-being is just as important as managing the physical aspects of living with a chronic condition.

Although this session has ended, its message continues to echo: through understanding, compassion, and community, families can find both healing and strength, together.

LEARN ABOUT HYMPAVZI™ SPONSORED BY PFIZER

BY ALLYSON KAMPS

Managing treatment for a bleeding disorder can sometimes feel like a full-time job, with infusions, schedules, and unexpected challenges along the way. To help community members stay informed and connected, The Coalition for Hemophilia B recently hosted two educational webinars sponsored by Pfizer.

Each session featured Brad Schoenfeld, Advocacy and Community Engagement Director, Pfizer, who led an informative discussion on recent developments in hemophilia care, followed by an open Q&A where participants could ask questions and share insights. Held on June 11 and July 9, the webinars created a warm and engaging atmosphere, thanks to Rocky and Marta, who brought energy, music, and fun while helping everyone feel welcome!

Thank you to Pfizer for sponsoring these educational sessions and for their continued collaboration with the hemophilia B community.

Pfizer

NAVIGATING THE HEMOPHILIA B TREATMENT LANDSCAPE

BY MARTA THOMAS

Overview Presentation and Sponsor Breakout Session

On May 20, 2025, The Coalition for Hemophilia B hosted an engaging virtual session exploring the current and emerging treatment landscape for hemophilia B. With so many new therapies now available, and more on the horizon, this program offered a timely and comprehensive overview to help families understand their options and feel empowered to make informed care decisions.

CSL Behring Breakout Session: Gene Therapy Discussion

The program began with a sponsor-led session presented by CSL Behring, featuring Vidhi Desai, MD, Medical Director. Dr. Desai led an in-depth discussion about HEMGENIX®, CSL Behring's FDA-approved gene therapy for adults with hemophilia B.

Dr. Desai explained how HEMGENIX® works as a one-time intravenous infusion that delivers a functional copy of the factor IX gene to the liver, allowing the body to produce its own clotting factor. She shared key data from the pivotal clinical trials, which showed that:

- 94% of participants achieved mild to normal factor IX levels following treatment.
- Most participants were able to discontinue their regular prophylaxis therapy.
- Patients experienced a significant reduction in annual bleed rates and a marked improvement in quality of life.

She also highlighted the importance of ongoing monitoring, noting that gene therapy requires long-term follow-up to track durability and safety outcomes.

Dr. Desai described HEMGENIX® Connect, CSL Behring's patient support program, which helps coordinate pre-treatment testing, home blood draws, and follow-up services to simplify the process for eligible patients. She emphasized that this therapy is not a fit for everyone, and that open discussion with a hematologist is essential before considering any gene therapy option.

During the Q&A segment, participants asked thoughtful questions about treatment eligibility, long-term outcomes, and what living with sustained factor production feels like. Dr. Desai encouraged anyone interested to engage in ongoing dialogue with their care teams and remain informed about new developments as data continues to evolve.

A Broad Overview of Treatment Options

Following the discussion, participants heard from by Dr. David Clark, Chairman of The Coalition for Hemophilia B, who guided participants through the full spectrum of hemophilia B treatment options, from long-standing therapies to cutting-edge innovations.

Dr. Clark began by revisiting the evolution of treatment, from standard and extended half-life factor IX products to non-factor rebalancing agents designed to help the body maintain clotting balance without directly replacing missing factor. He explained how these approaches can support different lifestyles and health goals, offering patients more flexibility and independence than ever before.

He also discussed gene therapy and its growing role in treatment, as well as ongoing research into gene editing and cell-based therapies that could shape the next generation of care. Dr. Clark's insights made complex science accessible, providing clear context for what's available now and what may soon be possible.
His central message resonated with attendees: while the expanding range of treatments is exciting, the most effective therapy will always be the one that best fits each person's individual needs, health goals, and lifestyle.

Education, Collaboration, and Empowerment

This two-part program underscored how important education and open discussion are in navigating an increasingly complex treatment landscape. Dr. Clark's comprehensive overview helped ground the conversation in the broader context of patient choice, while CSL Behring's presentation offered deeper insight into one of the newest therapeutic innovations available today.

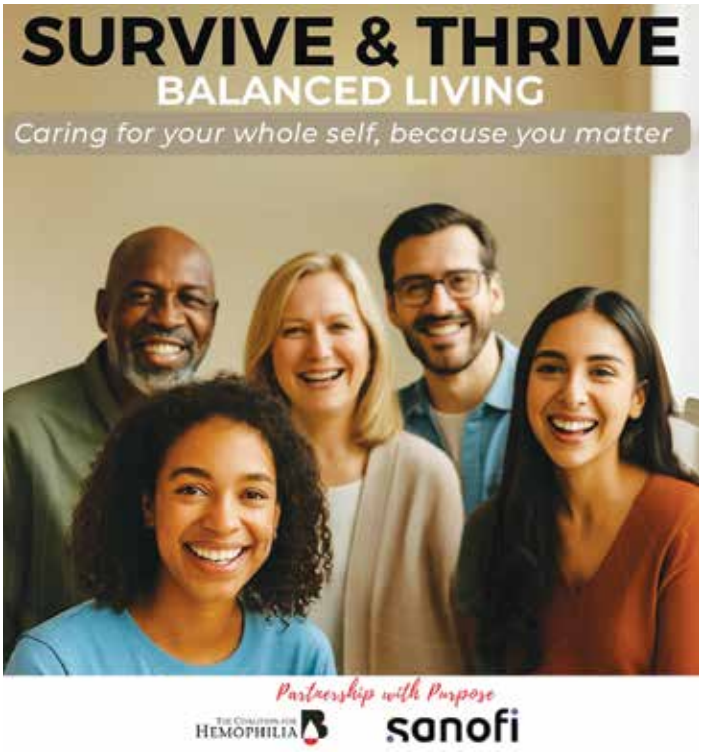
Together, these sessions reflected a shared goal: to ensure every individual living with hemophilia B has access to the information and support they need to make confident, informed healthcare decisions.

