THE HEART OF A MOVEMENT

35 Years of Strength, Service and Community

THE COALITION FOR HEMOPHILIA B

35 ANNIVERSARY

Dave Kim Wayne

For the Community. With the Community. Because of the Community.

WINTER 2025

HEMOPHILIA B NEWS

WINTER 2025

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FLIP OVER!

THE COALITION FOR HEMOPHILIA B

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.


Annual Symposium 2025

THE HEART OF A MOVEMENT: A LEGACY OF DETERMINATION, FAMILY, AND LOVE

BY TERRY STONE

The Coalition for Hemophilia B is celebrating a remarkable milestone — 35 years of supporting patients and families diagnosed with Factor IX deficiency across the country! In this celebration, we honor the relentless spirit, compassion, and vision that brought the Coalition to fruition. What began as one family's fight quickly transformed into a community triumph.

Hemophilia is a rare bleeding disorder that affects approximately 33,000 people in the U.S. Even rarer is hemophilia B, also known as Factor IX deficiency or Christmas Disease, which affects about 7,000 individuals. This condition occurs when someone is missing or has low levels of the Factor IX protein. Although there was suspicion of a condition similar to hemophilia A, Factor IX deficiency wasn't identified and named until 1952, when British researchers from Oxford University, Dr. Robert Macfarlane and Dr. Rosemary Biggs diagnosed a young boy living in Canada named Stephen Christmas.

John Taylor

IT ALL BEGAN WITH A DIAGNOSIS AND A DETERMINED FATHER

When John and Joyce Taylor's son was diagnosed with severe hemophilia B, they were shocked by the lack of information available. Joyce's father, a pharmacist, had unknowingly lived with mild hemophilia. In his 60s, he was given aspirin in the hospital while suffering from the flu, which resulted in uncontrolled internal bleeding and ultimately led to his death. This tragedy served as a wake-up call for the Taylors, highlighting just how serious and overlooked hemophilia B could be.

Determined to make a difference, John made it his mission to gather as much information as he could. He traveled across the country and around the world, meeting with scientists, hematologists, and researchers in the field of bleeding disorders. He aimed to share the most current and credible information with other families through a patient-focused newsletter, which eventually expanded from a single sheet to a 70-page publication viewed both nationally and globally. This effort culminated in the creation of The Coalition for Hemophilia B.

Kim Phelan

THE HEART OF THE CHAIN REACTION

Initially on her way to becoming a trader, Kim Phelan took a leap of faith and accepted a job at John's small, quiet upstart financial firm. For someone as outgoing and naturally talkative as Kim, the shift was unexpected, yet something about it felt like a calling she couldn't ignore.

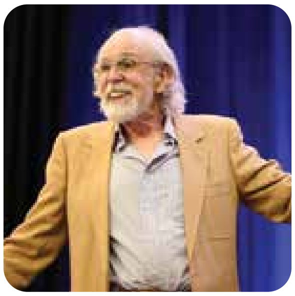
When John founded the Coalition in 1990, he invited Kim to join the board. What began as an opportunity quickly became a lifelong path, one rooted in purpose, service, and deep connection to the community. Serving as CEO, Kim often says she didn't choose the Coalition; the Coalition chose her.

Raised in a family that valued giving back, Kim began volunteering at a young age, keeping elderly neighbors company, spending time with seniors in nursing homes, and helping raise funds for multiple sclerosis research. As she grew older, that spirit of service only deepened. She worked with mentally challenged youth, babysat, and walked dogs, For five years, she also led an evening anxiety and phobia peer support group, offering others a safe space to be seen, heard, and supported, an experience that further shaped her compassion and care.

Kim was profoundly affected by what she witnessed at national events, families devastated by the HIV/Hep C crisis, parents forced to sell their homes, and patients left without support. In the early days, her family gathered around the kitchen table, sealing envelopes for the newsletter, knowing each one carried information that could change a life.

Today, the Coalition has grown nationally, but its heart remains the same, rooted in family, compassion, and an unwavering commitment to those it serves.

The family of William Drohan, for whom the CHB Memorial Scholarship is named, with CHB founder, John Taylor, 2008



Dr. David Clark

FROM SCIENCE TO SERVICE

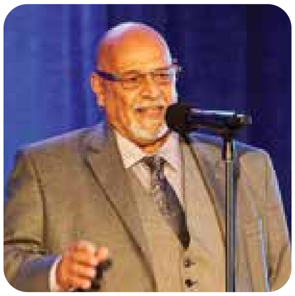
In 2006, John Taylor stepped down. His departure marked a pivotal moment in the Coalition's history one that required steady leadership and a clear vision for the future. Having been with the Coalition since its founding, Kim Phelan guided the organization through this important transition. She appointed Dr. Clark as Chairman of the Board, ushering in the next chapter with purpose, clarity, and passion.

A senior scientist with a Ph.D. in Chemical Engineering, Dr. Clark was part of Dr. William Drohan's lab research team at the American Red Cross. Together, they contributed to a pioneering effort to develop one of the first highly purified factor IX products. While that specific product did not ultimately reach market, the work itself helped advance the field in meaningful ways.

Dr. Clark also brought a rare and invaluable gift: the ability to translate complex clinical science into language that patients and families can truly understand. From the Coalition's earliest days, he has been a trusted voice in its newsletter, helping to guide the community through treatment decisions with clarity and confidence.

Wayne Cook

A VOICE BECOMES A LEADER



In 1992, Wayne Cook called Kim and asked, "What can I do? I want to help." A beloved and respected member of the hemophilia B community, Wayne was already well-known; however, this call marked the beginning of a powerful partnership.


Annual Symposium 2014


First Men's Retreat 2013


First Meetings-on-the Road 2011


First Women's Retreat 2015

As someone with severe hemophilia, Wayne brought lived experience, deep compassion, and fierce dedication to the organization. He connected with patients on a personal level and became a constant presence in their lives. Previously the president of his local hemophilia chapter, Wayne would begin serving as President of the Coalition in 2006, bringing the community's voice to every level of leadership.

With John Taylor's departure, the Coalition was left in capable hands. Together, Kim, Wayne, and Dave formed a trio that became the heart and soul of the Coalition, exemplifying leadership defined by compassion, commitment, and genuine care.

With a dedicated staff and enthusiastic volunteers, the trio creates programs that enrich our community and foster positive change. Together, the organization works tirelessly to achieve its goals and make a difference in the lives of those they serve.

Power of Progress

THE FIRST SYMPOSIUM

The first Coalition Symposium in 2007 brought together just over 100 people. Today, it hosts more than 1,000 attendees, including patients, caregivers, medical experts, and industry leaders. This growth is a testament to the Coalition's dedication to serving every patient with hemophilia B, from newborns to veterans. Kim recalls the early days when the exhibit hall was full of adults using crutches, scooters, or wheelchairs, their joints damaged from years of inadequate treatment.

2023

"Today, you see people dancing, running, and living full lives," she says. "It's heartwarming to see how treatment advances have truly changed lives."

At the symposiums, it's not just about the science; it's about human connection. Kim shares, "Wayne brings the warmth; he makes you feel like family. Dave brings the knowledge and the patience to answer a million questions. And me? I want people to have the best quality of life, and I am committed to doing everything I can to help."


2013


2014

The Pandemic Pivot

RESILIENCE IN ACTION

The COVID-19 shutdown occurred just one week before the 2020 annual symposium. The Coalition had to pivot quickly. The team enrolled in what they now affectionately call "Zoom School" to learn how to move educational programming online. Their first attempt with an outside virtual vendor crashed on opening

night, forcing the team to manage the chaos in real time. This experience was eye-opening and inspired the team to build their own online platform.

Today, the Coalition continues to offer both in-person and virtual programming, removing barriers to access and ensuring all patients, whether unable to travel, hospitalized, or homebound, can still connect and learn.

A Health Scare Hits Home

WAYNE'S JOURNEY

Year 35 also brought unexpected challenges. Wayne Cook, who contracted hepatitis C during the crisis years of contaminated blood products, experienced life-threatening complications. He needed a liver transplant, but also had a cardiac blockage, making surgery incredibly high-risk. Wayne underwent back-to-back surgeries in January and miraculously pulled through. He recently walked his daughter down the aisle on her wedding day.

Although his immune system remains fragile and he is not yet able to attend in-person events, he joins programs via FaceTime, bringing his trademark love and strength. "We all miss him deeply," Kim states, "but we know he'll be back with us soon."

Coalition Programs

FOR EVERY STAGE OF LIFE

Over the years, the Coalition has expanded its offerings to address the evolving needs of the community, which now include:

- **Patient & Family Support Programs**
  - ☆ Patient Assistance Program
  - ☆ Insurance Navigation Support
  - ☆ Mental Health & Wellness Workshops
  - ☆ College Planning Resources
  - ☆ B Education Hub (including multilingual resources and glossary)
- **Scholarships & Financial Aid**
  - ☆ William N. Drohan Educational Scholarship
  - ☆ Additional Named Scholarships
  - ☆ Travel and Conference Assistance Grants
- **Community Connection & Life Stage Programs**
  - ☆ Annual Symposium
  - ☆ On-the-Road Family Meetings
  - ☆ New Moms Program
  - ☆ Empty-Nester Conversations
  - ☆ Aging with Hemophilia Support Groups
  - ☆ Youth Camps & Leadership Retreats
  - ☆ Men's and Women's Empowerment Retreats
- **Leadership & Advocacy Development**
  - ☆ Teen and Young Adult Leadership Programs
  - ☆ Advocacy Training and Retreats
  - ☆ Patient Advisory Boards and Opportunities

Inaugural *The Beats Music Program*

One of the Coalition's most innovative programs is *The Beats Music* Program, which helps community members bond through rhythm and creativity. Kim explains, "Through music, we create a safe, fun space, and the relationships carry over into other programs."

B Voice

COALITION'S ADVOCACY IMPACT

Patients need timely, clear information about changes to insurance, Medicaid, Medicare, and other vital healthcare updates. The Coalition has gone above and beyond to stay on top of these developments and ensure that patients are informed and empowered to act.

To support this, the Coalition has created an Advocacy Action Sheet, a very popular and widely shared user-friendly resource filled with timely updates and several proactive steps patients can take. The organization hosts educational sessions that teach individuals the many ways to advocate, whether through storytelling, video, social media, or direct outreach. Everyone can find their voice and their own way to make a difference.

Kim notes, "We even launched a postcard campaign, giving patients the tools to contact their legislators directly. And we've remained active on Capitol Hill, meeting with congressional offices, writing letters, and working alongside over 100 organizations to unify our voices across the rare disease community."

To further support patient empowerment, the Coalition recently invested in a new advocacy platform designed to make it even easier for patients to engage, take action, and stay informed.

Kim shares, "With all the changes happening, it's even hard for us to keep up." That's why it's more important than ever to help patients understand what's going on. It's important to stay informed, be proactive, and gain confidence in the ability to make a difference; and we want them to know, we are here for them every step of the way.

Mental Health

OUR SHARED JOURNEY AND COMMITMENT

"Wayne, Dave, and I have all experienced anxiety or depression at different points in our lives. We believe that taking care of your mind is just as essential as taking care of your body, as the two are deeply connected."

The team strongly believes that struggling with any mental health challenge does not define your strength, intelligence, or abilities; it's simply a barrier that deserves attention, healing, and support. When physical health issues challenge a person's body, it is only natural that their mental health can also be affected. The Coalition is deeply committed to ensuring that every program it offers includes meaningful support for emotional well-being in some way, shape, or form. The Coalition is firm in its mission to break down the stigma and eliminate the barriers that prevent people from accessing the support they need.

