THE COALITION FOR HEMOPHILIA B

FALL 2025

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

HEMOB.ORG

MEN'S EMPOWERMENT RETREAT

SIDE BY SIDE: THE STARK BROTHERS' STORY

THE POWER OF GATHERING: MEETINGS ON THE ROAD

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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.



BROTHERHOOD, BALANCE, AND BREAKTHROUGHS: MEN'S RETREAT STRENGTHENS THE HEMOPHILIA B COMMUNITY

BY JACOB POPE

Held September 18 to 20, 2025, at the Atlanta Evergreen Resort at Stone Mountain, The Coalition for Hemophilia B Men's Retreat brought together men with hemophilia B and fathers of those with hemophilia B from across the country. The three-day retreat, generously sponsored by Sanofi, created space for reflection, learning, and connection through a thoughtful blend of workshops, outdoor activities, and shared meals that fostered deep conversations and lasting bonds.

Rocky Williams opened the retreat with a welcome session and the interactive B Connected icebreaker. Breakout discussions followed, focusing on key life experiences. Another session, *Transitions through the Ages*, gave participants space to discuss changing roles, mental health, and life stages. *Talks of Understanding*, led by Fel Echandi, offered a candid conversation for fathers and partners navigating family dynamics and emotional wellness. The evening concluded with a *Rap Session* filled with open sharing, laughter, and the start of new friendships. As one participant, Tyler, shared, meeting others helped him realize he was not alone.

Friday began with an early morning hike, encouraging mindfulness and movement. Sessions throughout the day blended creativity, education, and self-care. *Poetry in Action* invited expression through humor and verse. The








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Hemostasis Balance Activity with Shelby Smoak offered a hands-on look at the clotting cascade and highlighted emerging subcutaneous treatment options.

Manny Lopez held a session celebrating a decade of commitment by Sanofi to the hemophilia B community. Mark Cleary shared his career as a first responder and his passion for car racing. Matt Barkdull delivered *The Alchemy of Grief, Transforming Pain into Purpose*, a deeply personal session that helped attendees reframe loss as a catalyst for growth. Kevin Harris closed the afternoon with *Salt and Stone*, combining nutrition education with a hands-on spice rub workshop. The evening featured friendly competition during the *Bleeder Olympics*, followed by time under the sky at Stone Mountain Park with a drone and laser light show.

Saturday opened with *Standing Together*, a creative team exercise led by Rocky that symbolized unity and adaptability. Dr. Robert Friedman guided *Brotherhood in Rhythm*, using drums and movement to build trust and non-verbal connection. Donnie Akers, Esq., led *Be a Self-Advocate in Your Employment and Healthcare*, empowering participants with tools related to ADA rights and self-advocacy in medical and workplace settings. The afternoon included the *Summit Skyride*, exploration of Stone Mountain Park, and aquatic rehabilitation and fitness led by Douglas Stringham, blending movement with wellness. The retreat concluded with a *Murder Mystery Dinner Theatre*, closing the weekend with laughter, teamwork, and shared memories.

The retreat provided a powerful reminder that life with hemophilia B does not need to be navigated alone. Participants left with new friendships, practical tools, and renewed strength. Special thanks to Sanofi for their continued partnership and support of the hemophilia B community!

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SIDE BY SIDE: THE STARK BROTHERS' STORY

BY RENAE BAKER

Though I have worked with The Coalition for Hemophilia B for years, my immersion into the community really took place at the inaugural Beats Music Program in 2019. I found myself sharing lunch with a pair of affable brothers. They opened my eyes to what life with hemophilia B was like.

Now, one of my greatest annual joys is making music with Rick and Ramon Starks. It's as though these guys figured out the key to happiness, and they stand ready to mentor anyone. They share a diagnosis of severe hemophilia B, and a love of music, martial arts, and their dear families.

We met for a Zoom session. This chat is just the tip of the Stark brothers' iceberg. Seek them out at a live Coalition event – I predict you will leave the conversation smiling. For this article, I'm departing from my usual format in an effort to capture their chemistry.

Rick was born in 1954; Ramon in 1959. Bleeds were common, but boy, did they have fun! Living in Grand Forks, North Dakota, they'd go sledding in the winters.

Suddenly, they're boys again:

Ramon: You'd dive down on the sleds and slide across the ice. We'd go down hills, and we'd start bleeding from our kidneys and end up in the hospital.

Rick: What I remember most is banging my elbows on the ice. Those caused bleeds.

Ramon: We'd make our own snow hills and dig tunnels.

Rick: Big plows would come down the streets and leave huge piles. We dug out caves, like an igloo almost, and spend the day in it. Take a candle in there and it'd warm the whole thing up!

Renae: Have you ever had a fight?

Both: OH YES!

Ramon: We haven't had any real fights in over 30 years.

Renae: Was there a time when you guys looked at each other and realized, "Wow, I really treasure my brother?"

Ramon: I got infected with the HIV virus back from 1982, so in 1995, I got really sick from a CMV virus that attacks your spine. I went from 165 pounds to 115 pounds in 6 weeks. I was at death's door, and then you start appreciating your brothers. From that point on, we were very close.

Rick: I remember him being sick, and I didn't want anything to happen to him. I wanted to spend as much time with him as I could.

Ramon: We were always together. Ever since we were little boys, we'd always be hurt on the couch or in the hospital together at the same time.

Renae: You have other siblings.

Ramon: Yes. There are three boys and three girls in our family.

Rick: I'm the first.

Ramon: and I'm the fourth in line.

Renae: Do any of the others have hemophilia?

Both: Nope.

Ramon: We had an uncle. He died as a baby. He had a cyst on his neck. So, our grandmother took him to the doctor. They cut that cyst off, and he bled to death in her arms. So, we know he probably had hemophilia.

Rick: Our mother was diagnosed later in life, as were three of our aunts. We also have three cousins who had hemophilia. Two passed away from HIV/AIDS. My daughter and grandson are also affected. We have a cousin who's in his 80s now; he's actually no longer a hemophiliac since he had a liver transplant.

Renae: I know you guys love music. Are there other interests you share?

Rick: I signed up for martial arts when I was 18.

Ramon: I signed up too (they're already trying not to laugh). Our father was in the Air Force in the 1960s. He later became the assistant director of the U.S. Judo Association. My father received his brown belt in Judo from Kodakon. So, we were exposed to martial arts at a young age. We started taking Kung Fu from Dacasco's in Denver. Our teacher's son, Mark Dacascos, became a famous movie star martial artist.

Rick: He's the host of Iron Chef.

Renae: So, you guys were doing this before there was factor available, right?

Ramon: (laughter) Yeah, Mom and Dad almost got divorced over that!

Renae: Did you have bleeds from that?

Ramon: Well, yeah, but you exercised so hard at that school that you got in really good shape. I don't know how we got through it. We paid for it later!

Rick: We'd go to the hospital at night and get treated, get discharged in the morning and go right back to training again! One of the conditions was that we were never supposed to compete in tournaments [classic Rick smile], but we did! A lot! (Laughter.)

Renae: What are your biggest concerns for the bleeding disorders community?

Rick: [Suddenly serious.] My biggest concern is, because of the advanced treatments, people are becoming lackadaisical and think they don't need to be involved. They don't realize how far we've come and what it's taken to get here. I'm afraid they won't see the need until it's too late.

Renae: And what would that look like?

Rick: The advocacy wouldn't be there when we really need it. For example: third-party payer copays - the government had been suing to get rid of those. They've now dropped that suit. That happened because of the advocacy that was in place. If we no longer have that advocacy, they can run roughshod all over us and charge us whatever they want.

There are some people who have never had a bleed because they've been on prophylaxis their whole life. Imagine all of sudden, they get a bleed, and they don't know how to manage the pain. In our case, we didn't get anything for pain when we were growing up. So, pain is like no big deal to us. We know how to handle it. Narcotics are not the answer.