The Coalition for Hemophilia B extends its sincere gratitude to CSL Behring for sponsoring this event and for their continued partnership in promoting patient education, empowerment, and community engagement.

CSL Behring

SURVIVE & THRIVE

BY MARTA THOMAS



On June 3rd, community and connection took center stage during an inspiring Survive and Thrive educational session. Sponsored by Sanofi, the event brought members of our community together for an evening that was both informative and deeply meaningful.

The night began with Nicholas Cady, Sanofi CoRe Manager, introducing Qfitlia, a new treatment option for individuals with hemophilia B, with or without inhibitors. Attendees gained valuable insight into the product and the findings from its clinical trials.

The second portion of the program shifted focus to mental health and emotional wellness, led by Karen Boyd, LMSW, and David Rushlow, LMSW. Together, they created a thoughtful and inclusive space where participants could openly share their experiences and learn from one another.

Jaron, a community member, courageously opened the discussion by talking about his recent career transition and the grief he still carries for his late brother. His vulnerability opened the door for others to express their own stories. Participants talked about how they manage anxiety, practice mindfulness, and find comfort in daily routines. Coping methods included breathwork, prayer, music, exercise, and humor.

One memorable moment came when Melinda led the group in her "watch-your-watch jazz dance," a spontaneous and joyful movement that captured the spirit of the evening. Laughter and recognition filled the chat as attendees related to the symbolic rhythm of patience, pause, movement, and resilience.

Others shared stories about setting boundaries at work, advocating for their well-being, and navigating financial stress. These conversations reminded everyone of the strength within the community and the importance of mutual support.

This event served as a reminder that progress in health includes both physical and emotional well-being. The time spent together was filled with valuable information, heartfelt stories, and a collective sense of encouragement that will carry forward into the next gathering. Thank you to Sanofi for sponsoring such an empowering and heartfelt session.

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FORO LATINO DE HEMOFILIA B SPONSORED BY SANOFI

BY LAURA ECHANDI

"The community needs trustworthy, clear information in their language. Events like this help families feel supported and well-informed."
— Giraldo Gonzales, Community Education Manager

On Tuesday, June 17, 2025, Hispanic families joined a virtual educational session organized by The Coalition for Hemophilia B (CHB) an educational webinar sponsored by Sanofi was presented entirely in Spanish and focused on providing clear, accessible information about Sanofi's newly approved treatment option for hemophilia A and B, with or without inhibitors, for patients aged 12 and older.

The presentation was led by Giraldo Gonzales, Community Education Manager at Sanofi, who shared an overview of the therapy, including its administration method, clinical trial data, and practical considerations for patients and families. The event also featured an open question-and-answer segment, where participants could engage directly, ask questions, and share their own experiences managing hemophilia.

The high level of participation underscored how valuable it is for the community to receive reliable health information in Spanish, presented in a way that is both culturally relevant and easy to understand.

Through programs like this, The Coalition for Hemophilia B continues its mission to educate and empower all families affected by bleeding disorders, ensuring that every community has access to trustworthy, up-to-date information.

Special thanks to Sanofi for sponsoring this session and helping provide clear, accessible information for our Spanish-speaking community.

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CHB REPRESENTS AT FAMOHIO 2025

BY MATT MARLATT

My son, Nick, and I had the honor of representing The Coalition for Hemophilia B at the annual FAMOHIO event, hosted by the Southwestern Ohio Hemophilia Foundation from August 1–3 in Dublin, OH.

The weekend was filled with insightful speakers, key advocacy updates, and heartfelt conversations among families affected by bleeding disorders. It was especially rewarding to meet and connect with so many hemophilia B families from across the state, hearing their stories, sharing experiences, and finding common ground in our journeys.

A sincere thank you to the FAMOHIO Board of Directors for their warm welcome and for making CHB feel like part of this special and inspiring weekend.

ADVOCACY IN *ACTION*

BY MARTA THOMAS

Editor's Note: On July 4, 2025, after this article was written, H.R.1, the "One Big Beautiful Bill Act" was signed into law by President Trump after passing both houses of Congress.

On June 19, The Coalition for Hemophilia B (CHB) hosted a powerful advocacy session that left participants inspired and informed. The discussion focused on major legislation making its way through Congress the "One Big Beautiful Bill Act" and how it could affect critical programs like Medicaid, Medicare, the Affordable Care Act (ACA), and the Supplemental Nutrition Assistance Program (SNAP).

The session was led by Advocacy Consultants Lee Hall and James Romano, and Patrick Collins, with heartfelt storytelling from Sue Martin that helped everyone connect policy to real-life impact. Lee opened the discussion by breaking down what this proposed legislation could mean if passed. Millions of Americans could lose coverage or face major changes to these vital programs if the bill introduces stricter income checks, shorter enrollment windows, or increased paperwork requirements.

Jim then shared details about the Senate version of the bill, which adds even more challenges—like requiring individuals to work to stay on Medicaid and to recertify eligibility twice a year. He emphasized how these changes could directly affect the bleeding disorders community and encouraged everyone to contact their senators while their voices can still make a difference.

Patrick focused on the rare disease community, explaining how Medicaid cuts often limit access to essential treatments and therapies. For many, this could lead to skipped infusions or delayed care, creating serious health risks and long-term complications. His words hit home, reminding everyone that advocacy is not just about politics, it's about protecting lives.