Women Bleed Too

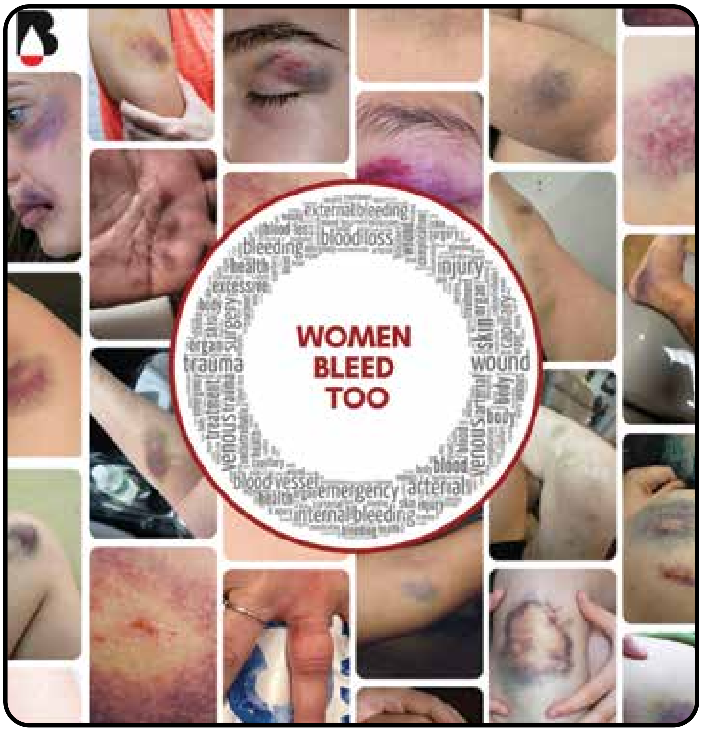
A CALL FOR RECOGNITION, DIAGNOSIS, AND CARE

The Coalition is dedicated to ensuring that women and girls with bleeding disorders are correctly diagnosed and treated. For too long, their symptoms have been dismissed, misdiagnosed, or minimized, often leading to devastating physical and emotional consequences. This issue is not just about heavy periods or bruising; undiagnosed bleeding can lead to joint damage, loss of mobility, chronic pain, and mental health struggles. In some instances, such as childbirth or car accidents, a lack of diagnosis can have disastrous results.

Dr. Clark has long advocated for awareness, writing clear and scientifically grounded articles to educate the community and healthcare providers. However, information alone has not been enough. Kim poses a crucial question: "Why, in this day and age, must females wait so long for a proper diagnosis? If words aren't going to change things, then we must show them the truth. We needed to make the invisible visible." This vital question gave rise to the Coalition's campaign: *Women Bleed Too*.

Over the years, the Coalition has quietly gathered countless heartbreaking stories and photos of women in our community, covered in bruises and swollen joints, suffering in silence. The injuries are real, the pain is real, and denial can be deadly.

The Coalition compiled the photos into a striking postcard designed to confront people with the reality



of undiagnosed bleeding in women. It was posted on social media, shared at the symposium, and sent in a mailing campaign. Although it may not be easy to look at, its impact is impossible to ignore. While the campaign is titled *Women Bleed Too*, it also applies to young girls and teens who bleed, many of whom grow up believing that their bleeding and pain is normal, or worse, imaginary.

The Coalition is proud to see the bleeding disorders community coming together around this issue, but the work is far from over. Kim emphasizes, "We will keep speaking out, we will keep showing the truth, and we will not stop until every affected woman and girl is diagnosed, treated, and supported with dignity and understanding."

Looking Ahead

OUR COMMITMENT CONTINUES

As the Coalition looks toward the next chapter, its mission remains clear:

- To be a resource for every person with hemophilia B
- To educate, advocate, and connect
- To evolve as the community evolves, without ever losing the human touch

"We are a family," Kim proclaims, "and we always will be." The heart of The Coalition for Hemophilia B is more than just a nonprofit organization; it's a movement built on determination, education, family, acceptance, and, most importantly, LOVE.

The Coalition has created a beautiful legacy of positively enriching the lives of those living with hemophilia B. They have built a warm and inviting community where everyone is welcome to come, engage, and learn. Their mission statement is not just a phrase; it's a living, breathing testament guiding their monumental efforts to make a difference in the lives of their hemophilia B family each and every day.

If you'd like to learn more or become part of this amazing community, please visit the Coalition's website at https://www.hemob.org/.
Email contact@hemob.org or call 212-520-8272.

* Reprinted with permission from *'The Heart of a Movement: A Legacy of Determination, Family, and Love'* by Terry Stone, Fall 2025, BioMatrix News, Volume 20, Issue 4, Pages 9–14.

THE COALITION FOR
HEMOPHILIA B

MISSION

The Coalition for Hemophilia B, Inc. is a national nonprofit dedicated to making quality of life the centerpiece of care for individuals with hemophilia B and their families. For over 35 years, we have advanced our mission through education, empowerment, advocacy, and outreach — always with a family–centered approach, because hemophilia affects the entire family.

Our programs unite patients and caregivers from across the country, creating lasting connections, trusted support systems, and opportunities to gain the knowledge needed to make informed decisions and advocate for themselves and their loved ones.

Our dedicated team brings decades of experience and is deeply engaged with the hemophilia B community, both personally and across every communication channel, to ensure no one faces this journey alone.

A Magical Autumn Evening:

CELEBRATING DEDICATION AND LEADERSHIP IN THE BLEEDING DISORDERS COMMUNITY

BY KIM PHELAN


AUTUMN IN NEW YORK
Eternal Spirit Award Gala
THURSDAY, OCTOBER 9, 2025
Honoring
JILL JOHNSEN, MD
&
GUY YOUNG, MD
TERRACE ON THE PARK, QUEENS, NY
THE COALITION FOR HEMOPHILIA B
HEMOB.ORG

This year's Coalition for Hemophilia B Eternal Spirit Award Gala held on October 9th in New York, was truly a night to remember, a beautiful celebration of compassion, leadership, and community. Held under the enchanting theme "*Autumn in New York*," the evening honored two extraordinary physicians, Dr. Jill Johnsen and Dr. Guy Young, for their remarkable service and dedication to the bleeding disorders community.

We were also deeply honored to recognize Senator Joseph P. Addabbo, Jr. with the *Legislative Leadership Award* for his outstanding advocacy on behalf of individuals with bleeding and rare disorders. Senator Addabbo has been a true champion for our community, helping to establish Bleeding Disorders Awareness Month in New York, expanding access to care, and turning compassion into policy through his tireless public service. We were especially pleased to have Jon Davis of the Bleeding Disorders Coalition of New York with us to help honor Senator Addabbo, whose leadership continues to strengthen the lives of those affected across the state.

The evening also featured the presentation of the *2025 William N. Drohan Scholarship Awards*, presented by Craig Drohan, son of the late Dr. William N. Drohan. The awards recognized exceptional students who embody Dr. Drohan's enduring legacy of learning, leadership, and service.










Guests enjoyed a night filled with warmth, music, and magic. A performer in the style of Frank Sinatra serenaded the audience, a magician captivated guests with charm and wit, and an interactive artist with a vintage typewriter delighted everyone with her spontaneous “typed” responses. In one especially memorable and amusing moment, Dr. Jill Johnsen herself typed the question: “Why do Factor IX levels in women not match their bleeding severity?” A clever nod to her pioneering research, and a moment that drew laughter from the crowd.

During cocktail hour, the Bill Gati Band filled the room with classic melodies, perfectly complementing the evening’s elegant autumn theme.

Although Dr. Guy Young was unable to attend in person, he joined us live via Zoom from across the country. His compassion and groundbreaking work have transformed the landscape of hemophilia care. A passionate advocate for patients, Dr. Young has been a driving force behind innovative therapies that are giving people new possibilities for the future. His research has advanced the field, but it is his heart that has advanced hope.

For so many, Dr. Young is far more than a doctor, he is their advocate, their problem solver, and their steady source of hope. He gives his whole heart to his patients, always going above and beyond to ensure they feel heard, supported, and empowered. As we said that evening, “Dr. Young, you hit it out of the park, and unlike the Yankees, you don’t need nine innings to prove you’re a true champion for patients.”

We also celebrated Dr. Jill Johnsen, a visionary whose courage and insight have forever changed how the world understands bleeding disorders. Her groundbreaking work revealed that women do not always bleed according to factor levels, helping redefine diagnosis and care for women and girls.

A heartfelt message from Shellye Horowitz reminded us of the impact of Dr. Johnsen’s work, sharing that a group of women across the country proudly call themselves the “Dr. Jill Johnsen Fangirls,” inspired by her advocacy and brilliance since the first national women’s hemophilia

conference in 2018. As said that evening, “Like the flowers and birds you love, your work has brought beauty, light, and new horizons to a field that needed it most.”

What made the evening even more special was the presence of Wayne Cook, President of The Coalition for Hemophilia B, who, after undergoing heart surgery and a liver transplant in January, was able to attend in person for the first time. His strength, perseverance, and dedication to the community added deep meaning to an already unforgettable night.

We extend our deepest gratitude to our sponsors, whose generous support made this evening possible, helping us honor community leaders, provide scholarships, and sustain our Patient Assistance Fund.

Thank you to our amazing MC Renae Baker, our fabulous team and volunteers and to everyone who joined us and shared in this magical evening.

Together, we celebrated compassion, innovation, and the enduring spirit of our community, truly an *Evening in Autumn* to remember.

ADVOCACY NEWS

IMPORTANT HEALTHCARE LEGISLATION UPDATES

BY JAMES ROMANO

Earlier this year, the U.S. House of Representatives passed a five-bill funding package that provides appropriations for the Department of Health and Human Services and several key federal health agencies. The package includes provisions and program extensions that directly impact children, families, and individuals living with rare and chronic conditions.

Funding Highlights

- National Institutes of Health (NIH): Funded at $48.72 billion, representing a $40 million increase over the previous fiscal year.
- Centers for Disease Control and Prevention (CDC): Overall funding was reduced by $100 million. However, the CDC's Bleeding Disorders Programs were level-funded at FY2025 levels, helping maintain vital research, surveillance, and community-based programs that support individuals living with bleeding disorders.

Key Pediatric Priorities

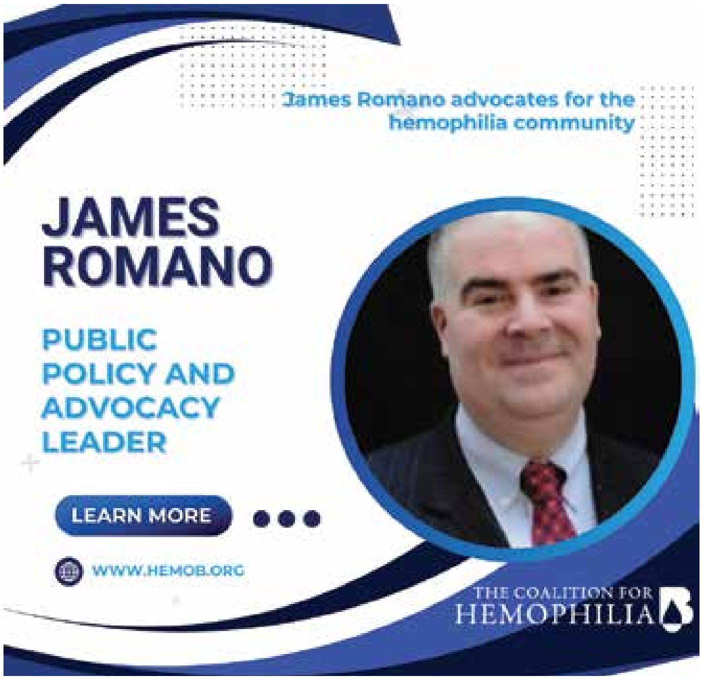
Two important pediatric healthcare initiatives were included in the package:

Accelerating Kids' Access to Care Act (AKACA)

This legislation helps children enrolled in Medicaid and CHIP access specialized medical care more quickly by reducing barriers that often delay diagnosis and treatment for complex or rare conditions.

Give Kids a Chance Act (GKAC)

This bill supports earlier access to therapies for children with serious health conditions and reauthorizes the Pediatric Priority Review Voucher Program, encouraging the development of treatments for pediatric diseases.



Copay Accumulator Legislation

Momentum continues nationwide to protect patients who rely on copay assistance. With the passage of legislation in New Jersey, 26 states, along with the District of Columbia and Puerto Rico, have enacted copayment accumulator bans, ensuring that manufacturer assistance counts toward a patient's deductible and out-of-pocket costs.

Additional states continue to consider similar legislation while patient advocates push for federal protections through measures such as the HELP Copays Act. These efforts remain critical for the bleeding disorders community, where access to specialized care and affordable treatment can make a life-changing difference for patients and families.

The Nat Lathrop Music Scholarship:

Inspiring the Next Generation of Musicians

BY SHELLY FISHER

Music connects us through rhythm, emotion, and shared experience. It gives voice to what words cannot always express and reminds us that creativity and courage often go hand in hand.

That spirit lives on through the Nat Lathrop Music Scholarship, created by The Coalition for Hemophilia B in loving memory of Nat Lathrop.

Now in its fifth year, this one-of-a-kind program empowers teens and preteens in the hemophilia B community to explore music as a source of joy, confidence, and emotional wellness. Each year, the scholarship helps cover the cost of instruments, lessons, band and orchestra fees, and performance programs, making it possible for young people to play, create, and grow.

Cole first applied as a fifth grader eager to join his middle school band. Today, he plays trumpet in his school's top band and thrives on the challenge. With help from the scholarship, he purchased his own trumpet, allowing him to practice regularly and build confidence.

For Nathan, music is closely tied to faith and healing. After teaching himself guitar through online videos, the scholarship allowed him to begin weekly lessons and grow as a musician. His goal is to play with his church's praise band and use music to uplift others.