Both of us probably live in a constant state of pain, but it's like white noise in the background. You just don't pay attention to it.

Renae: Do you think that these kids who are growing up now, who have never had a bleed are missing out on learning how to manage pain?

Ramon: Probably. The pain that we went through, and the pain kids go through now is totally different. Not to diminish what they go through, but I remember hurting my knee very badly when I was three. Back then, they would get four guys to hold you down, and they would pull your leg down, and your knee is the size of a soft ball, and they'd pull your knee straight, and then they would put a cast on it. That was their treatment.

Renae: Were you screaming?

Ramon: Basically, they gave you a stick to bite on, and Tylenol. That was it.

Rick: A lot of times, we hid bleeds, because we didn't want to go to the hospital.

Ramon: What if we have a national disaster and we couldn't get factor? What are people going to do? It'd be back to our "old school" days. We'll be back to wrapping things up in ice packs real quick.

Renae: You weren't expected to live to be adults.

Ramon: When we were young, if you lived to be 20 years old, you were lucky.

Rick: There was never any preparation for us to go to college. It was, "Well, you made it this far! I don't know what you're going to do, but good luck!"

Ramon: I have to give my mother a lot of credit. She had to be terrified, but she wouldn't show it. She let us learn to ride bikes and live our life. I'm sure she was thinking, "I don't know how long they're going live, but let them enjoy their lives as best they can." She'd stay up with us, late at night, rubbing our knees, or putting ice on them. I don't know how she slept. Six kids. Two of them are hemophiliacs…

Rick: Maybe that's why she had six kids. She had her replacements lined up for us! [Laughter.]

Renae: Is there something you would like to see the bleeding disorder community do?

Ramon: Well, you know what I'm going say, Renae. We just need more of that music stuff!

Rick: My call to action would be to not take the Coalition for granted. Volunteer. Last year, I volunteered 200 hours in person. We need young people to step up. We ain't gonna be around forever!

Renae: So, you don't see many younger people taking the baton?

Both: There are some…

Rick: You can't convince someone to want to take the baton. They have to be inspired on their own to do it.

Renae: OK, what are your best times together?

Rick: When we get to play guitar and bass together. Because we're brothers, we know where each other's going with the music.

Ramon: In June, we're getting together with the whole family, rent cabins at the lake, and just going to play music for five days.

Rick: Oh, I got my cabin BOOKED!

Renae: Well book me a cabin, boys! I can't imagine anything more fun than making music and laughing with the Starks brothers!

HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

Mild Matters: Survey of US Hematologists on Treating Mild Patients

10/30/25 People with mild hemophilia (factor IX levels between 5% and 50%) have historically received little attention compared with those with moderate (1 – 5% FIX) or severe (<1%) hemophilia. This overlaps with the neglect of women with hemophilia since they more often have the milder form of the disorder. A group of US researchers recently published the results of a survey of hemophilia treaters as part of the Hemophilia Federation of America's (HFA's) Mild Matters initiative.

The group points out that "Much of the clinical management for mild haemophilia has been extrapolated from research focused on severe haemophilia," which has led to significant disparities in the diagnosis and management of individuals with mild hemophilia, even at hemophilia treatment centers (HTCs). A number of recent studies have shown that mild hemophilia is not just a milder form of severe hemophilia; people with mild and moderate hemophilia have different issues and needs.

The survey was completed by 51 hematologists across the US, including 18 who only work with pediatric patients, 13 who only treat adults and 20 who treat both. 86.3% of the respondents worked at an HTC. Starting with diagnosis, about 55% of doctors recommend testing of male babies who are at-risk for hemophilia at birth. In comparison, only 20% recommended testing at-risk female babies at birth, even though they come from hemophilia families.

Not only is the rate of at-birth testing low for females, it is also low for males. The World Federation of Hemophilia (WFH) recommends that all male babies born to hemophilia carriers should be tested. One problem with this is that both factor VIII and factor IX levels usually increase during the first six months of life, so the results can change. Genetic testing could overcome that problem, but it is expensive and not available at all HTCs.

Once a diagnosis has been made, most survey respondents were uncertain about when to initiate treatment, since there is little research to guide them. Most respondents said they would initiate treatment (prophylaxis) based on the patient's clinical symptoms, not just factor levels. This is probably due to growing awareness that some mild patients can bleed more than expected. The authors point out that this is reassuring and highlights the need for individualized care.

Overall, the survey suggests that there is a lot that isn't known about treatment of mild patients, including men with mild hemophilia. This is a situation that needs to be rectified. [Lim MY et al., Haemophilia, online ahead of print 10/30/25]

Characteristics of Bleeding in Hemophilia Carriers

8/1/25 The bleeding tendencies of hemophilia carriers have not been explored well and are poorly understood. Most studies have only looked at women of reproductive age. A group of US researchers used the American Thrombosis and Hemostasis (ATHN) dataset to look at characterization of carriers throughout their lifespans.

The ATHN dataset includes information on 3663 hemophilia carriers, 2728 As and 935 Bs. Of the Bs, 0.8% were severe, 1.4% were moderate, 49.1% were mild and 48.2% had normal factor IX levels. They were divided into three age groups, 0 – 12 years, 13 – 49 years and over 50 years. Interestingly, of the 2958 bleeding episodes recorded, 82% were in hemophilia A carriers and only 18% in Bs. Among the 139 Bs with bleeds, the bleeds were most frequent in the 13 – 49 age group with 58% of the total bleeds, followed by the >50 year age group at 27% and 15% for the 0 – 12 year group.

For the 0 – 12 group, the most frequent bleeds were oral and nasal bleeds. The 13 – 49-year group was mixed with a variety of bleed locations. The most frequent bleed location for the >50 group was joint bleeds. Trauma was the most frequent cause of bleeding in all of the age groups. In the 13 – 49 age group, 38% of bleeds were treated with factor, compared to 21 to 27% in the other two age groups.

In the hemophilia B group, 673 carriers had baseline factor IX levels recorded. The median age for the first level measured was 17 years with a wide range of zero to 82 years. Although the A's factor VIII levels increased over time, the B's factor IX levels remained relatively constant. The majority of the hemophilia A carriers had normal factor VIII levels in the 40 – 60% range, and thus, the ones with bleeding episodes would be

classed as "symptomatic carriers" by the ISTH criteria. In comparison, most of the hemophilia B carriers had mild hemophilia with factor IX levels in the 5 – 25% range.

Unfortunately, the ATHN dataset does not contain enough information to determine whether there is a correlation between bleeding and factor levels for the carriers. Results from other studies suggest that bleeding in women does not correlate with factor level. [Swaminathan N et al., Res Pract Thromb Haemost, online ahead of print 8/1/25]

Bleeding Rates in Children on Prophylaxis

8/28/25 Most of the information we have on bleeding rates comes from clinical studies of individual products. Those studies usually only monitor efficacy of the subject's previous treatment for a short period of time, and may also be subject to bias due to patient selection criteria. Real-world data on bleeding during prophylaxis is generally lacking. The PedNet Registry, which collects data from 33 HTCs in 19 countries, was used to obtain a picture of annualized bleeding rates (ABRs) in children with severe hemophilia without inhibitors to serve as a comparison for other studies.

They looked at ABRs in 876 severe hemophilia patients (160 Bs) on full prophylaxis. The subjects were divided into three age groups based on age, from the start of prophylaxis to 5 years of age, from 6 to 11 years and from 12 to 18 years. The median age at the start of prophylaxis was 1.31 years for hemophilia A and 1.46 years for hemophilia B. For the complete group of subjects with hemophilia B, the overall ABR was 1.25 bleeds/year (0.37 for joint bleeds and 0.77 for non-joint bleeds).

The results for the individual age groups were not statistically significant from the overall results, and neither were the differences between the A and B groups. The only exception was that Bs in the 12 – 18-year group had a significantly higher ABR for joint bleeds (0.45) than the As (0.27). The authors state that this is not a clinically significant difference.