Sue Martin shared stories from her advocacy work with families, including one about a young man named Chase, who spoke out about his experience with

Hemophilia B to help others, even though this bill wouldn't directly impact him. His courage reminded everyone that every story matters and that speaking up can inspire real change.

The session also introduced participants to CHB's new Advocacy Action Program, a powerful advocacy tool designed to make getting involved easier than ever. Through the Advocacy Action Program, community members can receive timely alerts and updates, send personalized messages to elected officials, and access important advocacy resources—all in one place. This program helps empower patients and families to stay informed, take action quickly, and build confidence as strong, effective advocates for themselves and the wider Hemophilia B community.

The evening wrapped up with an engaging Q&A, where participants shared their own advocacy stories, from visiting lawmakers to using social media to raise awareness. The chat was full of encouragement and energy, and the night ended on a high note with a raffle and inspiring closing messages.

The key takeaway? Every voice has power. Whether you're new to advocacy or already involved, your story matters, and together, we can make a difference. The Coalition for Hemophilia B's ongoing advocacy efforts, including the new Advocacy Action Program, are helping ensure that every member of our community has the tools, knowledge, and confidence to speak up, take action, and protect access to the care we all depend on.

STRENGTH IN CONNECTIONS:

MOVEMENT THAT MATTERS

BY MARTA THOMAS

On June 23rd, we were delighted to have Wayne Cook back and hosting. Members of our Strength in Connections community gathered for a refreshing and uplifting session with Douglas Stringham, MS, LAT, ATC, who led us through a program designed to remind everyone that movement doesn't have to be strenuous to make a difference, it just has to be intentional.

Doug's approach was all about simplicity and joy. He introduced the idea of "movement snacks", small, easy motions you can sprinkle throughout your day to release tension, reset your posture, and feel more energized. Whether you're at your desk, in the kitchen, or relaxing at home, these gentle reminders to move can help restore balance and comfort in both body and mind.

Doug guided us through stretches from head to toe, neck rolls, shoulder openers, spinal twists, and easy hip and ankle movements. He explained how posture and breathing are deeply connected to how we feel and function. The focus wasn't on perfection or performance; it was on awareness, self-kindness, and connection, to our bodies, our breath, and each other.

The atmosphere was calm yet full of life. Participants smiled, laughed, and shared small victories in the chat, from realizing how much looser their shoulders felt to discovering how a simple stretch could ease stiffness from sitting. Doug's friendly tone made everyone feel welcome, no matter their fitness level or physical ability.

After the guided session, the group stayed to chat about ways to bring more movement into daily life. Some shared how they dance while cooking dinner or stretch before bedtime as part of their evening wind-down. Others talked about taking short walks between phone calls or using favorite songs as reminders to stand and move. Each story was a small spark of motivation, proof that wellness doesn't come from giant leaps, but from consistent, joyful steps.

The conversation naturally evolved into a heartfelt exchange about staying healthy as we age. Participants opened up about dealing with pain, fatigue, and motivation, and how community helps them keep going.

One attendee shared, "It's not about doing more. It's about doing what makes you feel alive." There was laughter, empathy, and plenty of encouragement, the kind of warmth that only comes from shared experience.

As the session closed, Doug left everyone with a final reminder: "Your body is your lifelong companion. Treat it gently, listen to it often, and keep it moving."

We wrapped up with a few raffle spins, cheerful smiles, and the shared feeling that we'd all taken a small but meaningful step toward better well-being. Everyone left with something to carry forward, a stretch, a breath, or simply a renewed sense of gratitude for the strength we find in staying connected.

NEBRASKA FAMILY EDUCATION WEEKEND: A DAY OF LEARNING AND CONNECTION

BY CHAD STEVENS

On June 28, 2025, I had the pleasure of representing The Coalition for Hemophilia B at the Family Education Weekend in Lincoln, Nebraska, where I attended as an exhibitor. The event, sponsored by the Nebraska Chapter of the National Bleeding Disorders Foundation, brought together families, caregivers, and advocates for a day of learning, sharing, and community.

From the very start, the atmosphere was warm and welcoming. We began the morning with a hearty breakfast (complete with plenty of bacon!) and an interactive icebreaker that had everyone laughing, moving, and connecting with new friends. It set a positive tone for the day ahead.

The educational sessions were both engaging and informative, covering important topics such as joint health and nutrition, caregiver burnout, spotlight on siblings, pain management, and tools for self-advocacy. Each presentation offered practical takeaways and sparked meaningful conversations among participants.

After another great meal at lunch, attendees divided into men's and women's roundtables for open, thoughtful discussions. These smaller group settings gave everyone a chance to share personal experiences, exchange advice, and find encouragement from others who truly understand life with a bleeding disorder.

During the exhibit sessions, I had the opportunity to meet even more incredible families from across Nebraska. Their openness, warmth, and stories of resilience made the day especially memorable.

As the weekend came to a close, I packed up and prepared for an early drive to the Omaha airport the next morning, made unforgettable by a spectacular lightning storm lighting up the Nebraska sky.

I'd like to extend my sincere thanks to the Nebraska Chapter for their hospitality and for inviting The Coalition for Hemophilia B to take part in such a meaningful event. It was an honor to represent the Coalition and connect with so many inspiring members of the bleeding disorders community.

STRENGTH IN CONNECTION: MOVING, REFLECTING, AND LAUGHING TOGETHER

BY DOUGLAS STRINGHAM, MS, LAT, ATC

On July 8th, I had the privilege of joining, for the second time this summer, another inspiring Strength in Connection community gathering for adults aged 50 and older. Every time we meet, I'm reminded that these events are about so much more than movement or discussion. They're about joy, laughter, and the incredible energy that comes from being together.