The scholarship also helped Owen return to band after purchasing his own saxophone, giving him the opportunity to continue playing through high school. Madilyn discovered her love of violin through her school orchestra, where music has become a joyful outlet for creativity and self-expression.

Since its inception, the Nat Lathrop Music Scholarship has supported dozens of young people in discovering their voices through music. The Coalition for Hemophilia B extends its heartfelt gratitude to the Lathrop family for their generosity and vision.

To support the Nat Lathrop Music Scholarship Fund, please visit hemob.org.

MENTAL HEALTH MONDAYS:

A Weekly Space to Unwind, Ground, and Come Home to Yourself

BY RENK KOCTURK

Mental Health Mondays were created as a supportive space to begin each week. Through stretching, breathwork, meditation, and simple regulating tools, participants are invited to slow down, reconnect, and bring their nervous system back into balance.

Busy seasons like the holidays can bring a mix of emotions. While there may be warmth and joy, the pace of gatherings and social expectations can also feel overwhelming. Taking even a few moments to pause and breathe allows us to respond with greater clarity and calm.

During one recent session, we explored two simple practices that help guide the body back into a grounded state. See techniques 1 and 2 below.

Mental Health Mondays take place every Monday from 1:00 PM to 2:00 PM EST and are made possible through CHB community support.

TECHNIQUE 1

5–4–3–2–1 SENSES METHOD

A quick grounding exercise

5 things you can see
4 things you can physically feel
3 things you can hear
2 things you can smell
1 thing you can taste or feel emotionally

Finish with three slow, deep breaths.

TECHNIQUE 2

BUMBLE BEE BREATH (BHRAMARI)

A calming vibration breath

1. Inhale gently through the nose.
2. Exhale with a soft humming sound like a buzzing bee.
3. Notice the vibration in your chest, throat, or face.
4. Repeat for three to five breaths.

This practice helps calm the nervous system and reduce feelings of overwhelm.

HEMOPHILIA LANDSCAPE
UPDATES

BY DR. DAVID CLARK

A number of the items below were presented at the American Society of Hematology (ASH) annual meeting, December 6 – 9, 2025 in Orlando, FL. Copies of the abstracts (summaries) for the presentations are available for free at https://www.hematology.org/meetings/annual-meeting/abstracts.

Trends in Mortality Among Bleeding Disorder Patients in the U.S.

12/6/25 At ASH, a group reported on their survey of mortality (death) in patients with bleeding disorders in the U. S. This is apparently the first analysis of bleeding disorder mortality trends and disparities across the entire bleeding disorder community. They looked at data from 1999 to 2020 in the CDC's WONDER Underlying Cause of Death database.

Between 1999 and 2020, there were 59,320 deaths attributed to bleeding disorders. The absolute number of deaths rose gradually by about 0.45 %/y (percent increase per year), but age-adjusted mortality rates (AAMRs) declined by about 1.68 %/y. AAMR is a standardized, statistical way to compare death rates between populations with different age structures. This removes age as a confounding factor, allowing for fairer comparisons over time or between different groups (e.g., states, genders) by showing relative mortality risk, rather than the actual absolute rate.

Hemophilia A deaths decreased by about 0.31 %/y. They didn't give a separate number for hemophilia B, but deaths from non-hemophilia A, hereditary factor deficiencies (including B and rare factor disorders) rose by about 3.99%/y.

Deaths from von Willebrand Disease (vWD) rose by about 1.39%/y. Regionally, deaths rose most in the West (+1.38 %/y), the South (+0.75 %/y) and the Midwest (+0.36 %/y). The Northeast showed a decline of 0.31 %/y. By state, the largest decline in mortality was in Alabama (-0.9 %/y) and the largest increases were in Arkansas and Delaware (both > 7 %/y).

Notable increases in mortality were seen in Arizona (+3.98 %/y), Georgia (+3.83 %/y) and Florida (+2.30 %/y). Large urban areas were fairly stable at -0.03 %/y, while as you moved away from urban to suburban to small towns and rural areas, the rates increased to about 1.36 %/y in the most rural areas.

By age, the largest increases were among adults 55 – 64 years of age (+2.83 %/y) and those in the 25 – 34-year group (+2.63 %/y). Those in the 75 – 84-year group saw a decline of 0.62 %/y, while those 85 and over showed a small increase of 0.26 %/y. Mortality increased in both sexes, but males saw a much larger increase (+0.88 %/y) than females (+0.12 %/y).

There were huge racial disparities with the highest increases in American Indians/Alaska Natives (+9.36 %/y) and Asian/Pacific Islanders (+5.73 %/y). Blacks showed an increase of 0.61 %/y, while whites showed a lower increase of 0.35 %/y. Hispanics showed an increase of 16.89 %/y compared to an increase of only 0.25 %/y for non-Hispanics.

What good is this data? It's only interesting for some of us, but it is crucial for public health workers. It shows which areas need more work and which are already doing well.

The most alarming are the racial and ethnic disparities. Also, as someone who lives in a very rural area, I was struck by the increase in mortality the farther you get from an urban center. Shellye Horowitz recently wrote in her column in Hemophilia News Today about some of her health challenges, and the additional preparations she has had to make because she lives in a rural area. [ASH abstract 4864]

Cardiovascular Disease in Hemophilia

12/6/25 As the hemophilia population ages, we are realizing that we don't know much about how many of the diseases/disorders of aging affect bleeding disorder patients. We have little experience because until recently, most hemophilia patients did not live to advanced ages.

One of the big areas of interest is in cardiovascular disease (CVD): heart attack, stroke, high blood pressure etc. Historically, it has often been assumed that hemophilia patients have a low risk for CVD; heart attack and stroke are both caused by clotting in the wrong place, so if you don't clot well, maybe you won't have problems. Three papers at ASH reported studies on hemophilia and CVD.

1) A group from the Mayo Clinic looked at 21 patients (18 male; 3 female) with hemophilia (12 As; 5 Bs; 5 Cs) who also have atrial fibrillation (AF). (Hemophilia C is a deficiency in factor XI.) Their hemophilia was either mild or moderate; there were no severes. Most of the patients had co-morbidities (other diseases/disorders besides hemophilia), including diabetes, high blood pressure and coronary artery disease. Fifteen of the subjects (71%) had never smoked; five had a history of cancer; and one patient had an HIV infection and was on antiretroviral therapy. To treat their AF, seven subjects were on aspirin and five were on apixaban (Eliquis, an oral anticoagulant).

Three subjects had at least one major bleeding episode following their AF diagnosis. One patient was on aspirin and the other two were not on anticoagulant/antithrombotic (AC/AT) drugs. One of the patients not on AC/AT also had a second major bleed, a muscle hematoma.

Two patients had fatal bleeds. One was an 83-year old male with moderate hemophilia A who had suffered a major GI bleed before his AF diagnosis. He died from complications from severe epistaxis (a severe nosebleed!). The other was a 56-year-old male with moderate hemophilia B who died from a spontaneous intracranial bleed (a head bleed).

One additional subject died from an ischemic stroke, a stroke caused by a clot blocking a blood vessel. He was a 71-year-old male with moderate hemophilia A who was taking apixaban. Neither his hemophilia or apixaban were able to prevent him from forming the clot.

The authors concluded that their findings demonstrate the limitations of current non-hemophilia guidelines for identifying hemophilia patients at risk of CVD. They recommend larger studies and evidence-based guidelines for treatment of hemophilia patients with AF. [ASH abstract 1299]

2) Another study came from Japan where the incidence of ischemic heart disease appears to be lower for Japanese hemophilia patients than for Western populations. (Some researchers suggest that this is because of the high proportion of seafood in the typical Japanese diet.) On the other hand, another Japanese study had shown that approximately 25% of HIV-positive hemophilia patients exhibited moderate-to-severe coronary atherosclerosis, a precursor/predictor of heart attacks.

The researchers performed coronary computed tomography (CT, a CAT-scan of the heart) on 126 patients (109 As; 17 Bs). The results are too complex to describe here, but the outcome suggests that the risk of CVD in the Japanese hemophilia population is comparable to that of the general population. That is, hemophilia patients have no higher or lower risk for CVD than non-hemophilia patients.

Major risk factors identified by the study included age, high blood pressure, dyslipidemia and diabetes. Dyslipidemia is a condition marked by abnormal levels of fats—high cholesterol or triglycerides, and/or low HDL ("good") cholesterol—in the blood. They also found that TNF-α and baPWV levels are potential markers for identifying individuals at risk for CVD. Tumor necrosis factor-alpha (TNF-α) is a compound produced by the immune system that regulates inflammation. Brachial-Ankle Pulse Wave Velocity (baPWV) is a non-invasive test measuring how fast a pulse travels from the brachium (arm) to the ankle, assessing arterial stiffness, a key indicator of cardiovascular health. [ASH abstract 4843]

3) The third study is similar to the overall mortality study described above, but focused just on mortality due to CVD. The results are similar, since CVD is one of the major causes of mortality in the U. S. A few striking results include that the states of Washington (+6.22 %/y), Arizona (+6.10 %/y) and Minnesota (+5.43 %/y) all showed steep rises in death rates involving bleeding disorders and CVD, while Vermont (-3.75 %/y), Michigan (-1.62 %/y) and Alabama (-1.23 %/y) showed marked reductions in mortality over the same period of time.

A survey like this can't tell us why these disparities exist, but it can point us to where we might want to look to figure out why. It may not mean that the higher mortality states have lower-quality healthcare. States like Arizona might have higher mortality rates because they attract older, retired people who bring their health problems with them.

One of the most concerning results is that the highest annual increases in CVD mortality is among the 25 – 34 year age group at +3.58 %/y. People this young should not be dealing with CVD, but apparently they are, and are dying from it. Good news for us old fogies is that the mortality rate for the 75 – 84 year group has been declining modestly at -0.35 %/y. [ASH abstract 4867]

Subclinical Atherosclerosis in Adolescents with Severe Hemophilia

12/26/25 As described above, younger people with hemophilia are dying of cardiovascular disease (CVD). Atherosclerosis, commonly known as "hardening of the arteries" is the build-up of plaque on the inside walls of arteries. In the coronary arteries supplying the heart with blood, atherosclerosis can eventually plug up the arteries, stopping blood flow and causing a heart attack, also known as a myocardial infarction. Identification of sub-clinical (too small to cause symptoms) atherosclerosis could make early intervention possible possibly preventing future more-serious events.

Carotid ultrasound is a well-established technique that can measure the wall thickness of the carotid arteries, which run along the sides of the neck and supply blood to the brain. The carotid artery wall thickness (CAWT) is a validated marker of future CVD risk. A group of researchers have studied CAWT in a group of 22 adolescent (ages 10 – 21) males with severe hemophilia A (17) or B (5), all of whom are on prophylaxis. Their CAWTs were compared with those of a control group without hemophilia. Weights, BMIs and blood pressure were similar between the two groups.

The hemophilia group had significantly higher CAWTs than the control group. The hemophilia group also had lower HDL ("good" cholesterol) and lower LDL ("bad" cholesterol), but the differences were not statistically significant.

This is apparently the first study to demonstrate significantly elevated CAWT measurements in adolescent males with severe hemophilia compared to age-and gender-matched healthy controls, despite similar physical and blood pressure profiles. The authors note that "this finding suggests that arterial remodeling in males with severe haemophilia may begin during adolescence, potentially predisposing some of them to future CVD." By remodeling, they mean changes in the arterial wall.

The lesson is that vascular remodeling, predictive of CVD, may begin in adolescence in the hemophilia population. The reasons are not yet known, but chronic low-grade inflammation, reduced physical activity and joint disease have all been proposed contributors. The simplicity of the ultrasound CAWT measurements may support timely interventions and reduce future CVD complications. [Trillo A et al., Haemophilia, online ahead of print 12/26/25]

The pieces above are not intended to scare you; they are intended to get your attention. Not that long ago people with hemophilia only lived into their teens, on average. They never had to worry about CVD and other diseases of aging. With better treatments, patients now have almost-normal life expectancies. However, living a long time is not the end of the story. You also want to have good health so you can enjoy that longer life.

The increases in mortality in relatively young people are especially alarming. Now is the time to start taking care of yourself. We are constantly being told to watch our diet, get plenty of exercise, get enough sleep, etc. Pay attention. Those things are all important.

An Online Self-Administered Bleeding Assessment Tool (Validated for Women, too!)

11/4/25 Bleeding assessment tools (BATs) play an important role in treatment of hemophilia and other bleeding disorders. The first widely-used BAT was developed by the International Society on Thrombosis and Haemostasis (ISTH) in 2010. It is a series of questions with associated scores to standardize reporting of bleeding symptoms. The ISTH-BAT was designed for use by healthcare providers, but in 2015 the Self-BAT was developed for patients to use themselves. It has been validated (shown to be consistently accurate) for hemophilia, von Willebrand disease, platelet disorders and hemophilia carriers.