The authors also looked at a subgroup of patients on extended half-life (EHL) products. Interestingly, bleeding rates for hemophilia A patients on EHL products were lower, but bleeding rates for hemophilia B patients were not much different from those with standard half-life products, except in the 12 – 18-year group. Other studies have shown mixed results. Some show lower bleed rates with EHL products while others show little difference.

Prophylaxis did not prevent all life-threatening bleeds: ten children (7 As; 3 Bs) had life-threatening bleeds on a full prophylaxis regimen; 4 had intracranial bleeds, 5 had iliopsoas bleeds and one had a pharyngeal (throat) bleed.

The bleeding rates from the study are fairly low but probably represent the rates that can be obtained by patients adhering to a good prophylaxis regimen. If your child's bleeding rates are significantly higher, this may be an indication to speak with your doctor about improving their treatment. [Ranta S et al., Haematologica, online ahead of print 8/28/25]

Treatment and Disease Burden in US Adults with Hemophilia B

6/27/25 As mentioned above, except for clinical studies, there is little information on the real-world experiences of adult hemophilia patients available. Treatment burden refers to the various issues surrounding using products, ranging from maintaining proper storage conditions for the product to the difficulty and frequency of infusions. It includes pain, anxiety, stress, venous access issues, etc., associated with performing intravenous infusions. Disease burden refers to issues resulting from the disorder itself, including joint issues, pain, psychosocial well-being, etc., all impacting the health-related quality of life.

A group of US researchers conducted a study of the treatment and disease burdens in adult patients with hemophilia. This was a non-interventional study examining medical records and survey responses in 446 patients (334 As; 112 Bs) without inhibitors. The study included 41 women (16 Bs). The demographics of the group are a study in itself. The subjects came from across the US, representing 40 states. About 80% were treated at HTCs.

The hemophilia B group was almost exactly split between those with severe hemophilia (48%) and those with mild or moderate severity (52%). Of the 41 women, 39 were mild or moderate and five were on prophylaxis. The two women with severe hemophilia both had hemophilia A. Most (86%) of the severe Bs were on prophylaxis, as were 38% of the moderates and 39% of the milds. Of the Bs on prophylaxis, most (84%) of the severes were on EHL products and most (86%) of the milds were on SHL products.

The group was whittled down to 230 As and 66 Bs by only including people for whom enough data was available to calculate ABRs and who had answered additional questionnaires. The demographics of the smaller group were very similar to those of the initial group. Interestingly, the B patients on SHL factor IX (mostly milds) had the same ABR as the patients on EHL products (mostly severes), an ABR of 1.9. About one-third of each group reported anxiety about whether their current treatment would adequately protect them from bleeds.

The authors state, "Despite recent advances in haemophilia treatment, the data demonstrate that for this cohort of predominantly young to middle-aged

adults, a continued treatment and disease burden exists. The burden appears unrelated to disease severity, as people with mild disease reported similar burdens to those with severe disease. This might suggest that prophylactic treatment itself and the frequency or mode of administration contribute to treatment burden, consistent with other studies."

As stated above for children, if your bleeding rates are significantly higher than an ABR of 1.9, this may be an indication to speak with your doctor about improving your treatment regimen. [Wheeler AP et al., Haemophilia, online ahead of print 6/27/25]

Switching from SHL to EHL factor IX Products

In contrast to the above two pieces, which suggested no benefit to bleeding rates between standard half-life (SHL) and extended half-life (EHL) factor IX products, two other recent studies do show a benefit with EHL products. It is not unusual in science and medicine for studies to show conflicting results. Probing the minute differences between studies can sometimes reveal an important variable that has been overlooked by one or both authors. More often, however, there is no identifiable difference, and we just have to learn to live with the discrepancies until we get more information.

6/21/25 A group from Finland looked at patients switching from SHL to EHL products. For hemophilia B, the EHL products required an average infusion rate of 0.9 infusions per week compared to 1.6 for SHL products. The total average factor IX consumption declined 28% from 221,685 IU/year with SHL to 160,209 IU/year with EHL. The cost of the products, however, increased from €180,930 (about US$209,900) to €236,208 ($274,000). (Note that factor prices are lower in the EU.) ABRs declined from 1.6 bleeds/year on SHL products to 0.8 on EHL. Adherence also improved. [Koivusalo M et al., Haemophilia, online ahead of print 6/21/25]

10/25/25 A group from Germany performed a similar study, just on hemophilia B. The EHL products required an average infusion rate of 1.06 infusions per week compared to 2.79 for SHL products. The total average factor IX consumption declined 37% from 159,578 IU/year with SHL to 100,248 IU/year with EHL. ABRs declined from 6.01 bleeds/year on SHL products to 2.58 on EHL. Health-related quality of life also improved, and no severe adverse events were observed. [Lonardi J et al., Haemophilia, online ahead of print 10/25/25]

Note that the results, even in these similar studies, are quite different. Why does a hemophilia B patient on SHL products in Finland have an ABR of 1.6, while a similar German patient has an ABR of 6.01? Does Finland use a better prophylaxis regimen? Note that Finland apparently use higher factor IX doses, which may be an important difference. In any case, these two studies show a real benefit for EHL products, except for cost.

PAIN!

Pain is an important topic in hemophilia. Many people with hemophilia experience chronic pain, often from joint damage. A quote from the first study below sums up the problem, "Pain has long been an associated symptom of living with haemophilia. Whilst treatment advances have demonstrated phenomenal success in reducing both bleed frequency and burden of treatment, the management of haemophilia-associated chronic pain has seen little improvement over the same period." Three recent studies have addressed pain management for hemophilia patients.

8/25/25 "Chronic pain is now acknowledged not just as a symptom of injury or illness, but as a disease entity in its own right." That quote and the quote above are from a study by a group from the UK who looked at hemophilia healthcare professionals' skills and knowledge in pain management. The study focused on European countries, so the results are not necessarily relevant to the US. They found a number of issues such as it being unclear whose responsibility it was to treat acute versus chronic pain. They also found that most hemophilia treaters had not received adequate education on treating pain and lacked confidence in their abilities.

One HTC had established a dedicated pain management center within the HTC, with excellent results, but that was unusual. Apparently, many people with hemophilia also feel safer when their pain management is handled within the HTC, but most HTCs felt that treatment of long-term chronic pain is best left to the patient's local general practitioner. Interestingly, their experiences with specialized pain clinics were almost universally negative, mostly because of long waiting times for appointments, unwillingness of the patient to be treated outside their normal HTC, and unwillingness of the clinics to accept many patients. [McLaughlin P et al., Haemophilia, online ahead of print 8/25/25]

9/12/25 A group from Italy is conducting an ongoing study to see whether new therapeutic approaches and drugs have been able to reduce chronic pain in patients with hemophilia. They presented an interim analysis recently covering 48 subjects (31 male; 17 female) with chronic pain. Of the men, 62% reported chronic pain, especially in the ankles. For the women, 71% reported chronic pain, especially in the back.

In light of the above study, about two-thirds of the men discussed their pain with their HTC, but 43%

felt it was not adequately addressed. Interestingly, most of the women did not respond to this question because they had never reported their pain. The interim results suggest that the newer approaches to pain management have not benefited most patients. [Pasca S et al., abstract PO-32 from the BIC International Conference, published in Haemophilia, vol. 31, supplement 3]

8/21/25 Because of poor results in relieving their chronic pain with traditional methods, many people have looked to alternative treatments, including acupuncture. Another group from Italy looked at acupuncture as a treatment for chronic pain by examining data from previous studies. They found four applicable studies which included the 37 patients (average age 41.4 years). The results showed meaningful pain reduction, reduced analgesic use and improved health-related quality of life with acupuncture.