Looking Back, Moving Forward

We began the evening with a thoughtful introduction from Jessie, representing our generous sponsor, Sanofi. Jessie invited everyone to pause and reflect on the past ten years, the moments that shaped us, the lessons we've learned, and how far we've come.

That short reflection exercise set the tone beautifully. People began sharing stories, some funny, some touching and the sense of community in the (virtual) room grew stronger by the minute. Taking time to look back helped us all see how much we've grown, both individually and together.

Posture: A New Perspective

Then it was my turn to lead the next part of the session, and I'll be honest, I knew the topic might raise a few eyebrows: posture. Now, posture doesn't exactly sound like the most exciting conversation starter. But I promised the group that by the end of the evening, they'd see it differently. Because posture isn't really about sitting up straight or chasing some perfect pose, it's about awareness.

Over time, life changes the way we move and feel. Our posture shifts constantly, even without us realizing it, hundreds of times a day, in fact. Instead of treating posture like something we have to fix, I encouraged everyone to think of it as something we can explore — to notice how we hold ourselves in different moments and how those small adjustments affect our energy and confidence.

So, we started simple — gentle movements, shoulder rolls, neck stretches, deep breaths — and a few shared laughs along the way. The goal wasn't perfection; it was connection. We moved together, supported one another, and discovered that posture tells a bigger story than just how we stand. It reflects our emotions, stress, and the experiences we carry.

Posture, Play, and Plenty of Laughs

As we kept going, something wonderful happened. Our conversation about posture turned into a lively exchange of stories. We started sharing "real-life posture moments" how we sit at the computer, cook dinner, or sleep (and wake up wondering what on earth happened to our necks!).

One participant cracked a perfectly timed joke about sleep positions, and before long, everyone was laughing. The kind of full, contagious laughter that makes your shoulders drop and your heart feel lighter. That's when I realized, this is what Strength in Connection is all about. Yes, we move our bodies, but we also move our spirits.

A Throwback to Fun and Friendship

After our movement and discussion, it was time for the ever-popular Throwback Trivia session, hosted by the one and only Rocky — the MVP of game night. Now, I'll admit, I didn't know all the answers (though I held my own during the Pink Panther section). But that didn't matter. What made it so much fun was the camaraderie, the teasing, the good-natured competition, and the shared memories that came up along the way.

Someone would shout out an old movie or song title, and suddenly we were swapping stories about where we were when we first heard it. For a few moments, we were all transported l, back to high school dances, road trips, and simpler times. That's the magic of these evenings. You don't have to know every answer to have a great time. You just have to show up, share a laugh, and let yourself enjoy the moment.

Why Connection Matters More Than Ever

When I think back on these sessions, what stands out most isn't any single exercise or trivia question, it's the feeling. Every time we gather, whether it's in person or

online, we leave with a little more lightness than when we arrived.

Sometimes, it's because we moved a bit and eased a sore muscle. Other times, it's because we said something that's been sitting on our minds or simply heard someone say, "Me too." By the end of the night, you can feel it, that quiet shift in the room when people realize they're part of something bigger than themselves. As we grow older, connection isn't just nice to have, it's essential. It keeps us vibrant, curious, and resilient. It reminds us that we're still learning, still laughing, and still very much alive.

Looking Ahead

If you haven't joined a Strength in Connection event yet, I can't recommend it enough. You'll stretch, you'll laugh, you'll reminisce, and you might even surprise yourself with what you discover.

These gatherings are a chance to move your body, lift your spirits, and be part of a community that genuinely cares. As one participant put it perfectly, "No matter what kind of day I've had, I always leave these events feeling lighter and happier."

A huge thank-you to Sanofi for making these moments possible and for supporting our ongoing commitment to the health and well-being of our mature adult community.

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LIVING WITH HEMOPHILIA B A CONVERSATION WITH DR. SIDONIO

BY MARTA THOMAS

On July 22, Novo Nordisk sponsored an educational virtual meeting for our community where Dr. Robert Sidonio led a meaningful discussion that gave space for honesty, vulnerability, and shared experiences. This session felt more like a heart-to-heart than a presentation. It was an open conversation between people living with hemophilia B and a doctor who truly understands.

Participants spoke candidly about the challenges of managing hemophilia B, including breakthrough bleeds, which can still occur even with regular treatment. Dr. Sidonio explained that many people with hemophilia B experience at least one bleed per year, and recovery can take several days, something that can disrupt routines, plans, and confidence.

Attendees also discussed how the unpredictability of bleeds impacts daily life physically, emotionally, and socially. From joint pain and canceled plans to the frustration of uncertainty, many shared that these setbacks can take a toll beyond just physical health.

Infusion routines were another major topic. Participants talked about the time and energy it takes to maintain their treatment plans, mentioning challenges such as scarred veins, fatigue, and the logistics of keeping up with supplies and travel. Dr. Sidonio encouraged everyone to stay in communication with their healthcare teams, emphasizing that as life changes, so do treatment needs, and it's always okay to speak up when something isn't working.

The conversation also looked toward the future, with participants expressing hopes for more personalized care, better access to treatment, and greater recognition of women with hemophilia.

The session wasn't just about medicine; it was about connection. It reminded everyone that they are part of a strong, understanding community where their voices matter. Whether participants shared their stories or simply listened, one message resonated throughout the evening: you are not alone. Thank you to our sponsor Novo Nordisk for making this event possible.

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NAVIGATING THE HEMOPHILIA B PRODUCT LANDSCAPE

BY ROCKY WILLIAMS

Overview Presentation and Sponsor Breakout Session

On July 24, 2025, The Coalition for Hemophilia B hosted another highly anticipated installment of its Navigating the Hemophilia B Product Landscape series. The evening brought together community members for an engaging and educational program designed to help families better understand the evolving treatment options for hemophilia B.