The Self-BAT is still only administered in a doctor's office, so a group of investigators from Canada has now developed an online version that can be taken in the privacy of one's home. Their work is focused on women, but the tool is valid for both sexes and many bleeding disorders.

Women with bleeding disorders, who might be afraid of the challenges and stigma of trying to convince a physician that they do indeed have a bleeding disorder, can take the questionnaire at home. This can help them to explore for themselves whether they have a bleeding problem. In a previous study, the researchers showed that women who arrive at a doctor's office with an abnormal online Self-BAT are more likely to receive a diagnosis and more likely to receive treatment!

Their article gives some distressing statistics. Bleeding in women is not easy to study since it is difficult to distinguish normal bleeding from excessive bleeding. Studies show that an estimated 30% of women have heavy menstrual bleeding (HMB) and that 30% of those have an underlying bleeding disorder. However, only about half of those women seek treatment, "despite 75% actively seeking information about their bleeding on the internet." This suggests that these women feel under-served and not really welcomed by the medical profession. Add to this the fact that surveys have shown that women in the general population are usually much more likely than men to seek medical care.

Hopefully, the online Self-BAT will help to rectify this situation. The researchers, who are all women, gave the online Self-BAT and the physician-administered ISTH-BAT to 63 female subjects, both with and without bleeding disorders. They found excellent agreement between the two, indicating that the online Self-BAT provides a good indication of bleeding behavior.

The online Self-BAT can be found on the internet at www.letstalkperiod.ca. [DeYoung V et al., Haemophilia, online ahead of print 11/4/25]

Treatment Preference for Prophylaxis

12/8/25 With the large number of products now available, how do you choose the one that's best for you? What kind of trade-offs are you willing to make among treatment attributes, for instance, between convenience and effectiveness? Pfizer would like to know this, so they can develop products that people want to use. They collaborated with a group of investigators in the UK to look at patient and caregiver preferences for prophylactic treatment in both the US and UK. The group performed an internet-based survey of 194 adults with hemophilia (US: 150; UK: 44) and 169 caregivers (US: 150; UK 19).

Interestingly, the adults with hemophilia reported less treatment burden in most categories than did the caregivers, but the relative ranking of the different product attributes was similar for both groups. Dose frequency was the most important attribute. Both groups preferred weekly dosing over daily dosing, but there was not much difference in preference between weekly dosing and longer-interval dosing, e.g., every 2 – 4 weeks.

The next most important attribute was bleeding rate, which suggests that the participants valued convenience of administration higher than bleeding rate. Some interesting trends emerged for bleeding rate. On average, avoiding an increase of one bleed per year was 1.6 times more important than reducing the number of bleeds by one per year among adults and 2.9 times more important among caregivers. This is consistent with the theory of loss aversion, which proposes that individuals are more sensitive to losses than to gains.

Among other attributes, eliminating a 5% risk of serious side effects, eliminating a 5% risk of developing inhibitors and changing to a preferred administration mode (intravenous (iv) vs. subcutaneous (sc)) and device type (syringe vs. pre-filled pen) had similar impacts on preferences. Each was approximately one-third as important as dosing frequency and half as important as bleed rate.

The participants significantly preferred sc injections over iv infusions, but with sc, there was only a slight non-significant difference in preference between a syringe and a pre-filled pen. Refrigeration requirements were seen as the least important, although most participants preferred products that did not require refrigeration.

Both adults and caregivers were willing to accept risk to switch from iv infusion to sc. On average, to switch from iv to sc, they would accept an increase in the risk of serious side effects, the risk of developing inhibitors or the risk of additional bleeds. To quote the authors, "In other words, switching from iv to sc is as important [on average] as avoiding 2.0 additional annual bleeds for adults and 1.5 annual bleeds for caregivers.

Note that these are average responses; significant variations in preferences were noted between subgroups based on age, country, number of bleeds over the past 6 months and treatment experience. This suggests that personal preferences should still be considered in shared decision-making. The authors conclude, "Prescribing a treatment that is more consistent with a patient's preferences is more likely to lead to increased treatment satisfaction and potentially increased adherence." [Lu H et al., Haemophilia, online ahead of print 12/8/25]

Kidney Function and Damage in Hemophilia B Patients

9/17/25 Hemophilia patients are thought to have a high prevalence of kidney dysfunction and damage. However, studies have mostly focused on hemophilia A, assuming that their results will also be applicable to hemophilia B. A group of investigators in the B-Natural study decided to look more closely at the situation in hemophilia B. B-Natural is an ongoing international study of the lifetime characteristics and progression of hemophilia B, which medicine calls the "natural history" of the disease.

The subjects included 209 patients with hemophilia B: 32% severe, 51% moderate and 17% mild. Their median age was 13 years (range 1 to 73). They were tested for a number of the different characteristics usually measured to assess kidney function. The findings were surprising.

The main finding is that hemophilia B patients without inhibitors do not have an increased risk of kidney dysfunction or damage compared to people without hemophilia. This may only be a problem for hemophilia A. An associated finding is that prophylaxis with factor IX has no apparent impact on kidney function or kidney disease markers. The primary risk factors for kidney disease in hemophilia B patients are age and high blood pressure, the same as in the general population. Thus, this may be one less problem for hemophilia B patients to deal with. [Astermark J et al., Res Pract Thromb Haemost, online ahead of print 9/17/25]

HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Winter 2025 With the recent approval of several new products for hemophilia B including rebalancing agents and gene therapy, product development is now in a lull. Much of the current research is focused now on seeing how these new therapies perform in the real world. Hemophilia B products can be separated into three categories, 1) improved factor products, 2) rebalancing agents and 3) gene therapy. These updates are divided into those three categories. Within each category, the entries are generally listed in order of the names of the organizations developing the products. A number of the items below were presented at the American Society of Hematology (ASH) annual meeting, December 6 – 9, 2025 in Orlando, FL. Copies of the abstracts (summaries) for the presentations are available for free at https://www.hematology.org/meetings/annual-meeting/abstracts.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using, also including products for inhibitor treatment.

CSL's Idelvion in Children

11/11/25 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. A group of researchers from Japan analyzed chart data from 21 hemophilia B patients less than twelve years of age who had been treated with Idelvion to look at efficacy and safety. All were previously untreated patients (PUPs).

At the start, 95% of the patients were less than two years of age, and 84% weighed less than 10 kg (22 lbs). Most (90%) were on prophylaxis with weekly dosing for 44% of the patients and every-two-week dosing for 56%. The average duration of treatment was 27 months, and no patients developed inhibitors during the study. In the prophylaxis group, no patients two years of age or older had bleeds, while in the younger group, 88% experienced mild bleeds. As expected, those on the two-week dosing regimen had lower trough levels (median 4%) compared to 10% trough levels in the weekly-dose group. Similarly, the two-week dose group had a higher annualized bleeding rate (ABR) of 8.2 compared to 4.9 for the weekly-dose group. The study shows that Idelvion is safe and efficacious in pediatric PUPS. [Nogami K et al., Haemophilia, online ahead of print 11/11/25]

12/15/25 A group of CSL researchers from Japan and the US looked at optimization of dosing using a population pharmacokinetics (PopPK) model in 113 subjects with ages ranging from one to 63 years. PopPK is a statistical approach that studies drug behavior across entire patient populations. It analyzes variability between individuals to develop more precise dosing guidelines and guide clinical decisions. Older pharmacokinetic (PK) models tried to fit everyone's data to a single, average PK curve, which ignores the variability between patients. PopPK models use additional patient characteristics to derive a PK curve that more closely matches each patient's physiology.

They found that body weight significantly influenced the actual half-life of the product and that half-lives were shorter in children under six. They found no meaningful ethnic effects in that PK parameters were similar in Japanese and non-Japanese patients. They also found that in patients 12 years or older, a trough level of about 5% could be achieved by several different dosing regimens: 1) 20-50 IU/kg weekly, 2) 50 IU/kg every ten days, 3) 75 IU/kg every two weeks or 4) 100 IU/kg every three weeks. In contrast, children under six usually required weekly doses of 40 – 50 IU/kg to maintain 5% troughs and had poorer bleed control with longer dosing intervals. This study shows the value of individualized PopPK dosing, especially with a possibility of three-week dosing in older well-controlled patients. [Terasaka N et al., Front Pediatr, 13:1710546, 2025]

Novo Reports Drop in NovoSeven Sales

11/5/25 Novo Nordisk markets NovoSeven, an activated factor VII bypassing agent for treatment of hemophilia patients with inhibitors. In their third-quarter earnings report they reported a 13% decline in sales of NovoSeven. This may reflect inhibitor patients switching to the new rebalancing agents. [Novo Nordisk 3rd Quarter earnings report 11/5/25]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so the blood clots when it should and doesn't clot when it shouldn't. The clotting cascade is a complex system of clotting factors that promote clotting plus anticoagulants that inhibit and control clotting. In a person without a bleeding disorder, the clotting and anticoagulant activities are in balance, so the system produces clots as needed but doesn't clot when it shouldn't. 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.

Novo's Alhemo Shown to Be "Non-inferior" to Prophylaxis

12/6/25 Novo Nordisk markets 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. Alhemo is approved for use in patients 12 years of age or older. At ASH, they presented a more extensive analysis of their Phase III study data that shows that Alhemo gives annualized bleeding rates (ABRs) that are comparable to those obtained by patients on prophylaxis with clotting factor. In statistics, one cannot prove that things are the same or identical. In statistics you can only show that two things are "not different," based on the data that you have. That's a subtle, but important, point.

Those of you who have been following the Coalition's Product Landscape presentations might have noticed that the ABRs for all three current rebalancing agents are not that impressive. They show huge decreases in patients who were previously using on-demand treatment, but don't seem to show much improvement over factor prophylaxis. Their main advantage for patients on factor prophylaxis is their ease-of-use with subcutaneous injection. However, both Novo and Pfizer (see Hympavzi section below) have noticed that several patients in their study groups ended up with much higher ABRs than the rest of the group after treatment with the rebalancing agents.

These patients significantly skew the results when you look at averages. In the last issue, we described the problem that a few extreme data points can significantly affect an average, and that in such cases the "median" is better to use than the average. The median is just the middle value when the data are ranked from low to high or high to low. When the data are analyzed without the high-ABR patients, the results look much better. For hemophilia B, the new results show that the estimated average ABRs for Alhemo stayed the same at 3.1 on either factor prophylaxis or Alhemo prophylaxis. (For hemophilia A, the results showed a decrease on Alhemo, from an average of 3.7 on factor prophylaxis to 2.8 on Alhemo.)

Thus, in addition to giving better results for those patients who did respond well to Alhemo, this study shows that there is a small group of patients for whom Alhemo (or Hympavzi, as shown below) does not work very well. In the Alhemo study, there were three non-responders, two with hemophilia A and one B. There were no common risk factors identified among the three, and they did not develop inhibitors to Alhemo. In a rough approximation, three out of 51 subjects (6%) in the Alhemo study were non-responders, and 12/116 (10%) did not respond to Hympavzi, in the study reported below. Pending better data, this suggests that up to 10% of patients might not respond well to a rebalancing agent. It is currently unknown whether patients who don't respond to one of the products will respond better to another. [ASH abstract 1288]

Pfizer Reports Additional Data on Hympavzi for Patients Without Inhibitors

12/6/25 Pfizer markets Hympavzi (marstacimab) as a rebalancing agent for treatment of patients with hemophilia A or B without inhibitors who are 12 years of age or older. Hympavzi is a weekly, subcutaneous, monoclonal antibody product that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. At the ASH meeting they presented several papers based on their Phase III clinical studies.

In their Phase III BASIS study, the investigators noticed a small group of subjects who had higher annualized bleeding rates (ABRs) than most of the rest of the patients. In the main study, the average ABR for patients receiving on-demand treatment prior to starting Hympavzi was 39.9, which dropped to 3.2 on prophylaxis with Hympavzi. Similarly, the average ABR for patients initially receiving prophylactic treatment was 7.9, which dropped to 5.1 on Hympavzi. However, 12 of the 116 total patients in the study were only able to attain an ABR greater than 12 on Hympavzi. The investigators wanted to see whether they could identify characteristics of those patients that made them less affected by Hympavzi.