There were few side effects to acupuncture, including no significant bleeding. Pain reduction was experienced only for the joint treated, not the whole body. Limitations to the study include the small number of subjects and the fact that there is no good way to blind the subject to the fact that they've had acupuncture. Thus, a placebo effect is an important possibility. The authors recommend larger high-quality studies to confirm their findings. [Demeco A et al., Haemophilia, online ahead of print 8/21/25]

Prosthesis Survival after Hip Replacement in Hemophilia Patients

7/9/25 A group from China did a literature review of prosthesis survival after total hip arthroplasty (THA, total hip replacement). THA is known to be successful in alleviating pain and improving joint function, but the challenges associated with hemophilia result in increased risk of complications such as bleeding, infection and prosthesis loosening. In addition to bleeding risk, hemophilia also introduces the issue of poor bone health in many patients.

They looked at 14 previous studies which covered 190 THAs. A total of 175 prostheses survived, but that means that about 8% failed, slightly worse than for the general population. The primary cause of failure was aseptic loosening, that is loosening of the prosthesis not caused by infection.

The hip joint is a ball and socket joint with a ball on the top of the femur (thigh bone) that fits into a socket in the hip bone. In THA, the ball at the top of the femur is cut off and replaced with a prosthesis which has a metal ball attached to a long stem. The stem of the prosthesis is inserted into the marrow canal of the femur where it is held in place with an acrylic "bone cement." The socket part of the prosthesis is often a plastic cup which is cemented into place in the hip bone. In both cases, poor bone condition can fail to adequately support the new parts, resulting in loosening. The poor support can also result in fracturing of the metal prosthesis itself because of the significant amount of weight that the hip joint supports.

THA is an inherently bloody operation because the surgeon has to dig through a lot of muscle tissue to get to the joint itself. Therefore, perioperative bleeding is an important concern. (The prefix "peri" means near, around or enclosing, so perioperative means before, during and after an operation.) The authors emphasize that THA in hemophilia patients must be well-planned and include experts in both hemophilia and orthopedic surgery. They conclude, "This paper confirms the findings of previous studies that THA is an effective treatment for advanced stage haemophilic arthropathy, offering significant pain relief and improved function. However, its high rate of complications has to be reduced with a correct perioperative hemostasis and a good surgical technique." [Chen L et al., BMC Musculoskelet Disord, online ahead of print 7/9/25]

The Role of IL-10 In Preventing Joint Damage

8/25/25 Iron is essential for good cell health, but too much iron can cause cell damage and cell death. That seems to be what is happening with hemophilia joint damage. Bleeding into the joint leaves red blood cells behind which can "poison" the joint with their iron-containing hemoglobin. The presence of the excess iron triggers a number of immune system pathways that eventually lead to cell death and joint damage. Interleukin-10 (IL-10) is a small molecule that can regulate immune responses, suppress inflammation, promote healing and manage cancer.

Previous studies have shown that IL-10 controls inflammatory responses in chondrocytes, which are the cells found in cartilage. A group from China set out to explore IL-10's role in hemophilic joint damage using chondrocytes from both patients with hemophilic arthropathy (HA, joint damage) and from patients with osteoarthritis (OA). Osteoarthritis is the usual age-related arthritis. The chondrocyte cells were obtained from patients undergoing knee replacement for HA or OA, but the experimental work was performed in cell culture in the laboratory.

The biochemistry of the results is fairly complex, but in general, they show that HA chondrocytes exhibit elevated iron deposition and lower IL-10 expression compared to OA chondrocytes. Addition of IL-10 appears to protect the cells and reduce cell death, thus potentially improving joint health. This laboratory study provides good theoretical support for the potential use of IL-10 in treating hemophilic joint damage, but larger-scale clinical studies will still be needed. [Luo F et al., Haemophilia, online ahead of print 8/25/25]

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† The average dose for adolescents and adults receiving prophylaxis every 7 days was 37 IU/kg.
‡ The median AsBR for people who started on 7- or 14-day prophylaxis was 0. For people who switched to prophylaxis from on-demand, the median AsBR was 0.7. AsBR=annualized spontaneous bleed rate.

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IDELVION®, Coagulation Factor IX (Recombinant), Albumin Fusion Protein (rFIX-FP), is used to control and prevent bleeding episodes in children and adults with hemophilia B. Your doctor might also give you IDELVION before surgical procedures. IDELVION can reduce the number of bleeding episodes when used regularly as prophylaxis.

IDELVION is administered by intravenous injection into the bloodstream and can be self-administered or administered by a caregiver. Do not inject IDELVION without training and approval from your healthcare provider or hemophilia treatment center.

Tell your healthcare provider of any medical condition you might have, including allergies and pregnancy, as well as all medications you are taking. Do not use IDELVION if you know you are allergic to any of its ingredients, including hamster proteins. Tell your doctor if you previously had an allergic reaction to any FIX product.

Stop treatment and immediately contact your healthcare provider if you see signs of an allergic reaction, including a rash or hives, itching, tightness of chest or throat, difficulty breathing, lightheadedness, dizziness, nausea, or a decrease in blood pressure.

Your body can make antibodies, called inhibitors, against Factor IX, which could stop IDELVION from working properly. You might need to be tested for inhibitors from time to time. IDELVION might also increase the risk of abnormal blood clots in your body, especially if you have risk factors. Call your healthcare provider if you have chest pain, difficulty breathing, or leg tenderness or swelling.

The most common side effects of IDELVION are headache and dizziness. These are not the only side effects possible. Tell your healthcare provider about any side effect that you experience, and contact provider immediately if bleeding does not stop after taking IDELVION.

Please see full prescribing information for IDELVION, including patient product information.

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.

IDELVION is manufactured by CSL Behring GmbH and distributed by CSL Behring LLC.
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CSL Behring

HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Fall 2025

There is a huge amount of new product development going on in hemophilia B. The potential new products can be separated into three categories, 1) improved factor products, 2) rebalancing agents and 3) gene therapy. These updates are divided into those three categories. Within each category, the entries are generally listed in order of the names of the organizations developing the products.

IMPROVED FACTOR PRODUCTS

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

Relationship between Dose, Factor Levels and Bleeding with CSL's IDELVION®

CSL Behring

5/19/25 There is a movement today to guide dosing of clotting factor concentrates according to each individual patient's pharmacokinetic (PK) response to a product, rather than simply a set dose according to the patient's weight. Some patients degrade a product much more rapidly, while others may let the product remain in their bloodstream much longer. Also, the same dose of factor IX may have a larger effect on bleeding in one patient than in another. It makes no sense to give those patients the same dose. In fact, a recent study shows a substantial, 182%, variability in bleeding behavior between individuals with similar factor levels.

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 The Netherlands analyzed the data from the five clinical studies performed for Idelvion to try to determine relationships among dose, factor level and bleeding behavior. The subjects included 114 patients with a median age of 26 (range 1 – 61) and baseline factor IX levels of 2% or less, over 514 bleeding episodes. A statistical analysis of the combined group data suggests that at factor levels of zero, they would have an average of 7.3 bleeds/year. The simulation predicts that bleeding levels would be cut by half at a factor IX level of 12%, and at a level of 20% the bleeding rate would be cut to zero (range 0 – 3). Note that this represents only the average predicted behavior for the group overall. Individual patient's results can vary widely from that average. [Koopman SF et al., Clin Pharmacol Ther, online ahead of print 5/19/25]

CSL Updates Comparison of IDELVION® with ALPROLIX® and Rebinyn®

CSL Behring

9/18/25 In the last issue we presented a CSL study comparing the extended half-life products Idelvion and Alprolix. That study has now been updated to include Rebinyn. The results for Alprolix showed that it required higher amounts of factor IX to achieve similar bleeding rates as Idelvion. In this update they showed that the amounts of factor IX used with Rebinyn were similar but achieved significantly worse bleeding rates.