Novo Nordisk Sponsor Presentation: A Closer Look at Alhemo®

The evening began with a sponsor presentation by Novo Nordisk, led by Jonathan C. Roberts, MD, who introduced Alhemo®, the company's new treatment option for hemophilia B.

Dr. Roberts explained how Alhemo® works, shared highlights from clinical trials, and demonstrated the prefilled pen delivery system, designed to make treatment administration more accessible and user-friendly. His presentation sparked thoughtful questions and genuine excitement among attendees.

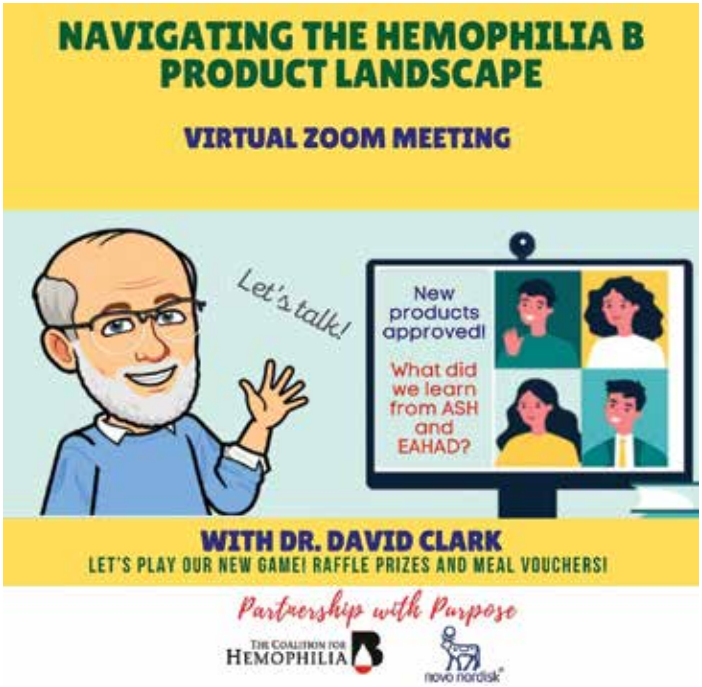
As Dr. Roberts reminded everyone, "You are your own patient advocates, and you're asking the right questions."

Educational Overview: Understanding the Hemophilia B Treatment Landscape

The second part of the evening featured a comprehensive presentation by Dr. David Clark, Chairman of The Coalition for Hemophilia B, who provided a clear overview of today's hemophilia B therapies and what lies ahead.

Dr. Clark discussed the range of available treatments, from shorter half-life products to rebalancing agents, gene therapy, and subcutaneous treatments, along with other emerging innovations that continue to reshape care for people living with hemophilia B. His ability to make complex science accessible helped participants feel confident and informed about the growing number of options available to meet individual needs and lifestyles.

Dr. Clark emphasized that while innovation continues to expand the treatment landscape, personalized care



remains essential, the best treatment is always the one that fits each person's goals, health profile, and daily life. Audience members expressed their appreciation throughout the chat:

"Always an informative presentation, Dr. Clark!"
"Great as always!"

"Thank you, Dr. Clark. I always enjoy your presentations."

Community Connection and Engagement

To close the evening, attendees joined the always-popular *Are You Smarter Than Your Hemophilia B?* trivia challenge, a fun and interactive way to reinforce learning from the sessions. Laughter, connection, and lighthearted competition filled the virtual room as participants tested their knowledge and celebrated each other's wins.

The Coalition for Hemophilia B extends sincere thanks to Novo Nordisk for sponsoring this educational event and for their continued partnership in supporting the hemophilia B community.

Programs like this exemplify the Coalition's mission to empower patients and families through education, connection, and advocacy, ensuring that everyone has access to clear, trustworthy information about their care.

We look forward to seeing you at our next session, ready to learn, share, and grow together!

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ROOTED IN CONNECTION: CHARLOTTE FAMILY MEETING ON THE ROAD INSPIRES STRENGTH, KNOWLEDGE, AND COMMUNITY

BY ERICA GARBER

There was something truly special about being together in Charlotte, North Carolina, on August 16th for our 2025 Family Meeting on the Road. Held at the Fairfield Inn & Suites Charlotte Uptown, the event welcomed families from across the region for a day filled with connection, learning, and renewal. From morning movement to heartfelt discussions, the day invited participants to slow down, reflect, and rediscover the power of community.

Starting with Joy and Movement

The day began with music, laughter, and an uplifting sense of energy thanks to Dr. Robert L. Friedman, Ph.D., an internationally recognized speaker, therapist, and long-time Coalition collaborator. In his dynamic session, *Shake, Rattle & Roll*, Dr. Friedman led participants through rhythmic exercises and playful movement designed to release stress and build group connection.

It was more than an icebreaker, it was a joyful reminder that healing isn't only found in medicine or routines, but also in the freedom to laugh, move, and share space with others who understand. The room came alive with rhythm and smiles, setting the perfect tone for the day ahead.

A Space to Be Heard

That spirit of openness flowed naturally into the first rap session, led by Kevin Harris, CNC, BCS, SFC, ARC Instructor, WFR, a member of the hemophilia community and co-founder of Trails to Healthy Living. Through guided questions and honest storytelling, Kevin created space for participants to share their experiences, what living with hemophilia B means to them, and how they navigate challenges as individuals and families.

For many, they felt truly heard. The conversations were filled with empathy, reflection, and encouragement, a testament to the power of being among people who "get it."