With only 12 patients, no significant conclusions could be made about the high-ABR group. In general, they were older, half were Asian, and they had worse joint health. Five of the subjects switched to a higher (300 mg) dose and their ABRs were reduced into the zero to 10.1 range, still high for some. Eight of the subjects continued into an extension study where they will continue to be monitored. [ASH abstract 4838]

12/6/25 From the 116 total patients in the twelve-month active treatment study, 107 decided to continue for another 12 months in an extension study. One of the goals in the extension study was to look at target joints. At the beginning of the active treatment study, before any Hympavzi treatment, 80 subjects had one or more target joints and 25 had three or more target joints. A target joint is defined by ISTH (International Society on Thrombosis and Haemostasis) as a joint that experiences three or more bleeds within a six-month period. A target joint is considered resolved (healed) if it experiences two or fewer bleeds in a 12-month period.

The researchers observed that by the end of the extension study (a total of two years on Hympavzi for most patients) about 88% of patients who had entered the study with one target joint, about 76% of those with two target joints and about 82% of those with three or more target joints had their target joints resolved. This suggests that Hympavzi can help heal target joints. [ASH abstract 4844]

12/6/25 Another part of the Phase III studies looked at the effect of Hympavzi on adolescents (12 – 17 years old) who had been on prophylaxis with clotting factor. In 18 patients (13 As; 5 Bs) without inhibitors, they saw an average decrease in ABR from 3.36 prior to the study to 2.98 after twelve months of Hympavzi treatment. This compares with an ABR reduction from 9.11 to 5.74 in the adults. They saw significant variability in the hemophilia B patients where two patients had ABRs of zero after treatment, but two other Bs had ABRs of 19.48 and 11.10, most of which were from traumatic bleeds (bleeds caused by injury).

Levels of Hympavzi in the adolescent patients were 2 to 2.5 times higher than in the adults, and the clearance of Hympavzi was 29% slower than in the adults. However, correcting for the differences in body weight, the clearance in children was only 3% slower than in adults. Note that Hympavzi is given at a fixed dose of 150 or 300 mg, not by body weight. [ASH abstract 4845]

Pfizer Also Reports on Studies of Hympavzi in Inhibitor Patients

12/6/25 Although Hympavzi is currently only approved for patients without inhibitors, their Phase III study also included 51 subjects with inhibitors (48 patients treated on-demand and 3 on prophylaxis). At ASH, they presented their inhibitor data, which will presumably be submitted to FDA in the near future to request an additional approval for inhibitor patients. The on-demand patients' ABRs fell from a pre-treatment level of 19.78 to 1.39 after treatment with Hympavzi. Patients also reported improvements in health-related quality of life. Adverse events were common but mostly mild, with no reports of thromboembolic events. The safety in inhibitor patients was consistent with those of the non-inhibitor group and previous studies. [ASH abstract 306 and simultaneous publication: Matino D et al., Blood, online ahead of print 12/6/25]

Pfizer Reports Death of an Inhibitor Patient in the Hympavzi Clinical Studies

12/22/25 Tragically, a hemophilia A inhibitor patient in the clinical studies for Hympavzi died of an apparent thrombotic stroke and brain bleed on 12/14/25 after minor surgery. The patient had been on Hympavzi for three years during the Hympavzi Phase III and extension studies. The patient had a minor surgical procedure during which they continued on Hympavzi and received additional recombinant factor VIIa. The patient had several co-morbidities (other medical conditions) and took several other drugs. Pfizer and the trial investigator are conducting an investigation. [Pfizer and WFH/NBDF statements 12/22/25]

Sanofi Reports on Minor Surgeries with Qfitlia

12/6/25 Sanofi markets Qfitlia (fitusiran) as a rebalancing agent for treatment of patients with hemophilia A or B. Qfitlia is a monthly, subcutaneous injection that reduces the amount of antithrombin, an anticoagulant, being made. Qfitlia is approved for use in patients 12 years of age or older. In a previous study of 60 major surgeries with Qfitlia, they found that bleed control was rated excellent or good in 95% of cases. At ASH, they provided additional data on Qfitlia in 71 minor surgeries in 44 patients. This included 12 hemophilia B patients with inhibitors and four Bs without inhibitors.

Again, 95% of surgeries had good or excellent bleed control, with only one rated as "moderate." None of the patients were required to discontinue Qfitlia prophylaxis prior to surgery. Only one of the surgeries required use of an antithrombin III concentrate to reverse the effects of Qfitlia. Antifibrinolytics, such as tranexamic acid and aminocaproic acid, which are often used to treat bleeding in dental and other minor surgeries, were not required in 67% of dental surgeries and 87% of all minor surgeries. These results suggest that minor surgeries can be safely and effectively performed on patients treated with Qfitlia, regardless of their inhibitor status. [ASH abstract 4850]

Use of Sevenfact in Treating Breakthrough Bleeds on Qfitlia

12/6/25 Patients on any of the rebalancing agents are still susceptible to breakthrough bleeds. Non-inhibitor patients can be treated with clotting factor, while inhibitor patients require bypassing agents (BPAs) such as NovoSeven and Sevenfact. Both BPAs are recombinant activated factor VII molecules, though they differ slightly in structure and properties. In the Qfitlia clinical studies, only NovoSeven, at a reduced dose of 45 µg/kg, was used to treat breakthrough bleeds. At ASH, a group of investigators presented data on experimental studies using Sevenfact instead of NovoSeven. These were lab-based studies of thrombin generation when mixing hemophilia A plasma with the various products.

Thrombin generation is a measure of clotting potential. The clotting cascade produces thrombin as the next-to-last step. Thrombin converts fibrinogen to fibrin, which is a "sticky" protein that forms the actual clot. The experiments show that Sevenfact doses of 75 and 125 µg/kg give equivalent thrombin generation as 45 µg/kg of NovoSeven. Clinical studies in humans will still need to be conducted, but these results give good starting doses for those studies. [ASH abstract 1295]

Adherence with Qfitlia

12/6/25 The rebalancing agents are designed to simplify treatment with subcutaneous injection and dose intervals ranging up to monthly, or even every-other-month for some patients. This is expected to improve patients' ability to maintain their prescribed dose schedule. Investigators analyzed the Phase III data for Qfitlia to determine whether that, in fact, happened. The study confirmed that 96% of patients maintained high overall levels of compliance. In addition, there were low rates of discontinuance of Qfitlia treatment, presumably due to low rates of adverse effects. [ASH abstract 1298]

Star Reports Interim Results on VGA039 for von Willebrand Disease

12/7/25 Star Therapeutics is developing VGA039 as a rebalancing agent for treatment of patients with von Willebrand Disease (vWD). VGA039 is a monthly, subcutaneous injection that inhibits protein S, an anticoagulant. While we focus on hemophilia B, some of our members and/or their families are also affected by vWD.

As mentioned above, rebalancing agents may work for many different types of bleeding disorders. VGA039 is being developed for vWD patients, but may also turn out to be useful for hemophilia and other clotting factor deficiencies. Unlike the current rebalancing agents, which inhibit either tissue factor pathway inhibitor (TFPI) or antithrombin, VGA039 is directed against a different anticoagulant, protein S. Exploring the effects of inhibiting different anticoagulants may give us a better idea of the advantages and disadvantages of targeting the various anticoagulants.

At ASH, Star's clinical investigators presented interim results from their ongoing Phase I/II studies, which included 16 subjects with various types of vWD, including 1, 2A, 2M and 3. One goal of the study is dose determination, so the subjects were treated with various doses. The results so far, in eight subjects who have completed treatment, show a median 81% decrease in bleeding episodes. The only adverse event was a headache after one subject's first two injections. There were no thromboembolic complications and no injection site reactions. The selected dose has not been announced, but it is being used in VGA039's Phase III studies, which began in October. [ASH abstract 308]

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 with a new gene to perform a specific function such as producing factor IX.

CSL Reports Final Analysis of Hemgenix Clinical Study Data

12/7/25 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. In spite of the initial slow take-up of hemophilia gene therapies, aside from the clinical studies (54 subjects) Hemgenix has now been received by over 75 patients in eight countries, including over 50 in the U.S.

At ASH, they reported on the final five-year data from their Phase III clinical study. Fifty of the 54 original subjects in the study completed five-years of follow-up. Their average factor IX level was 36%, which remained stable for the entire five years with no evidence of a decrease. The average ABRs over the study were 41.5% at the end of year one; year two: 36.7%; year three: 38.6%; year four: 37.4%; and year five: 36.1%. The drop from year one is probably real, but the variations among the other years are probably not statistically significant. They just represent patient variability and assay uncertainty.

Other characteristics also looked good. The pre-treatment ABR of 4.16 has declined to 0.40 after five years. Only one patient resumed prophylaxis, so 94% remained free of prophylaxis after five years. Hemgenix was generally well-tolerated with no serious adverse events. Most adverse events (AEs) occurred during the first four months after treatment. There were two deaths in the study, but neither was judged to be treatment-related, that is, caused by Hemgenix.

The most common AEs were liver enzyme elevations, which were treated successfully with corticosteroids. Although this was the end of the original study, subjects who consent are being enrolled in an extension study, which will monitor them for up to 15 years post-infusion. [ASH abstract 538 and simultaneous publication: Pipe SW et al., N Engl J Med, online ahead of print 12/7/25]

Inhibitor Development in a Hemophilia A Gene Therapy Patient

12/6/25 Two big unanswered questions in gene therapy are: 1) what happens if you give gene therapy to an inhibitor patient? and 2) what happens if a gene therapy patient develops an inhibitor? The fear is that if you give an inhibitor patient gene therapy, their liver will start cranking out factor, which their immune system will keep trying to get rid of. This sets up an ongoing war within the body in which the liver could be the loser. This is why the current hemophilia gene therapies are not indicated for inhibitor patients.

A number of years ago, some studies in dogs suggested that gene therapy might actually tolerize inhibitor patients to the factor. Constant exposure to factor from gene therapy seemed to cause tolerization similarly to the way that Immune Tolerance Induction (ITI) is used to eliminate inhibitors in some patients.

In ITI, large, frequent doses of clotting factor are administered to a patient, and if successful, the patient's immune system gets used to the presence of the clotting factor and stops producing inhibitor antibodies. ITI is successful in about 70% of hemophilia A patients but only in about 30% of Bs. Can we extrapolate these results to suggest that gene therapy might be useful in those inhibitor patients for whom ITI is also successful? Not yet. Besides, we don't have any way of predicting which patients will be successful with ITI.

More recently, one research group gave gene therapy treatments to two inhibitor patients. One did well and appeared to lose his inhibitor, but in the other, the gene therapy didn't work and his inhibitor persisted. Now we have a new piece of the puzzle. At ASH, a group from Canada presented data on a hemophilia A gene therapy patient who developed an inhibitor. The patient was in a clinical study of giroctocogene fitelparvovec, a gene therapy that was being developed by Pfizer and Sangamo but has since been discontinued. Although the product was discontinued, the study is still ongoing since Pfizer has an obligation to continue to follow the subjects. If not for that, we probably wouldn't know much about this case.

The patient is a 47-year-old male with severe hemophilia A. Before gene therapy, he was on prophylaxis with Jivi, a factor VIII concentrate. He has a complex medical history. He has HIV and HCV infections and in 1987 developed immune thrombocytopenia (ITP). In ITP, the immune system attacks platelets, which further decreases the blood's ability to clot. He went through a number of treatments including immunoglobulin therapy, steroid therapy and even removal of his spleen. Fortunately, since 2000, his platelet counts have returned to normal. The patient has no individual or family history of inhibitors and no risk factors for inhibitor development.

After gene therapy, things looked promising. His factor VIII level rose to 44.2%. However, he developed recurring episodes of liver inflammation and went through three courses of steroid treatment within the first year. Following each course of steroids, his factor levels gradually decreased but were improved by the next course of steroids. At Day 334, he showed presence of a mild inhibitor (0.6 BU). By Day 453, he had developed a high-titer inhibitor of 5.6 BU. He had no bleeding episodes but developed joint pain, which might have been due to microbleeds. Over the next year, his inhibitor titer increased to a maximum of 647.9 before falling to 416.9 by Day 690. He had no detectable factor VIII in his bloodstream and was placed on Hemlibra prophylaxis.

This was a rare event, the first report of inhibitor development in over 280 hemophilia A patients treated with gene therapy. It answers the question of whether someone on gene therapy can develop an inhibitor, but at the same time, it raises a host of new questions. The first question for many of us is whether the same thing could happen with hemophilia B gene therapy.