These results suggest that Idelvion is the best product, on average. Does that mean that everyone should switch to Idelvion? Only those people who are average! As we often point out, an important medical principle is that every patient is different. The piece above shows that people can have widely different bleeding tendencies even with similar factor levels. This is no different. We know from real-world experience that some people do better on Alprolix, some do better on Idelvion and some do better with Rebinyn. You need to work with your physician to determine which product is better for you, not just for the average person with hemophilia B. We are fortunate to have a wide variety of products, which makes it more likely that you can find one that works well for you. [Olivieri M et al., Adv Ther, online ahead of print 9/18/25]

Safety of Higher Initial Doses of HEMA Biologics' SEVENFACT®

HEMA Biologics

7/17/25 HEMA Biologics markets Sevenfact, an activated factor VII product for treatment of inhibitor patients. Sevenfact may be given with an initial dose of either 75 µg/kg for mild or moderate bleeds or 225 µg/kg for severe bleeds. The initial dose is followed by additional doses of 75 µg/kg at specified intervals, as needed. To assure physicians who might be worried about thrombogenicity with the higher dose, an international group of researchers looked at the safety of the higher dose based on the data from three clinical studies that supported the product's licensure.

They found that the safety and efficacy of both initial doses were similar with no associated thrombotic events, as well as no other significant safety concerns. Note, however, that subjects with any history of thrombotic events were excluded from the studies. The authors state that patients who tend toward thrombosis should work closely with their doctors to determine the best dose for them. They also point out that the higher, 225 µg/kg initial dose may work faster and may not require additional doses. [Carcao M et al., Haemophilia, online ahead of print 7/17/25]

Pain Reduction with SEVENFACT®

6/19/25 An overlapping group of researchers also looked at pain reduction after treatment with Sevenfact, using the clinical study data. Pain levels at the start of a bleed and at specific intervals after the start of treatment were measured using a visual analog scale (VAS), The VAS, a 100-point scale, has been used to assess pain in a number of clinical settings, including hemophilia. The study looked at five adolescents and 22 adults (all As or Bs with inhibitors) over 468 bleeding episodes. Pain is often used as an indicator of the start of a bleed as well as an indication that treatment is complete when the pain level returns to baseline. Note that some of these patients endured considerable baseline pain, even between bleeding episodes.

The results showed that the VAS score decreased (less pain) 30 – 58% over the first three hours after Sevenfact infusion, and continued to decrease over the first 24 hours after treatment. This occurred regardless of patient age and whether they received a 75 µg/kg or 225 µg/kg initial dose, see above. Interestingly, those with higher baseline pain levels required larger pain reductions before the reductions were noticeable. About 10% of bleeds were treated with analgesics (pain relievers, including opioids), and those bleeds were not included in the analysis. The authors also did a statistical analysis of the VAS results which demonstrated that it may be a clinically useful tool for gauging pain in this setting. [Buckner TW et al., Haemophilia, online ahead of print 6/19/25]

Real-World Experience with Sanofi's ALPROLIX®

9/12/25 Sanofi markets Alprolix, an extended half-life (EHL) recombinant factor IX concentrate. The factor IX in Alprolix is fused to the Fc portion of an antibody molecule to give it a longer half-life. A group of European researchers analyzed the data from the B-MORE study, a 24-month real-world study of Alprolix. The analysis included 137 subjects receiving Alprolix prophylactically for more than six months. The subjects were divided in two age groups, <12 years and ≥12 years, plus a subgroup of subjects ≥50 years old.

The results show a average annualized bleeding rate (ABR) for the <12-year group of 0.98 and an annualized joint bleeding rate (AJBR) of 0.2. For the overall ≥12 year-group, including the ≥50 year-subgroup, the corresponding results were an ABR of 0.87 and an AJBR of 0.46. The ≥50 year-subgroup had an ABR of1.18 and an AJBR of 0.82. All groups had a median infusion frequency of once per week. Their factor IX consumptions per week were 51.03, 45.56 and 47.29 IU/kg, respectively for the youngest to oldest groups. There were no reported inhibitor developments or serious adverse events. Thus, Alprolix appears safe and effective across all age groups. [Glosli H et al., abstract PO-23 from the BIC International Conference, published in Haemophilia, vol. 31, supplement 3]

You might be wondering why sometimes we quote an average value (also called a mean value) and sometimes a median value. With a large amount of data, those values tend to be equal, but with the smaller cohorts in most hemophilia studies, the median actually tends to give the better representation of the group, especially when there are outlying values. For instance, if you had result values of 4, 1, 3, 2 and 10, the average is 5.0. That average makes it look like most of the results are relatively high, even though four of the five values are less than the average. The median is the middle value, when the data are arranged sequentially. Thus, if we arrange the values as 1, 2, 3, 4, 10, the middle value is 3, which is a much better representation of the data set. Most of the values are closer to three than to five. We generally quote whichever values, average or median, the authors list in their article, and if they show both values, we try to pick the one that gives the best overall estimate of the data.

REBALANCING AGENTS

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

application for other bleeding disorders.

Results from a Study of Novo Nordisk's Alhemo® in Young Children

10/23/25 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. An international group of researchers has now conducted a study of Alhemo use in young children six years of age or younger. The study included five subjects with severe hemophilia B who also had inhibitors. In addition to inhibitor development, all five subjects had also experienced allergic reactions to factor IX. Their median age for inhibitor development was 15 months with Alhemo therapy starting at a median age of 21 months.

After at least 12 weeks on Alhemo, the group's annualized bleeding rates (ABRs) ranged from zero to four. Only two of the five subjects experienced spontaneous bleeds, which both resolved after a dose adjustment. Thrombin generation (a measure of clotting) increased, and no thrombotic events occurred. Inhibitor levels also decreased since there was no further factor IX exposure. However, it is likely that inhibitor levels would increase again if the subjects were given factor IX. [Levy-Mendelovich S et al., J Thromb Haemost, online ahead of print 10/23/25]

GENE AND CELL THERAPY

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

Be Biopharma Publishes Pre-Clinical Data

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

Be Bio recently treated their first patient (see last issue) and now has published their pre-clinical data. The various experiments show that 1) addition of a PFIX gene does not interfere with the biology, viability or differentiation of the B cells, 2) the transformed B cells are able to produce large quantities of active PFIX, which can be correctly modified by vitamin K and 3) they have developed a large-scale production process to produce the modified B cells. The patient's B cells are transformed in the laboratory using the CRISPR-Cas9 method, and the researchers showed that 4) the integration site for the new gene was reproducible with few off-target effects (few instances of the gene being put in the wrong place), and 5) that gene insertion maintained the stability of the B cells' genome. In mice transplanted with human B cells, they showed that 6) the B cells rapidly sequester in the bone marrow of the mice and 7) secrete their factor IX into the mouse's bloodstream.

Once the mice had been treated, the researchers showed that 8) factor IX levels decreased slightly right after transplantation but then remained steady for up to 184 days. They also saw the presence of human antibodies in the mice, suggesting that the transplanted cells were still viable and producing antibodies as well as factor IX. They showed 10) that the level of any extraneous PFIX DNA that had not been integrated into the B cell's genomes was very low.

They showed, importantly, 11) that the mice could be re-dosed with additional transformed B cells with no immune reaction. They also showed that 12) the increase in PFIX levels was approximately linear, that is, for instance, if a mouse got a number of transformed cells that raised its PFIX level to about 10 ng/ml, an additional transplant of the same number of cells would raise the PFIX level to about 20 ng/ml. Finally, 13) a 28-day toxicity study in mice showed no problems.

This list gives you an idea of the extensive kinds of studies that companies perform during their pre-clinical studies. They also perform stability studies to make sure the product is stable before transplantation. Additional studies and validation exercises are also required for the manufacturing process. All of this is just to get FDA's permission to go ahead with clinical studies in patients. It's a long road to development and approval of new products. [Liu H et al., Mol Ther, online ahead of print 7/30/25]

BioMarin to Divest ROCTAVIAN® Gene Therapy for Hemophilia A

10/27/25 BioMarin Pharmaceutical markets Roctavian, the only approved gene therapy for hemophilia A. Because of poor sales, they announced that they want to divest Roctavian. They had previously decided to limit their sales to just three countries, the U.S., Germany and Italy in order to reduce their costs.