Learning, Resources, and Connection

Between sessions, attendees visited the exhibit hall, where they connected with our partners and supporters. Families explored educational resources, discovered new services, and asked

thoughtful questions about treatment options. These conversations helped empower participants with knowledge and confidence to manage their care. Many families left feeling more informed and hopeful about their next steps.

Exploring the Future of Treatment

One of the day's most engaging sessions focused on gene therapy. C.J. Hansen, BSN, RN, of The Ohio State University's Hemostasis and Thrombosis Center, joined Michael Joshua, a 23-year-old who received HEMGENIX® in 2023, for an honest, insightful discussion about the evolving landscape of gene therapy.

Together, they explored both the science and the lived experience, what it means to receive gene therapy, how life can change afterward, and what emotional adjustments may follow. Attendees appreciated hearing from both a medical professional and a patient, making this complex topic accessible and deeply personal. The conversation sparked thoughtful questions about the future of treatment and the hope that innovation brings to our community.

Advocacy and Action

Next, Bobby Wiseman and Lee Hall led a powerful session on urgent policy issues affecting the hemophilia B community.

From shifting insurance coverage to proposed state-level changes that could affect access to care, their discussion helped participants better understand the legislative landscape. Most importantly, attendees learned practical ways to get involved—writing to representatives, sharing personal stories, or joining advocacy coalitions. Their message was clear: when we raise our voices together, we can create real, lasting change.

Living Fully with Hemophilia B

In his deeply personal session, *The Road Less Traveled*, Dale Bodyk shared his reflections on living with hemophilia B. His words centered on choice, not in having the condition, but in how one responds to it.

Dale spoke candidly about resilience, preparation, and finding purpose. He reminded everyone that while we can't always control our circumstances, we can choose our attitude, our community, and our courage. His talk was moving, authentic, and filled with hope.

Caring for the Mind and Spirit

The day's emotional heart came during *Stronger Together*, led by Dr. Whitley Grant-Goodman, who explored the emotional and mental side of living with hemophilia B. Her compassionate approach helped participants reflect on how meaningful connection—within families, among care teams, and across the








broader community, can reduce isolation and foster healing. Using the A.S.K. framework, she shared tools to build empathy and communication, reminding everyone that mental health is just as vital as physical health.

Attendees left feeling uplifted, understood, and equipped with new strategies for emotional well-being.

Empowerment in Motion

In the afternoon, Kevin Harris returned to lead *Control Your Narrative*, an energizing session that combined movement, mindfulness, and empowerment.

Participants explored how daily habits, how we move, speak, rest, and respond, shape our mental and physical health. Through open discussion and gentle physical challenges, Kevin encouraged everyone to reclaim a sense of agency in their lives.

It was a powerful, hands-on reminder that even small, intentional choices can add up to meaningful transformation. The outdoor setting added a sense of renewal and freedom that perfectly reflected the theme of the day.

Engaging Young Minds

While the adults were in sessions, children and teens enjoyed a day designed just for them. The youth program blended creativity, learning, and adventure, culminating in an exciting excursion to Discovery Place Science.

Hands-on activities and new friendships made it a memorable experience. Parents expressed gratitude knowing their children were not only having fun, but also learning in a safe, inclusive space that reflected the same spirit of community as the main sessions.

Honoring Our History, Inspiring Our Future

The final sessions brought the day full circle. In *Our History, Our Story*, Matthew Barkdull, MS, MBA, LMFT, MedFT, traced the remarkable evolution of hemophilia B care reminding us how far we've come through science, advocacy, and perseverance. His reflections offered perspective and pride in the progress made by generations before us.

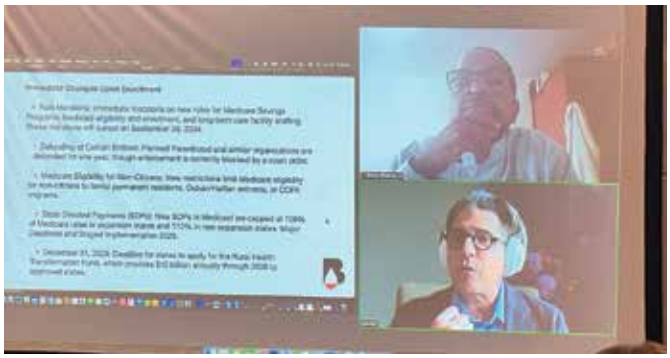
The closing session, *B-Amazing: We Are in This Together*, led by CHB Executive Director Erica Garber, MA, Ed.M., brought a joyful close to the day. Through lighthearted games and thoughtful reflection, Erica helped participants celebrate the day's lessons and carry its spirit forward. Laughter and gratitude filled the room as everyone left feeling renewed, connected, and proud to be part of something bigger than themselves.

Gratitude and Looking Ahead

We are deeply grateful to CSL Behring for their generous support of this event. Their continued partnership makes it possible for families impacted by hemophilia B to come together, learn from experts and one another, and strengthen the bonds that keep our community thriving.

CSL Behring

For upcoming dates and registration details, visit hemob.org/events.



CHB REPRESENTS AT THE 2025 NATIONAL BLEEDING DISORDERS FOUNDATION CONFERENCE

BY ROCKY WILLIAMS

From August 21–23, the National Bleeding Disorders Foundation (NBDF) held its annual conference in Aurora, Colorado, The Coalition for Hemophilia B team was proud to attend and represent our community throughout the weekend.

One of the highlights of the conference was the awards ceremony, where leaders and changemakers from the bleeding disorders community were recognized for their exceptional contributions. Honorees included Ryan Crowe, recipient of the *Loras Goedken Leadership Award*; Ray Stanhope, honored as *Lived Experience Expert of the Year*; Makenzie Sledd, PT, DPT, named *Physical Therapist of the Year*; and Antonio Jenkins, celebrated as C*hapter Volunteer of the Year*. Their dedication and passion continue to inspire all of us who work to strengthen and support this community.