We don't know, but in the last issue, we noted that hemophilia A inhibitors develop in a somewhat different manner than hemophilia B inhibitors. Also, hemophilia A gene therapy, although very similar in many aspects, results in a gradual decrease in factor VIII production, while factor IX production after hemophilia B gene therapy seems to remain stable. We don't know why the factor VIII levels fall, but there is a good chance that it involves the immune system. Does the constant surveillance of the liver by the immune system lead to a greater chance of inhibitor development? Did the patient's medical history, which included a lot of involvement of his immune system, set him up for this? There is still a lot of research to be done. [ASH abstract 1297]

A Possible Path to Gene Therapy Redosing

12/6/25 One of the significant issues in AAV gene therapy is that the treatment cannot be repeated. Once the original treatment is given, the immune system makes antibodies against the AAV virus, and those antibodies will neutralize any future treatment using AAV. In fact, one of the reasons that such large doses of AAV gene therapy need to be given is that the immune system immediately starts to produce anti-AAV antibodies when the therapy is first infused. The large dose is a brute-force attack to overwhelm the immune system before it can make enough antibodies to neutralize all the AAV particles. However, over time the immune system can produce enough antibodies that it would be able to fend off a second treatment.

At ASH, a group from China presented data on the use of CTLA4-Ig to temporarily stop the production of anti-AAV antibodies so a second dose could be given. CTLA4-Ig (Cytotoxic T-Lymphocyte Antigen 4-Immunoglobulin) is a group of proteins that can suppress immune responses. The researchers performed laboratory and animal studies, after which they tried it in five hemophilia A patients. The patients were given CTLA4-Ig prophylactically starting three days before an infusion of BBM-H803 (the Chinese hemophilia A gene therapy). Over 26 weeks of observation after the infusion, they found that the patients produced much lower levels of anti-AAV antibodies. They have not yet tried an actual redosing of the gene therapy, but the lower anti-AAV levels are promising. [ASH abstract 1293]

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.

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

CSL Behring

MEET MICHELLE:

LIVING PROOF THAT A HEMOPHILIA DIAGNOSIS CAN SAVE YOUR LIFE

BY SHELLY FISHER

With a dome of ivy surrounding her, sunlight peeked around her favorite egg chair in the bright living room, but nothing could outshine Michelle's resilient nature – or so I thought – until her son came in, and her smile lit up the room. Gathering him into her arms for a brief hug, he was soon gone as quickly as he had come, but Michelle's smile lingered and reached her eyes when she said, "That's my oldest son. He has hemophilia too."

Though she was the first born with hemophilia in her family, Michelle's younger brother was diagnosed before her. She later learned that her mother carried a genetic mutation that gave both her and her brother hemophilia B. After her brother's diagnosis when she was 14 years old, her family joined their local bleeding disorder community, but it wasn't until Michelle was 24 years old that she received a diagnosis of her own. That confirmation validated everything she experienced growing up, including one incident in particular that continues to impact her health to this day.

At nine years of age, Michelle fell eleven feet out of a treehouse onto her back. "The paramedics said that I should have been paralyzed." Covered in bruises and suffering a broken ankle, Michelle sustained a concussion and was wheelchair bound for three years. Doctors repeatedly placed her in walking casts, and the pain was so severe she would often pass out. After a second experimental surgery helped stabilize her ankle, her foot remained purple and struggled to heal. Only after intense physical therapy did Michelle learn to walk again.

Throughout her teenage years, she watched her brother experience bleeding episodes and couldn't help but compare them to her own experiences. She suffered from severe menorrhagia that even birth control pills could not resolve. Michelle and her mother discussed the likelihood that she had hemophilia B as well, but at that time women were rarely tested.

Finally, at 24 years old and expecting her first child, Michelle asked her obstetrician about hemophilia testing. Her doctor's uninformed response led her to seek out a high-risk OBGYN. After her factor IX levels tested in the low range, her new doctor referred her to a hematologist. At six months pregnant and limping from severe hip pain, Michelle walked into the office of the doctor she had been asking for throughout most of her life.

He didn't hesitate. "You have hemophilia."

After watching her walk down the hall and into the office, the hematologist didn't even need to see her levels. "Are you having an issue right now?" he asked.

"My hip is really bothering me," Michelle answered. An ultrasound confirmed his suspicion, she had a hip bleed. As they talked further, Michelle explained that her joints had popped in and out of socket throughout her life. Preliminary testing revealed that not only did Michelle have hemophilia B, but she also met the markers for Hypermobile Ehlers-Danlos Syndrome (hEDS), a condition that worsened her bleeding due to microbleeds and eventually contributed to the "severe bleeding tendency" notation in her medical file. While she processed receiving not one, but two diagnoses that explained years of pain, her

hematologist told her, “You’re not leaving here without factor. I am so sorry that you have been living this way, but you won’t have to ever again.”

After years of her family asking questions no one would answer, Michelle sat in the hematologist’s office during the required observation period after receiving factor. She later described what happened next as a sort of out-of-body experience. Through tears she remembered thinking, “Is this what everyone else feels like? This is unbelievable.” Michelle credits her obstetrician and hematologist with saving her life.

After an amnio test indicated that her son was also positive for hemophilia B, her doctors planned a Caesarean birth to ensure his safety and Michelle’s. Unable to receive an epidural due to suspected hEDS, she woke up 45 minutes later and met her firstborn in the NICU. Her second son was also born successfully via Caesarean, thanks to her diagnosis.

In 2023, a massive abscess in Michelle’s intestines required surgery and a factor drip for a month. There is no doubt in her mind that her diagnosis once again saved her life.

Just a week before our interview, Michelle and her son were in a car accident after another driver ran a stop sign and struck them at 55 miles per hour. “I’m just so glad that we’re okay,” she shared. “But I know that part of the reason we’re okay is that we had factor in our bodies.”

Michelle is a passionate advocate within the bleeding disorders community. “I try to make sure that young girls get diagnosed because I want to save them from the pain that women experience when they don’t get factor until later in life.”

Her personal mantras, “it is what it is” and “get real and deal” reflect the type of conversations she values within the community as well.

“I want the deep and dirty programming,” she said. “I want the programming that maybe makes people uncomfortable because out of that discomfort comes growth. That’s why we love the Coalition. We talk about the hard stuff and how we can make it better. That’s why it feels like family. There are people there that literally hold your hand, sit by your bed in the hospital, and send you funny memes.”

After recently losing her father, Michelle shared that the CEO of The Coalition for Hemophilia B, Kim Phelan, reached out personally to offer condolences, and the organization sent her mother a peace lily. “That’s what you do when a family member dies,” she said, “they were checking on us all the time.”

Michelle's connection to the Coalition runs deep. "From the people the CHB chooses to work for them to the events like the symposiums, we don't just want to go every year – we can't wait to go because it's exactly what we've asked for."

When asked to sum up how her family has been impacted by the Coalition, she didn't hesitate.

"They meet us where we're at."

Advocacy runs in the family. Michelle shared that her mother was currently on Capitol Hill lobbying for access to care and treatment choices because, as she put it, "policy affects real people."

At the end of her life, Michelle hopes to look back and see a different outcome for girls with bleeding disorders.

Today she speaks nationwide on behalf of the bleeding disorders community while raising two sons who are the center of her world. Through it all, Michelle holds onto the perspective that has guided her journey. "This is not the end of your story," she says to young people newly diagnosed. "This is the beginning. You are now part of a huge family. You're not alone. You're understood. It's not going to be like this forever – and you will find stable ground." And for Michelle, that ground has been found not only through diagnosis and treatment, but through the community that helped her stand.

LEARNING TOGETHER: UNDERSTANDING THE NEW TREATMENT HYMPAVZI

BY MARTA THOMAS

As part of The Coalition for Hemophilia B's commitment to education, two virtual sessions held on September 9 and November 5 introduced community members to HYMPAVZI® (marstacimab), Pfizer's new treatment option for hemophilia A and B. The sessions offered a welcoming space for learning, questions, and thoughtful discussion about emerging therapies.

This is a paid Pfizer announcement

LEARN ABOUT ™ (MARSTACIMAB-HNCQ)

In this session, we will cover some high-level information on what HYMPAVZI is and how it works.

Speaker: Bradley Schoenfeld, Hemophilia Advocacy & Community Engagement Team Lead, Pfizer

Meal vouchers will be provided!

Both sessions were led by Brad Schoenfeld from Pfizer's Hemophilia Advocacy and Community Engagement team. Brad explained that HYMPAVZI is a subcutaneous treatment option for individuals ages 12 and older who do not have inhibitors. Unlike traditional intravenous therapies, it is delivered through an injection under the skin, which may be especially helpful for those who experience challenges with vein access.

Brad also demonstrated the prefilled pen used to administer the therapy, designed with a simple click to unlock and click to deliver system.

During the Q&A, participants asked thoughtful questions about treatment use, coexisting conditions, and women with bleeding symptoms. The sessions left participants feeling informed and hopeful while reinforcing CHB's commitment to providing high quality education.

Thank you to Pfizer for sponsoring these educational sessions and for their continued partnership.

NAVIGATING GENE THERAPY TOGETHER: INSIDE THE SUPPORT NETWORK COMMUNITY

BY BOBBY WISEMAN

Over the past six months, the *Gene Therapy Support Network* at The Coalition for Hemophilia B has grown from a new informational offering into something much more meaningful: a space where people can come together to learn, ask questions, and support one another through one of the most important conversations in hemophilia care today.

What began as a forum focused on understanding gene therapy has quickly evolved into a trusted community. Participants gather not only to hear the latest information, but also to share experiences, reflect on personal decisions, and connect with others navigating similar questions.

In the early months, sessions focused on building trust, establishing confidentiality, and creating a welcoming space for open dialogue. As relationships formed, conversations deepened. Participants began returning not just for knowledge, but for the sense of connection that comes from speaking with others who truly understand.

Discussions now include partners, caregivers, and family perspectives, recognizing that treatment decisions often affect entire families. Participants are also helping shape future sessions by requesting lived experience perspectives, family voices, and clear clinical insight to complement peer conversation.

As gene therapy continues to reshape the hemophilia landscape, the Gene Therapy Support Network has become an important bridge between medical innovation and real life experience. Most importantly, it reminds participants that they do not have to navigate these decisions alone.

The Gene Therapy Support Network is offered by The Coalition for Hemophilia B and is sponsored by CSL Behring.

CSL Behring

EXPLORING NEW TREATMENT OPTIONS IN HEMOPHILIA CARE

BY JENNIFER DEGLOPPER

On October 1st, members of the hemophilia B community gathered for an educational event sponsored by Novo Nordisk focused on Alhemo®, an FDA-approved treatment for people with hemophilia A or B, with or without inhibitors.

The session featured Dr. Shveta Gupta, a hematologist specializing in the care of girls and young women with bleeding disorders. Dr. Gupta explained how the treatment works, reviewed key clinical trial data, and demonstrated the prefilled injection pen.

Discussion also addressed ongoing challenges in hemophilia care, including bleed protection, vein health, and reducing the burden of frequent infusions.

Special thanks to Novo Nordisk for sponsoring this informative and engaging program.

novo nordisk®

WHERE LEARNING MEETS LAUGHTER: INSIDE THE CHB B EDUCATION HUB

BY KIM PHELAN

This fall, The Coalition for Hemophilia B's *B Education Hub* became a lively virtual gathering place through fun and engaging livestream events hosted by Rocky and Chris. Each week, community members from across the country joined to laugh, play games, and spend time together in a relaxed and welcoming space.

But the B Education Hub is much more than a livestream. It is a dynamic online environment designed for learning, support, and connection within the hemophilia B community. The Hub offers a wide menu of resources, including educational articles, discussion spaces, and a multitude of groups designed to meet the diverse needs of our community. Whether you are a new parent, a long time community member, or someone simply looking to connect and learn, there is truly something for everyone.

The Hub also features learning courses designed to help you move at your own pace, allowing you to explore important topics when it works best for you.

The platform serves as a private internal social network where participation remains confidential and conversations stay within the community, creating a safe space to connect and ask questions.

Members are often encouraged to explore the Hub through fun challenges, including scavenger hunts to discover glossary terms and helpful information that strengthen knowledge across the community.

Throughout the year, the Hub also features surprise events, livestream wellness programs, and interactive activities that bring people together. With so many resources available, we warmly invite you to join the B Education Hub and experience all it has to offer!

SMALL STEPS TO STRONGER DAYS

BY MARTA THOMAS

The October 14th *Strength in Connection* session brought together community members ages 50 and over for a practical conversation about movement, pain, and staying active. Led by physical therapist Makenzie Sledd of St. Louis Children's Hospital, the session focused on how small, consistent movements can support strength and reduce discomfort.

Makenzie introduced "movement snacks," short activities designed to maintain mobility and build strength. Examples included simple sit to stands from a chair and light resistance exercises using household items.

Participants also discussed the difference between arthritis and bleed related pain. The session concluded with light exercises and trivia, leaving everyone encouraged to keep moving.

YOUR HEALTH, YOUR VOICE: ADVOCACY IN ACTION

BY MARTA THOMAS

On October 21st, members of the bleeding disorders community gathered for an engaging *Advocacy in Action* session focused on two issues that affect every family: health insurance and the power of voting.