Although Roctavian is for hemophilia A, this is an additional indication of the sluggish hemophilia gene therapy market. Hemophilia gene therapy was once assumed to be a blockbuster product, but so far hasn't even been able to repay its high development costs. As we have been reporting, the reasons for the slow uptake of gene therapy in the hemophilia community are complex and controversial. One of the main reasons may be resistance of payers to the high prices: $2.9 million for Roctavian and $3.5 million for Hemgenix for hemophilia B. Another important reason seems to be the complicated logistics required to get gene therapy into patients.

Meanwhile, several companies are going forward with development of more advanced hemophilia gene therapies. Plus, China and India have been reporting good results with their own home-grown gene therapy treatments. [BiopharmaDive article 10/27/25]

CSL Study Compares Cost Effectiveness of HEMGENIX® and ALPROLIX® in Sweden

9/12/25 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. To help justify the high cost of Hemgenix, CSL, in consultation with Parexel International, developed a cost-estimation model comparing a Swedish patient's one-time use of Hemgenix with continuing prophylaxis with Alprolix clotting factor concentrate. The results show a lifetime cost savings of about 18 million Swedish kroner, about $1.8 million US dollars, with Hemgenix. The cost of the drugs represents 98.4% of the savings. [Yan S et al., abstract PO-21 from the BIC International Conference, published in Haemophilia, vol. 31, supplement 3]

women & girls with hemophilia

articles to support, educate, and empower

MEET ELIZABETH: LIVING WITH HEMOPHILIA, LEADING WITH MUSIC

BY ALLYSON KAMPS

"Learning to navigate a world that wasn't built for me helped me support others in the same boat."

"I might not be a music therapist if it hadn't been for hemophilia," Elizabeth VanSant told me as she shared her early experiences living as a young woman with severe hemophilia B. Diagnosed at just 3 months old, it was a scary and uncertain time for her parents. She talked about early childhood experiences, including learning to self-infuse, which came with anxiety and medical trauma, insecurities about port scars, and frustrations with how often she needed treatment. But in the end, Elizabeth's story is one of acceptance, compassion, and authenticity.

Childhood dance classes often left her with injuries and bleeds, so she found another way to express her creativity. In third grade, she started piano lessons. At a time when she was learning how to manage her hemophilia, especially self-infusing, the piano bench became a place to process emotions. Elizabeth has since expanded her instrument repertoire to include guitar, flute, percussion, and her voice.

In addition to its emotional impact, playing piano offered a surprising physical benefit. "It made my veins really big," Elizabeth said. "Even just being there and moving your hands, even just your fingers, can really help with vascular health and making those veins pop." The fine motor movement, coupled with mindful breathing, helped prepare her for self-infusion and strengthened her connection to her body in a way that traditional exercise couldn't always offer during periods of healing.

Elizabeth learned to self-infuse at sixteen, showing that it's okay to learn at your own pace. "We progress at different rates, and it's important to recognize your own personal needs," she said. "And it is okay to feel the feels." She also credits her community for supporting her journey, from self-infusion booths at The Coalition for Hemophilia B events to leadership and mentorship through Gut Monkey. "That was something that was really impactful for me, just surrounding myself with other folks."

Today, Elizabeth brings the same patience and compassion to others that helped her as a kid. When she first discovered music therapy, "it was almost like a lightbulb went off." Two years ago, she opened her own music therapy practice focused on neurodivergent individuals in the Pacific Northwest and is now working to expand it into a group practice.

When asked what a typical session looks like, she said, "We're always working on goals and objectives. So maybe somebody has a lot of energy, and they want to throw a ball back and forth. We're also working on communication skills, so I might sing, 'I'm ready,' or like, 'Catch!'" She explained that using simple, rhythmic melodies and phrases that are easy to repeat can help



encourage expressive communication. Depending on what the client feels like doing that day, sessions might also involve lying on the floor, dancing, playing with bubbles, or singing.

Turning her passion for music into a career meant Elizabeth had to find new outlets for creative expression. “My house is full of canvases,” she said. “I paint, sew, build Legos – anything where I’m not making music.” These hobbies offer her space to recharge and engage with creativity just for fun, reinforcing the importance of play and variety in a life that requires both discipline and healing.

Elizabeth is open about her life with hemophilia and sees it as part of what led her to her career. “Learning how to navigate that world [one that wasn’t built for me] has helped me support my clients as they navigate a world that also wasn’t built for them.”

Many mornings, Elizabeth can be found singing Frank Sinatra at assisted living facilities – something completely different from the one-on-one sessions she leads in her private practice. There are no treatment goals or plans, just the joy of connecting through music. Still, the heart behind it is the same. “It’s important to bring all aspects of yourself to whatever it is that you do,” she said. “Don’t hide who you are, and you never know who will hear.”

For Elizabeth, being open is a form of advocacy. “That word is so often tied to Hill Days and whatnot, and that’s not me. I just share my story and know that whoever might be listening, they might have a small takeaway from that.” She understands not everyone is ready to share openly and offers a suggestion: “Start with even your loved ones, your closest friends – what you might be going through on a day-to-day basis, your thoughts, or even just your diagnosis. That’s important. So start small. That’s how everything big happens, by starting small.”

Elizabeth’s journey from figuring out life with hemophilia to building a music therapy practice, shows how sharing your story can be a powerful way to connect with others. With honesty and creativity, she’s making a difference one note, one conversation, and one shared experience at a time.


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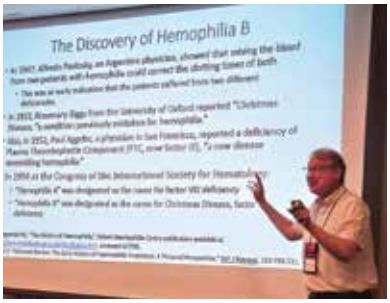
MEDEXUS PHARMA

FALL MEETINGS ON THE ROAD: A SEASON OF CONNECTION, LEARNING, AND JOY

BY ROCKY WILLIAMS



WISCONSIN

This fall, The Coalition for Hemophilia B hit the road to bring learning, laughter, and connection to families across the country. Between September and October, our family-friendly *Fall Meetings on the Road* series visited Milwaukee, WI; San Diego, CA; Minneapolis, MN; Manchester, NH; Phoenix, AZ; and Pittsburgh, PA. These events creating welcoming spaces for the hemophilia B community to connect, share experiences, and strengthen bonds.

For many families, the weekend began with a welcome dinner the evening before each meeting. These relaxed gatherings allowed longtime friends to reconnect and new attendees to feel immediately included, setting a warm tone that carried into the next day.

Each meeting day started bright and early with coffee, conversation, and friendly smiles as participants checked in and explored the exhibit hall. After a heartfelt welcome from The Coalition for Hemophilia B and our sponsors, the energy rose with *Shake, Rattle, and Roll*, led by Dr. Robert Friedman. This lively, music filled session got everyone moving and laughing, creating momentum for the day ahead.

CALIFORNIA

CALIFORNIA



The morning continued with the *Grounded and Growing Community Rap Circle*, where participants shared personal stories, listened with intention, and found strength in shared experiences. Throughout the day, families engaged in a thoughtful mix of education, wellness, and community building. Sessions on gene therapy and new subcutaneous prophylaxis treatments gave attendees the opportunity to hear directly from medical experts as well as individuals with lived experience.

Empowerment remained a central theme. In *Advocacy in Action*, led by Bobby Wiseman and Lee Hall, participants explored how everyday stories can drive meaningful change in healthcare and policy.

While adults attended educational sessions, teens and tweens enjoyed an offsite field trip focused on teamwork and adventure, giving them space to connect and build friendships in their own way.