We are deeply grateful to everyone who stopped by The Coalition for Hemophilia B booth during the conference. It was wonderful reconnecting with old friends, meeting new faces, and celebrating the unity, resilience, and spirit that make this community so strong.

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UPCOMING EVENTS

For more information and to register: hemob.org/events

EVERY MONDAY 1-2 PM EST
Mental Health Mondays in the B-Hub
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OCT 27, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

Espaniol
NOV 4, 2025 • VIRTUAL
Foro Latino de Hemofilia B
Potenciar las voces latinas en la hemofilia B

NOV 7 - 10, 2025
BOULDER CREEK, CA
Generation IX Project:2025 Redwoods Leadership Program
Leadership in the Redwoods in partnership with GutMonkey

NOV 13, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

NOV 18, 2025 • VIRTUAL
Survive & Thrive, Mental Health
Tools for Wellness,
Support in Community

NOV 19, 2025 • VIRTUAL
B-Leaders Teen Virtual Event
Teen Virtual Rap Session
Camp; Game Night

NOV 24, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

DEC 3, 2025 • VIRTUAL
Hemophilia B Product Landscape
Understand your treatment options with Dr. David Clark!

DEC 6, 2025 • VIRTUAL
Gingerbread House Decorating + Trivia Night
Join us as our community gathers to decorate gingerbread houses and enjoy fun games together!

DEC 9, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

SHARE YOUR STORY

Are you ready to share your story and help others? Whether you have an incredible career, an extraordinary family, or a tale of triumph, we want to hear from YOU! You will collaborate with an in–house writer to help you communicate your story in a compelling and meaningful way. The best part is that no previous writing experience is necessary! To add your voice and share your insights with The Coalition for Hemophilia B, please contact us at contact@hemob.org.

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>
> SHANNON L. ALDER

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- The program does not cover medical bills.
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We are here to stand beside you when life feels overwhelming, offering help and hope in the moments when it matters most.

The Coalition for Hemophilia B is a national nonprofit serving the hemophilia B community for more than 35 years.

LEARN MORE: https://www.hemob.org/financial-assistance
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THE COALITION FOR HEMOPHILIA
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THE COALITION FOR HEMOPHILIA

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For information, contact Kim Phelan, 917-582-9077, kimp@hemob.org

SURVIVING AND THRIVING WITH HEMOPHILIA B: A TEEN EVENT

BY DANNY

On June 24, 2025, Rocky and I hosted an awesome teen event called Surviving and Thriving with Hemophilia B. It was seriously one of the best experiences ever! We got to hang out with other teens who totally get what it's like to live with hemophilia B, and we shared tips, stories, and a lot of laughs.

One of my favorite parts was the icebreaker, it wasn't just a typical "say your name" kind of thing. It turned into a real conversation about how hemophilia B affects our lives and how we deal with it every day. Everyone jumped in with advice and encouragement, and it felt more like talking with friends than anything else.

The rap session with Matt Barkdull was another highlight. He brought so much energy and asked the best questions, it was super fun to answer them and hear everyone else's stories. At some point, I forgot I was even hosting because I was so into what people were saying!

And the games, oh man, the games were epic! We played *Survive the Internet*, which was hilarious. Seeing everyone's creativity (and sense of humor) made the whole event even better. The trivia was also awesome, it wasn't the boring kind you expect, but the kind that actually makes you think and laugh at the same time.

Planning the event was almost as fun as hosting it. Rocky was the best co-host ever, he helped out whenever I needed it and kept the vibe positive. I had a lot of freedom to make it my own, which made it feel really special.

Honestly, hosting this event was one of the coolest things I've done with The Coalition for Hemophilia B. I'd do it again in a heartbeat!

If you're a teen in the program, you have to try hosting one of these events, it's a total blast from start to finish!

MEET JACK: LIVING LIFE ONE SWIM STROKE, BEAT, AND BITE AT A TIME

BY SHELLY FISHER

Jack is 13 years old and already living life to the fullest! Between swim team, water polo, music, and cooking, this soon-to-be 8th grader proves that hemophilia B doesn't hold him back, it just makes him stronger.

When we met, Jack was in the middle of his summer, getting ready to be the "big man on campus" next year. He was excited about everything ahead: water polo, swim meets, and The Coalition for Hemophilia B's Beats Music Program in Nashville, TN. Jack says he's a big fan of water polo, where he usually plays the wing position. "It's great exercise, and I like playing with my teammates," he shared. "We all rotate between offense and defense depending on who has the ball."

He's also on a swim team this summer and says his favorite stroke is the backstroke. With two water polo practices a week, weekend scrimmages, and swim meets, Jack was ready to spend a lot of time at the pool, and he couldn't be happier about it.

Outside the water, Jack's love of music shines. He plays the trombone and was pumped to be part of the Beats Music Program this summer. A big fan of rap, he was excited to bring his own style and sound to Nashville.

Jack's also looking forward to his school trip to Washington, D.C., next year. One of his favorite classes is Spanish. "My teacher made it really fun and interactive," he said, even showing off a few conversational phrases during our chat.

On the weekends, Jack likes to kick back with a good John Wick movie or a few rounds of video games. But what surprises most people is that he's also an amazing cook. Breakfast is his specialty, especially his crispy egg, bacon, cheese, and tortilla wraps. "You just crisp it up!" he said proudly. He even made beef wellington for Christmas dinner after seeing it on one of his favorite YouTube channels. "I thought, why not try it?" And according to his mom, it turned out perfectly.