The discussion was led by longtime community advocates Lee Hall and Bobby Wiseman, who guided participants through an evening that blended practical education with lived experience and thoughtful conversation.

Lee opened the session with important reminders about open enrollment and upcoming policy changes. With Medicare updates and potential ripple effects across insurance plans, he encouraged participants to review insurer communications carefully and remain connected with their care teams and support partners. He also reminded the group that rising medical costs are not always bleeding disorder related, making it important to review all healthcare coverage. Bobby followed with practical guidance on navigating dual enrollment and understanding the differences between government funded plans and other coverage options. Participants asked thoughtful questions about documenting infusions and early voting, receiving helpful advice and encouragement.

The evening also featured Al Farrier, New York's Deputy Commissioner of Elections, who spoke about the connection between voting access and healthcare policy.

This program was made possible through the generous support of Sanofi.

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PARENTING WITH PRESENCE: FINDING AWE, CONNECTION, AND RESILIENCE AS A FAMILY

BY MARTA THOMAS

On October 23rd, parents and caregivers in the hemophilia B community gathered for *Resilience and Radical Curiosity: How Families Can Grow Through Awe* with Deborah Farmer Kris.

Hosted by Ashley Zebley and featuring parenting expert and author Deborah Farmer Kris, the session created a welcoming space where families could connect and share the realities of raising children while navigating a chronic condition.

Ashley, a mother of three, opened the evening by grounding the conversation in lived experience. She spoke openly about the emotional layers of caregiving and the challenges parents sometimes carry quietly. Her message resonated with many in the group: even in difficult moments, connection and support can make the journey feel less isolating.

Deborah Farmer Kris, author of Raising Awe Seekers, introduced a refreshing perspective on resilience. Rather than pushing through stress, she encouraged parents to notice moments of curiosity and wonder that naturally exist in everyday life. These moments of awe, she explained, help regulate stress, strengthen emotional resilience, and deepen parent child relationships.

Throughout the discussion, Deborah shared simple tools parents can begin using immediately. By slowing down, noticing small moments of beauty, and naming them out loud, families can create powerful opportunities for connection.

Parents in attendance reflected on their own experiences, sharing small but meaningful moments that reminded everyone that awe is already present in daily life when we take time to notice it. The evening concluded with gratitude, encouragement, and a shared reminder that families in the bleeding disorders community are never alone on this journey.

This program was made possible through the generous support of Sanofi.

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CARING FOR THE CAREGIVER: REFLECTIONS FROM THE LATIN PROGRAM OF COALITION B

BY LAURA ECHANDI

On November 4th, The Coalition for Hemophilia B hosted a special session in its *Foro Latino de Hemofilia B* series, held in Spanish and dedicated to families and caregivers of people living with hemophilia B. The program opened with a brief presentation from Sanofi.

Amalia (Amy) Vega, Community Relations and Education Manager at Sanofi, introduced Explore a Therapy with Subcutaneous Injection for Hemophilia A and B, inviting participants to learn more about the benefits, safety, and process of starting this treatment.

The evening continued with a thoughtful discussion led by Dr. Joharys Aybar, a psychologist specializing in couples therapy and sports psychology and author of Getting Hooked is Easy, Staying is the Complicated Part. Originally from Puerto Rico, Dr. Aybar guided attendees through an important and often overlooked topic: caring for the caregiver.

“We cannot take care of others properly if we are not well ourselves,” she reminded participants. Caregivers shared the realities of supporting loved ones with hemophilia B while balancing daily responsibilities and emotional stress. Dr. Aybar encouraged simple but meaningful self-care practices such as rest, healthy nutrition, meditation, time outdoors, and open conversations about emotions.

The session closed with a powerful reminder: self-care is not a luxury, it’s essential.

Special thanks to Sanofi for sponsoring this session.

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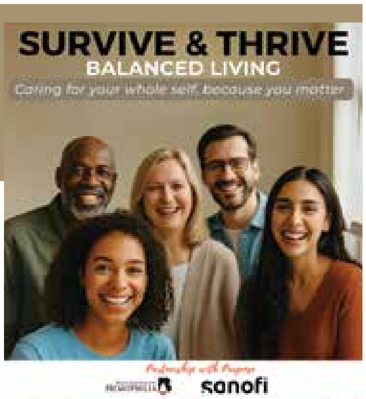
SURVIVE & THRIVE: A NIGHT OF CONNECTION AND COMMUNITY



BY JENNIFER DEGLOPPER

On November 18th, The Coalition for Hemophilia B community gathered online for another *Survive & Thrive* virtual session, an evening centered on connection, encouragement, and meaningful conversation.

The Survive & Thrive series is designed to support the mental, emotional, and everyday well-being of individuals and families living with hemophilia B. Led by licensed mental health professionals, each session combines education, guided discussion, and peer connection, creating a welcoming space where participants can share experiences and learn practical self-care strategies.

Thanks to the generous support of Sanofi, participants came together to learn, reflect, and strengthen the bonds that make this community so unique.

The evening opened with Ashley Smith, Community Relations and Education Manager at Sanofi, who introduced a therapy delivered through subcutaneous injection. Ashley shared helpful insights about how the treatment works, along with key safety considerations and what steps individuals can take if they are interested in learning more.

Facilitators Karen Boyd, LMSW, and David Rushlow, LMSW, then guided the group through thoughtful discussion, creating a safe and supportive environment for sharing. As Karen described it best, the group truly felt like “one beautiful community.”

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STANDING STRONG TOGETHER: GENERATION IX IN THE REDWOODS

BY MATT MARLATT

The Generation IX Project has been providing members of the hemophilia B community with engaging and educational experiences since 2014, and this year was no different. Nestled among the majestic redwoods of Boulder Creek, California, Camp Campbell served as home for this year's Gen IX program, held November 7–10.

The Coalition for Hemophilia B brought together teens and young adults for hands on activities, group discussions, and meaningful conversations designed to strengthen leadership skills. The program blends experiential learning with adventure based challenges to help participants build confidence, teamwork, and resilience. Presented in partnership with GutMonkey, an organization dedicated to experiential and interactive learning, the event was made possible through the generous support of Sanofi.

Upon arrival at Camp Campbell, participants shared dinner and introductions before enjoying a special screening of Giants Rising, a film exploring the remarkable root systems of the redwoods and their ability to endure fire. The documentary highlights lessons in resilience and longevity, offering powerful parallels to the hemophilia B community and its deeply rooted support network that helps individuals face daily challenges with strength and unity.

The next morning, early risers embraced the calm of the San Lorenzo River, taking in the beauty of the redwoods while expressing their creativity through art. After breakfast, Sanofi delivered an inspiring presentation on the power of connection and education within the hemophilia community, reminding everyone of the strength that comes from learning and growing together.

Participants then gathered in small groups to reflect on how redwood groves create environments that shelter and sustain diverse life, much like compassionate leaders foster spaces where those with bleeding disorders feel seen, supported, and valued. We discovered that redwoods stand tall because their roots are intertwined, a powerful reminder that true resilience is not born from standing alone, but from standing together.

Following lunch, the group took on the Challenge Course Experience, a high ropes aerial adventure that called for courage, trust, and teamwork. As participants set shared goals and supported one another through each obstacle, they learned that leadership often means reaching new heights, both physically and emotionally, through connection and encouragement.

Saturday night's activities were both meaningful and memorable. The evening began with a powerful presentation from Sanofi focused on the importance of sharing your story and recognizing the growth that comes through adversity. This message echoed the resilience of the redwoods, trees that not only withstand fire but use it as a catalyst to regenerate and grow stronger.

The night concluded with the 1990s Wacky Game Show Megamix, an energetic and laughter filled event led by the GutMonkey team. Participants teamed up to take on a series of creative challenges centered around bleeding disorders and the strength of the redwoods. Each activity was designed to spark teamwork, confidence, and leadership. It was an evening that blended inspiration, learning, and joy, a true celebration of growth through connection.

Sunday began with an early risers' program designed to spark new connections and encourage participants to step outside their comfort zones. After breakfast, the group boarded a shuttle bound for the Redwood Forest Steam Train at Henry Cowell Redwoods State Park. Powered by a steam engine dating back to the 1890s, it is one of the oldest still operating in regular passenger service in America.

The train carried us over trestles and through towering redwood groves, winding steadily toward the summit of Bear Mountain. As the engine climbed higher, the view unfolded into something breathtaking, a reminder of nature's strength, history, and the enduring spirit of growth that mirrors our own journeys with hemophilia B.

After the train ride, participants spent the afternoon exploring and hiking through the majestic redwood forest, reflecting on the lessons of connection, courage, and leadership that had defined the weekend.

The final night unfolded around a glowing campfire, where meaningful conversations flowed and the group reflected on the power of the deeply rooted support system within the hemophilia B community. Themes such as courage, owning your story, and advocating for yourself rose to the surface, timeless reminders of what true leadership looks like.

The evening wrapped up with a cherished GutMonkey tradition: creating goal bands. Each band served as a personal symbol of intention, a daily reminder of the goals participants are reaching for and the strength they carry forward from this experience.

As the weekend concluded, participants left Camp Campbell with renewed confidence, meaningful connections, and a deeper understanding of what leadership truly means. The towering redwoods reminded us that strong leaders, like strong trees, are grounded by their roots and strengthened through connection.

Leadership within the hemophilia B community is not about standing apart, but about lifting others, creating space for growth, and inspiring resilience through unity. The Generation IX Project continues to cultivate this spirit, empowering future leaders to rise together, grounded in purpose and connected in strength.

This event was offered by The Coalition for Hemophilia B, facilitated by GutMonkey, and made possible through the generous support of Sanofi. We extend our heartfelt thanks to both partners for helping bring this experience to life.

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Comments

"This is my fourth Gen IX and [my] favorite part of the program is the chance to connect with young bleeders in such a supportive, collective space. When I'm feeling frustrated, it can be hard to find people my age who understand what it's like to navigate bleeding complications. But here at Gen IX, the group is so close knit that anything can be openly shared and talked through. Having the opportunity to experience that year after year is truly a privilege and an honor."

"Gen IX is awesome. It's always meaningful to connect with people who face similar challenges. I look forward to coming back each year to reconnect with friends and build new relationships."

"I really enjoyed reconnecting with nature in a way I rarely get to. Seeing the interconnectedness of the redwood trees was an incredible experience, one I wouldn't have had without Gen IX. I loved the connection made between the roots of the redwoods and the roots of our own bleeding community. It truly is a reminder that we are all stronger together."

HEMOPHILIA B PRODUCT LANDSCAPE WITH DR. DAVID CLARK

BY JENNIFER DEGLOPPER

On December 3rd, members of our community gathered for an engaging and informative program, *Hemophilia B Product Landscape* with Dr. David Clark, sponsored by Regeneron. Individuals and families affected by hemophilia B came together for an evening focused on learning, connection, and meaningful conversation about current treatments and promising developments within the hemophilia B landscape.

Dr. Clark, Chairman of The Coalition for Hemophilia B, guided attendees through an overview of today's treatment options. His presentation explored plasma-derived and recombinant factor therapies, extended half-life products, and gene therapy. While the number of available options continues to grow, Dr. Clark emphasized that treatment decisions remain highly personal and should be guided by each individual's bleeding history, lifestyle, and response to therapy.

The discussion also touched on investigational therapies currently in development. Participants asked thoughtful questions about navigating insurance coverage and working closely with their healthcare providers when considering treatment options.

The evening concluded on a lighter note with a lively hemophilia-themed trivia game led by Dr. Clark, reinforcing key insights while keeping the session interactive and enjoyable.

Programs like this highlight the importance of staying informed and connected as the hemophilia B treatment landscape continues to evolve for patients and families.

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GINGERBREAD DECORATING EVENT

BY KALEY MARTIN

On December 6, 2025, we held our 6th annual *Gingerbread Decorating* event. Our wonderful sponsors, Medexus Pharma and Paragon Hemophilia, kicked off the event with a round of holiday trivia and a cheerful game of "this or that". Friendly debate filled the chat as families passionately defended their favorite holiday traditions, from ice skating adventures to cozy memories of hot cocoa.

From the youngest toddlers to parents and family members, everyone joined in with holiday spirit while demonstrating their unique decorating styles. The spirited competition was delightfully broken up by a fan favorite, Rocky William's dad jokes.

Behind the scenes, beautifully decorated Christmas trees glowed as candy began spilling across living room floors. Creativity truly blossomed with unexpected design choices, including Twizzlers, Oreos, and Little Debbie Cakes. Sprinkles flew freely as families jokingly wondered whether their gingerbread creations would maintain their structural integrity through the weekend.