MINNESOTA






NEW HAMPSHIRE


Between sessions, the atmosphere stayed warm and engaging. Networking breaks, shared meals, and conversations with partners in the exhibit hall encouraged connection, while *Walk and Talk* activities invited participants to continue discussions outdoors, highlighting the many ways wellness and self- advocacy can show up in daily life.

The day concluded with *Our History, Our Story*, created and presented by Dr. David Clark at several meetings, with Matthew Barkdull presenting at others. This was followed by *B Amazing: We Are in This Together* – a joyful closing session filled with laughter, reflection, and shared gratitude.

Across every city, the feeling was the same. These were more than meetings. They were heartfelt gatherings that celebrated connection, resilience, and community. We are deeply grateful to everyone who joined us and to our generous sponsors, CSL Behring and Novo Nordisk, whose partnership made this nationwide series possible.

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ARIZONA

PENNSYLVANIA

FAMILIA DE SANGRE 2025

BY LAURA ECHANDI

From September 5 to 7, the ***9th Annual Familia de Sangre Conference*** took place at the Anaheim Marriott in California, organized by the Hemophilia Foundation of Southern California. Spanish-speaking families from across the country gathered for three days of learning, connection, and community.

Created for families living with bleeding disorders, the conference offered education, support, and meaningful opportunities to share experiences. The Coalition for Hemophilia B was proud to participate, reconnecting with familiar faces and meeting new families while sharing information about upcoming events and programs.

ADVOCACY IN ACTION

BY MARTA THOMAS

This September 10th session resonated deeply with many participants, focusing on how sharing your story, even in small, everyday moments, can be a powerful form of advocacy. Lee Hall and Bobby Wiseman helped guide the conversation, grounding it in both policy and personal experience.

Lee opened with a clear and accessible overview of recent healthcare policy developments, including the One Big Beautiful Bill Act and its potential impact on programs such as Medicaid and the Affordable Care Act. He explained how decisions made in Congress can directly affect access to care, especially for those who rely on these programs most, helping participants better understand what's at stake.

Jim Romano joined to provide live updates from Capitol Hill. He shared insights into the current budget process, possible timelines, and how upcoming decisions could affect the bleeding disorders community. Jim encouraged attendees to watch for action alerts and reminded everyone how simple and effective it can be to reach out to elected officials when advocacy is needed.

A powerful highlight of the evening came from Grant, who shared his personal experiences navigating healthcare and insurance challenges with his family. His story put a human face on policy discussions and underscored why advocacy matters on such a personal level. Erica Garber led a thoughtful activity called "Circles of Connection," inviting participants to reflect on where they feel safe sharing their stories. This moment sparked meaningful conversation as people named siblings, healthcare providers, neighbors, and others they trust.

Bobby added humor and reassurance, reminding everyone that advocacy doesn't require perfection, every conversation and shared story counts. The session closed with raffle winners, smiles, and a reminder that the B Education Hub remains a space for learning and connection.

Thank you to Medexus for sponsoring this meaningful gathering.

MEDEXUS PHARMA

HEMOPHILIA B PRODUCT LANDSCAPE WITH DR. DAVID CLARK

BY MARTA THOMAS

With generous support from Be Biopharma, our community gathered on September 23rd for an informative evening focused on current and emerging hemophilia B treatment options. The session brought together both longtime community members and new attendees, creating a welcoming space for learning and discussion led by two respected experts in the field.

Dr. Michael Wang, Vice President of Clinical Development at Be Biopharma, opened the program with an engaging overview of B cell therapy and its potential role in hemophilia B care. Drawing on his experience as a pediatric hematologist and former HTC director, Dr. Wang explained how engineered B cells may one day produce Factor IX within the body, potentially offering durable protection without the need for preconditioning or frequent infusions. Attendees asked thoughtful questions about redosing, safety considerations, and the progress of ongoing clinical trials. Dr. Wang shared that early data is encouraging and noted key milestones, including the successful infusion of the first patient earlier this year.

Dr. David Clark, Chairman of The Coalition for Hemophilia B, followed with a comprehensive overview of the current treatment landscape. He outlined available options ranging from plasma derived and recombinant therapies to extended half-life products and gene therapy. Dr. Clark emphasized that while having more options is positive, treatment decisions remain highly individual and should reflect how each person responds to therapy.

He also discussed investigational treatments in development and how future therapies aim to improve bleed protection, extend dosing intervals, and provide alternatives for those not eligible for gene therapy. Additional discussion covered switching products, insurance considerations, and how to approach conversations with healthcare providers when evaluating new options.

The evening concluded on a lighthearted note with a hemophilia–themed trivia game created by Dr. Clark, reinforcing key takeaways while keeping the session interactive. Overall, the program highlighted the importance of staying informed and empowered as treatment options continue to evolve.

BIOPHARMA

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

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KITCHEN CABINET HERBALISM: EVERYDAY WELLNESS ROOTED IN TRADITION

BY MARTA THOMAS

Guided by herbalist and somatic healing practitioner Renk Koçtürk, this September 25th session offered a calming and welcoming introduction to ***Kitchen Cabinet Herbalism***, focusing on everyday ingredients and ancestral wisdom. Rather than relying on expensive products, the conversation centered on what many already have at home and how to use those items with care and intention.

Renk invited participants to see autumn as a season for slowing down, grounding, and preparing for winter. She spoke about the deep herbal knowledge passed through Black, Brown, and Indigenous communities, reminding attendees that many family traditions are rooted in lived healing practices shared across generations.

The group explored common herbs such as garlic, ginger, rosemary, and thyme, discussing how they can support both physical and emotional wellbeing. Renk emphasized that herbalism is relational, how we grow, prepare, and speak to plants matters as much as how we use them.

Participants learned practical techniques, including preparing ginger tea by boiling sliced ginger for at least seven minutes to fully release its benefits, and creating rosemary steam rituals to support respiratory health and calm the nervous system.

Throughout the evening, Renk addressed questions about safety and dosage, reminding everyone to listen to their bodies and view herbalism as a complement, not a replacement, to medical care. Attendees left with simple tools, renewed confidence, and a deeper appreciation for the healing wisdom already present in their kitchens.

THE COALITION FOR HEMOPHILIA B EXHIBITS AT HEMOPHILIA OF INDIANA ANNUAL MEETING TRADITION

BY JENNIFER DEGLOPPER

On September 26 and 27, Matt Marlatt and I represented The Coalition for Hemophilia B at ***Hemophilia of Indiana's Annual Meeting*** in Indianapolis. The event was well attended, and we were grateful for the warm hospitality extended by everyone involved. We enjoyed the opportunity to talk with hemophilia B families.

Highlights included the *Teddy Bear Clinic*, where attendees practiced infusion skills in a fun, hands, on way, and Believe Limited's Science Fair Roadshow, which engaged participants of all ages.

Thank you to Hemophilia of Indiana for hosting such a welcoming and engaging event!

THE COALITION FOR HEMOPHILIA B EXHIBITS AT 2025 NATIONAL CONFERENCE FOR WOMEN WITH HEMOPHILIA

BY JENNIFER DEGLOPPER

On October 3rd – 5th, I represented The Coalition for Hemophilia B at the ***Hemophilia Foundation of Michigan's 2025 National Conference for Women with Hemophilia and Rare Factor Deficiencies*** in Detroit. The conference provided a powerful space for connection, learning, and empowerment for women living with bleeding disorders.

Attendees participated in breakout groups focused on rare factor deficiencies, mid-life and young adult experiences, family planning, and Spanish speaking communities. This annual conference remains a meaningful and inspiring gathering for women across the bleeding disorders community.

UPCOMING EVENTS

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

Coalition members who join our virtual events will receive food vouchers! Please make sure your camera is on and you're visible during the event

EVERY MONDAY 1-2 PM EST
Mental Health Mondays in the B-Hub
CONNECT.HEMOB.ORG

JAN 22, 2026 • VIRTUAL
Adulting Unlocked Career Building Workshop
Join us for a fast-paced, power-packed virtual workshop where we'll dive into everything you need to stand out professionally: resume glow-ups, LinkedIn optimization, interview confidence, and real-world career strategies that actually work.