Jack describes himself as "tough, a bit brave, and maybe a little wild," and that confidence comes from everything he's overcome. Diagnosed with moderate hemophilia B when he was just four weeks old, Jack has had his share of challenges, his first bleed happened at seven months, and he's been through infections, allergic reactions, surgery, and even a couple of broken bones. "I've been around a lot of needles,

a lot more than I'd like," he said. When he was five, he got a port, which made treatments easier and gave him peace of mind. Through it all, Jack has learned how to take care of himself and keep doing what he loves.

When asked how he'd support a teen who was newly diagnosed, Jack didn't hesitate:

"Get involved with The Coalition for Hemophilia B and your local chapter! There are lots of people who have this, and they can really help you understand it and not feel alone."

Jack knows this from experience. He's attended The Coalition for Hemophilia B's symposium in Orlando, several virtual teen events, and an On the Road event in St. Louis. "I enjoyed meeting new friends there and I still stay in touch with them," he said. "I also learned some tips to get veins." One of his favorite memories? "Doing late-night magic tricks with Rocky!"

Jack says The Coalition's symposiums and events have had a major impact on his life. "They've helped us through some tough times," he said.

Jack and his mom also enjoy volunteering and giving back to the community that's supported them along the way. "It's nice to help others who are going through the same thing," Jack said.

When asked who he's most grateful for, Jack smiled and didn't miss a beat:

"My mom. She's been there every time I'm in the hospital, always talking to the doctors about what to do next."

Through it all, Jack and his family try to stay positive and grateful, no matter what comes their way. Jack's upbeat attitude and fearless spirit make him a perfect example of what it means to live life to the fullest with hemophilia B. Whether he's swimming, cooking, or jamming out in Nashville, Jack is showing the world that anything is possible when you dive in with heart, humor, and courage.

MEET KELLEN: A GIFTED TENOR WHO FOUND HIS VOICE

BY SHELLY FISHER

Kellen is 14, funny, talented, and not afraid of the spotlight. Whether he's belting out songs in choir, crushing home runs on the baseball field, or jamming on his electric guitar, this soon-to-be high school freshman is living proof that having Hemophilia B doesn't stop you from finding your rhythm in life.

When we talked, Kellen was relaxing at home, surrounded by sunlight and summer vibes, getting ready to start ninth grade. He's been singing as a tenor for three years and can't wait to join his high-school choir. "I don't really have a favorite type of song," he said. "I just like singing what my director picks, it's always something different." After making the cut for his school's select choir and performing in competitions, Kellen discovered that he wasn't just good, he was gifted.

Next year, he's looking forward to harder classes (minus the dissecting in science labs, "not my favorite part," he admitted with a laugh). He's also hoping for more hands-on projects like the rocket lab he loved in middle school.

Outside of school, Kellen's got a serious love for sports. He's been playing baseball for as long as he can remember and still treasures the team ball he got after hitting a home run at age 12. "Everyone ran out of the dugout to congratulate me, it was awesome," he said. These days, he's taking golf lessons too and can often be found at his local course working on his swing.

But music is where Kellen really shines. Along with choir, he plays the electric guitar and even earned a spot in The Coalition for Hemophilia B's Beats Music Program in Nashville.

"Going to the Grand Ole Opry was so cool," he said. "The acoustics were amazing, clear and crisp, not loud or harsh like some places."

Kellen's also got a great sense of humor. "My friends would probably say I'm funny because I tease them a lot," he said. (Rumor has it he may have also teased a teacher after a memorable ski-trip wipeout.)

Kellen was diagnosed with hemophilia B when he was just five months old, after doctors discovered bleeding in his brain. He had surgery to relieve the pressure and began treatment right away. His mom learned everything she could about hemophilia B while he received daily factor infusions through a port during recovery. Later, testing revealed that his mom is a carrier, but Kellen remains the only one in his family with the condition.

When Kellen and his family attended The Coalition for Hemophilia B's Symposium in Florida, everything changed. "It felt like finding a second family," he said. "I kind of found my voice there, and it meant a lot to meet other people with hemophilia and get to know them." He says if he met another teen who was just diagnosed, he'd tell them:

"It's gonna be okay. You can do all the things, just take your factor."

During their time in Florida, Kellen and his dad joined the Let's Play Nine Golf Outing, hosted by The Coalition for Hemophilia B, that gave them the chance to hit the course together. After the symposium wrapped up, the family added an extra day to their trip to visit SeaWorld, where Kellen fearlessly tackled roller coasters and water rides (though his mom skipped a few of the scarier ones). "Getting everyone on the water ride together was definitely a highlight," he said.

After the symposium, Kellen didn't stop using his voice. He joined his mom at the state capitol to meet legislators and share what life with hemophilia B is really like. Their efforts helped lead to a local proclamation officially declaring March as *Bleeding Disorders Awareness Month* in their community.

Kellen says his parents are his biggest supporters. His mom is the family researcher and advocate, while his dad keeps things light with humor and a shared love for Jim Carrey movies, especially Dumb and Dumber. "My dad always knows how to make me laugh," Kellen said.

When asked what song he'd choose as his walk-up music for baseball, Kellen didn't hesitate:

"*Springsteen* by Eric Church. It just fits, it's like the soundtrack to my summer before high school."

As the lyrics say,
"Funny how a melody
sounds like a memory, like a
soundtrack to a July Saturday
night."

For Kellen, that melody is his life, full of music, laughter, courage, and finding his voice, both on stage and off.

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Summer 2025

IN THIS ISSUE:

MEET KELLEN!

MEET JACK!

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 11–18 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 rockyw@hemob.org for your next steps!