During a game of Kahoot, we learned about gingerbread traditions from around the world. Families shared their own holiday traditions and seasonal wishes as we enjoyed a virtual parade of gingerbread homes. This event is a highlight of the season and one that our community eagerly anticipates every year creating unforgettable memories.

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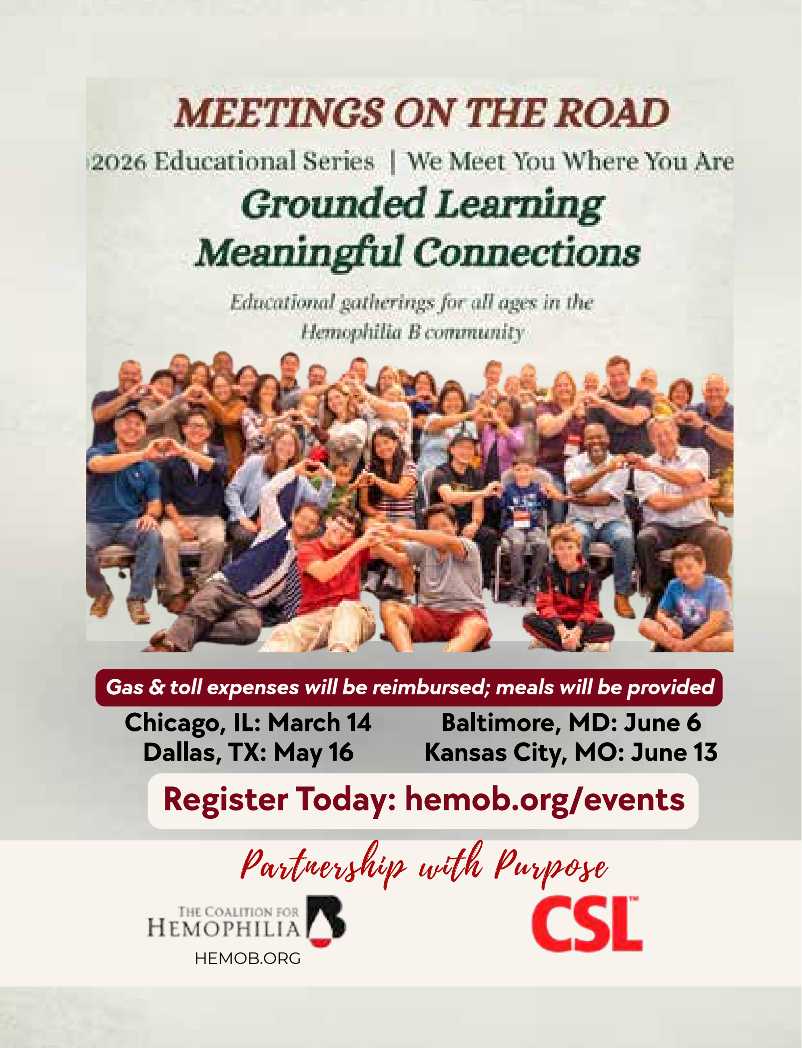

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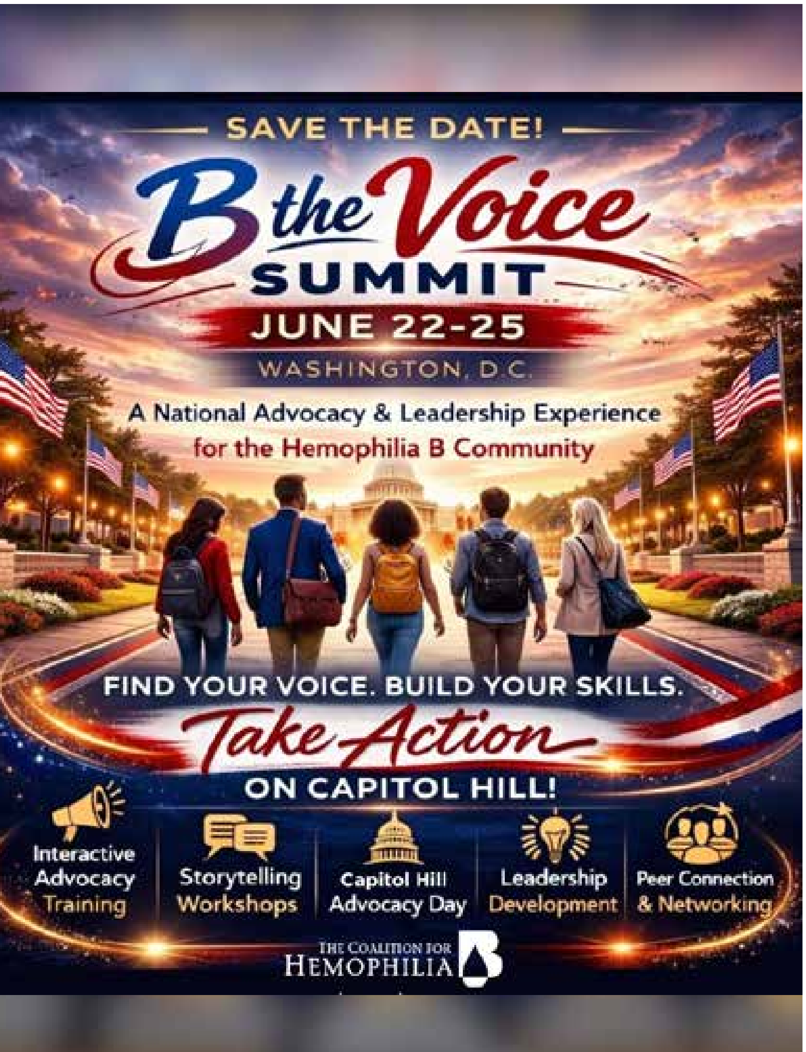

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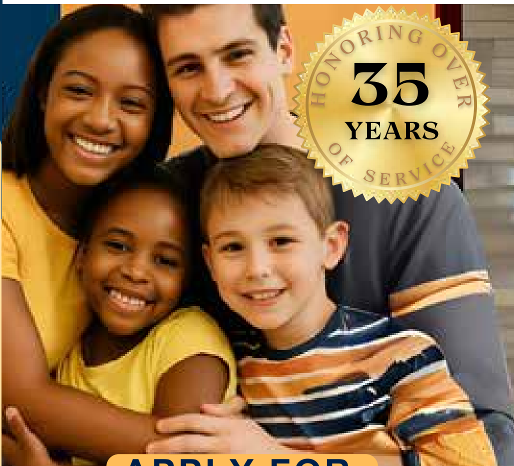

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>
> SHANNON L. ALDER

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- The program does not cover medical bills.
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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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For information, contact us at 212-520-8272, contact@hemob.org

WHY DODOREMI WAS MORE THAN JUST A GAME

BY ROBERT ROBLES

Hosting the B Leaders ***DodoReMi Teen Event*** was something I'll never forget. Bringing together music and community, especially for teens in the bleeding disorders community, meant a lot to me.

B Leaders was created by The Coalition for Hemophilia B to give teens ages 13 to 19 a space that feels welcoming, fun, and just for them. As part of that effort, DodoReMi, a bird themed rhythm game, uses music to bring people together, even through a screen.

One of the best parts of the night was watching music break down walls. Teens who may have felt shy or hesitant at first began opening up, responding, and interacting with one another. Music created a shared experience that allowed conversation and connection to happen naturally.

What made DodoReMi especially special was how interactive it was. This wasn't something you just watched. It was something you were part of. It felt less like a presentation and more like hanging out. Raffles and food vouchers added to the celebration and showed the thought behind the event. These details mattered. They showed the thought and care that went into creating an experience that felt supportive and joyful.

Beyond the fun, the most important message was simple: you matter, and you are not alone. Hearing "you matter" and "you're not alone" can mean everything, especially when you're figuring out who you are. Seeing teens build confidence and friendships through shared interests reminded me why spaces like this matter.

DodoReMi proved that music, creativity, and community can help teens feel seen and supported, and I can't wait to see B Leaders grow and reach even more of you!

MEET DYLAN! HE HAS RHYTHM AND CARNIVOROUS PLANTS TOO!

BY SHELLY FISHER

With dinner cooking on the stove, Dylan talked about fifth grade, Minecraft, music, and what he wanted to be when he grew up.

When I asked what he liked about school Dylan said, “My teachers, and I actually have sixth grade math.” Although he has a penchant for crunching numbers, he also shared, “I like to draw.” When I asked him how his friends might describe him, Dylan offered, “I think they would say I am silly.”

He stays busy with Code Ninjas, orchestra, and Tae Kwon Do. He plays the double bass, like the cello, only bigger, and practices Tae Kwon Do to “keep fit.” When asked what a typical Saturday was like for him, Dylan answered with a big smile that he liked to “just be like a blob,” adding that he liked Jack Black movies like Minecraft and the Kung Fu Panda Movies.

In addition to movies, Dylan and his family enjoy playing games on the weekends. His favorite game is Uno Show ’em No Mercy, though his big brother usually wins.

“I want to be a botanist,” Dylan said. “I like carnivorous plants.” He owns a pitcher plant and hopes to study plants one day.

When our visit turned to Dylan’s diagnosis at four months, his mom shared that they had noticed that just holding him was causing bruising on his skin. Dismissed all her life for several issues, it was when they took him to the doctor they discovered they both had hemophilia B. Dylan manages several ankle bleeds a year and treats with a once a week infusion of clotting factor.

The Coalition for Hemophilia B symposiums and meetings on the road are mainstays for Dylan’s family. Dylan’s mom likened the community to a second family. In those early years, hospital visits were frequent, and the Coalition’s support meant a great deal to their family. Both of Dylan’s parents volunteer at the symposiums and on the road events as well, and Dylan enjoys introducing himself by name and saying, “I have hemophilia B, otherwise known as the Christmas disease.”

Dylan’s advice for other kids: “Don’t be afraid of getting poked. You’ll get used to it. Always treat your bleeds.”

Surrounded by his family and cats Oreo, Marshmallow, and Dorito, Dylan’s world is full of laughter and support. When asked who helps him most, Dylan grinned as his dad dramatically pointed to himself from across the room. Dylan shared that his mom, dad, and Aunt Sara help him most, especially with infusions.

Dylan loves Jack Black’s “I Am Steve,” but his bigger goal is simple: don’t let anything slow you down. I am pretty sure nothing is stopping this bassist, botanist, artist as he pursues his goals, laughs with his family, and last but not least, feeds and studies his carnivorous plants.

MEET ELISEA! SHE HAS SOME EXCELLENT PLANS AND GOING THE DISTANCE!

BY SHELLY FISHER

A few weeks into sixth grade, Elisea was already enjoying her new school year. She shared her love of art, track, and her dreams of becoming a trauma surgeon. After attending their first CHB event this year, she and her mom felt like family within the community and have been volunteering ever since to spread awareness about hemophilia B.

Art is her favorite class. "It's a place you can be creative and do your own thing," she shared. She especially enjoys free drawing. Though she hadn't joined any clubs or sports just yet, she was enjoying easing into a new year and making plans for the spring.

Elisea shared that she "loves track," and plans to join the team this spring with a preference for long distance events. When asked if she had any blue ribbons yet, she answered confidently, "Not yet."

On weekends, Elisea sleeps in, eats, plays games, and spends time with friends. They describe her as "chaotic, funny, chill, and helpful." She jokes that she's known for her big appetite and love of food. She is the friend who is there for everyone and tries to stay positive.

Two years ago, joint pain led to testing that revealed she was a carrier of hemophilia B and sickle cell anemia. Recently, a hematologist diagnosed her with mild hemophilia B, and she treats with clotting factor as needed. Her experiences strengthened her goal of becoming a trauma surgeon. "I want to save lives and give people a second chance."

At their first On the Road event in Arizona, Elisea and her mom connected with others in the community and learned they are not alone. She loved dressing up like Mr. Burns on The Simpsons, trick or treating, and the comfy bed. Her mom shared, "The minute you walk in, you're already family." Elisea and her family hope to pay their positive experience forward. "As of right now, we attend conferences, volunteer at camp, advocate even if it's just for our family at times and try to spread the word on this condition."

Her advice to others: "We can do this together. You can power through it and believe in yourself. You're not alone. There's a great community that can help."

When I asked if there was anyone she would like to mention as an inspiration, she shared that her mom is amazing and "always keeps pushing forward." She added that her mom is also "a little magic." Elisea also confided that Ari and Ellie were two friends that "encourage her and stay positive even when life is hard. Even more than me."

Fittingly, her favorite song is "The Elements of the Periodic Table," and she loves quoting Mr. Burns from The Simpsons. There's no doubt this future trauma surgeon will go the distance, making excellent plans as an advocate in the hemophilia community.

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Winter 2025

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MEET ELISEA!

MEET DYLAN!

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 mattm@hemob.org for your next steps!