FEB 10, 2026 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over.

FEB 19–22, 2026
ORLANDO, FL
Men's Empowerment in Action Retreat
A unique three-day experience, during which men are given a safe space to share their journey with others and gain support.

MARCH 10, 2026 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over.

APRIL 8, 2026 • LAS VEGAS, NV
Let's Play IX Golf Charity Event
Raising funds to make it possible for children with hemophilia B to participate in a fun strength and skills-building activity offering a healthy lifestyle option they can continue for a lifetime.

APRIL 9–12, 2026
LAS VEGAS, NV
The Coalition for Hemophilia B Symposium 2026!
The Coalition's Annual Symposium convenes medical, research, mental health, and policy experts alongside industry leaders and advocates to deliver education, support, and meaningful connection. The event builds community and empowers individuals to move forward with greater independence, confidence, and success in their careers and lives.
Travel grants available!

SHARE YOUR STORY

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

2026 ANNUAL SYMPOSIUM

APRIL 9–12, 2026 | LAS VEGAS

https://www.hemob.org/annual-symposium

THE COALITION FOR HEMOPHILIA

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BCares

THE COALITION FOR HEMOPHILIA B PATIENT ASSISTANCE PROGRAM

> "ONE OF THE MOST IMPORTANT THINGS YOU CAN DO ON THE EARTH IS TO LET PEOPLE KNOW THEY ARE NOT ALONE."
>
> SHANNON L. ALDER

We know that life can present unexpected challenges. Families may face difficult times such as reduced work hours, job loss, unemployment, hospitalization of a loved one, or even unexpected natural disasters. Our Patient Assistance Program was created to support patients and caregivers during these times of need. This program provides emergency funding to help ease the burden of sudden financial hardship.

- The program does not cover medical bills.
- Assistance is intended for urgent, short-term needs that directly affect patients and their families.

We are here to stand beside you when life feels overwhelming, offering help and hope in the moments when it matters most.

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

LEARN MORE: https://www.hemob.org/financial-assistance
PLEASE DONATE: https://www.hemob.org/donate

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Linkedin: https://www.linkedin.com/company/coalition-for-hemophilia-b/

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

MADDIE'S GOING THE DISTANCE AND LOOKING FORWARD TO SUPPORTING THE HEMOPHILIA B COMMUNITY

BY SHELLY FISHER

A freshman in college, Maddie squeezed me in between psychology courses, weekend work, daily jogs with a family member, and plans to visit a haunted house. With a fall full of her favorite activities, she is looking ahead to a future as a mental health counselor and continuing her involvement in the hemophilia B community.

College has been a "big switch" for Maddie, and she is enjoying the added independence and flexibility in managing her coursework. While she was not participating in clubs at the time of our interview, she plans to join a psychology research club. "I would be looking into research for psychology articles, which sounds pretty cool," she shared. Maddie is keeping an open mind about where her studies may lead but knows that mental health counseling is her goal. "There's still so much to explore in that field." She also noted that her psychology professor has quickly become her favorite, describing her as engaging, fast-paced, and a little funny.

On weekends, Maddie works as a cashier and courtesy clerk at a local grocery store, the same store her family has patronized for years. Outside of work, she enjoys spending time with her family and friends, where she is known as "the funny one," and focuses on improving her running distance alongside her brother.

With a known family history of hemophilia B, Maddie was identified as a carrier at birth and later learned she has mild hemophilia after requiring treatment following surgery. As a cross-country runner, she has remained active without issue.

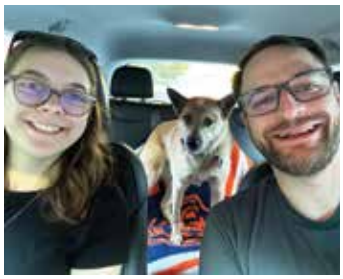
"Running is something that takes a lot of time to improve," she said, noting the patience and persistence it requires.

When asked what advice she would give someone newly diagnosed with hemophilia, Maddie offered thoughtful reassurance. "Everybody's experience is different. Make sure you have a good support system. Take baby steps and find your comfort zone as your own advocate."

Maddie most recently attended The Coalition for Hemophilia B's Meeting on the Road event in Milwaukee, where she especially enjoyed learning about the history of hemophilia. She found the small group discussions meaningful, particularly hearing perspectives from older participants. "It allowed me to be much more aware from a national and world view," she shared.

For her family, CHB events throughout the year have fostered a strong sense of connection. "I love the talks and learning all that I can, and we always help with cleanup," Maddie said. These experiences have also shaped her professional goals. "I've learned that there can be a mental toll for those managing hemophilia B, and I'm interested in providing therapy for anyone going through that."

With the support of her parents and a strong community behind her, Maddie is ready to go the distance and give back to the hemophilia B community, with friends and family cheering her on every step of the way.



LIGHTS! CAMERA! ACTION! THERE'S A STAGE IN JOSHUA'S FUTURE AND HE WANTS TO PUT THE SPOTLIGHT ON HEMOPHILIA B!

BY SHELLY FISHER

Just two days into November, Joshua carved out time from a quiet Sunday to talk about senior year, future goals, and his passion for giving back to the hemophilia B community. With much of the school year already behind him, Joshua admitted to a bit of senioritis and was eager to begin the next chapter of his life.

When asked how school was going, Joshua answered simply, "School's going fine." While he enjoys history, his true passion lies in modeling and acting. Joshua participated in theatre classes throughout his freshman, sophomore, and junior years and feels confident in the skills he gained. "I'm really good at memorizing lines and knowing where to be," he shared. "But play acting and movie acting are very different. I want to be in a movie, not a play.

Joshua has been building his portfolio and résumé with the support of a talent agency and acting classes, keeping his options open. "Actors can't be picky. They should be flexible," he said. Open to a variety of film genres, from action to comedy, Joshua is just as adaptable when it comes to modeling. "I love a good outfit. I'll model whatever comes my way."

Outside of school, Joshua enjoys spending time with friends, often hanging out at the mall or relaxing together. Known as "the mom" of the group, he's the one reminding everyone to act responsibly and stay calm in public. If a friend is feeling down, Joshua is quick to step in with encouragement and humor, even though he admits he's often the target of his friends' pranks.

Joshua has always known he has hemophilia B, but it wasn't until fourth grade, after injuring his shoulder at recess, that he experienced severe symptoms requiring an infusion. He still experiences some tingling and numbness in that area, but it hasn't stopped him from doing the things he enjoys. His hematologist continues to monitor the issue, and Joshua remains open to exploring physical therapy if needed.

When asked what advice he would give someone newly diagnosed, Joshua responded with his characteristic ease. "You have to live it. It doesn't anchor you down. You just have to be aware. It never stopped me from doing what I wanted to do."

The Coalition for Hemophilia B has played an important role in Joshua's life, providing education, resources, and connection for his family. He especially enjoys the teen programming at CHB symposiums and recently attended the Florida event, where he loved visiting an interactive museum with peers. "There's something special about doing things like that with people who understand what you're going through," he said.

Joshua also volunteers with his local bleeding disorder community and serves as a youth leader at camp, where inclusion and connection matter most to him. With a stage clearly in his future, Joshua hopes to use every spotlight to raise awareness for hemophilia B. "I want to show people that they can do whatever they want," he shared. Joshua's first scene is just beginning — roll tape.




B inspired!

Stories and artwork from teens in the Hemophilia B Community

FALL 2025

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- LIGHTS! CAMERA! ACTION! THERE'S A STAGE IN JOSHUA'S FUTURE AND HE WANTS TO PUT THE SPOTLIGHT ON HEMOPHILIA B!

MEET JOSHUA!

MEET MADDIE!

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!