THE COALITION FOR HEMOPHILIA B

SPRING 2025

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

SYMPOSIUM 2025

MEET BECKY: PRESIDENT'S AWARD FOR MATHEMATICS, SCIENCE & MOTHERHOOD (ACCORDING TO US!)

GUMBO & GENE THERAPY: A SOUTHERN SPIN BY BOBBY

NO BULL: WISDOM FROM HEMO FARMER CHRIS

CONTENTS

FEATURE STORY: **35 YEARS STRONG: THE COALITION'S LARGEST AND MOST JOYFUL SYMPOSIUM YET! – PAGE 3**

PRODUCT LANDSCAPE

ADVOCACY

HUMAN INTEREST

WOMEN BLEEDERS

EVENTS RECAP

EVENTS RECAP continued...

UPCOMING EVENTS

B INSPIRED TEEN SECTION

THE COALITION FOR HEMOPHILIA

MISSION

TO MAKE QUALITY OF LIFE THE FOCAL POINT OF TREATMENT FOR PEOPLE WITH HEMOPHILIA B AND THEIR FAMILIES THROUGH EDUCATION, EMPOWERMENT, ADVOCACY, AND OUTREACH.

35 YEARS STRONG: THE COALITION'S LARGEST AND MOST JOYFUL SYMPOSIUM YET!

BY KIM PHELAN

From heartfelt reunions to groundbreaking discussions, the 35th Annual Symposium of the Coalition for Hemophilia B was truly unforgettable. Held April 9–13 at the Renaissance Orlando at SeaWorld, it welcomed our largest crowd in history, a vibrant, joyful, and deeply connected gathering that honored where we've been and looked ahead to where we're going.

The theme *We Meet You Where You Are* echoed throughout the event, not just in programming, but in the personal connections, shared wisdom, and meaningful moments that unfolded across the weekend. Whether it was your first symposium or your nineteenth, you were part of something extraordinary. This gathering celebrated 35 years of standing strong as a community, rooted in love, resilience, and purpose.

From our earliest days to this incredible milestone, we've seen remarkable progress, driven by your stories, strength, and advocacy. And while challenges remain, like insurance barriers and the continued need for better recognition and fair access for women and girls, we're moving forward together. The sense of unity, purpose, and possibility was palpable.

Powerful Sessions & Cutting-Edge Conversations

The symposium was packed with dynamic sessions focused on the future of care, holistic wellness, and personal empowerment. Over a decade ago, novel therapies for hemophilia B began changing the landscape. In 2025, we saw even more FDA-approved products or approvals on the horizon, some within weeks of the symposium. The excitement was real.

The exhibit hall was bustling, became a hub of learning, sharing, and laughter, with a special moment when young children delivered handmade thank-you notes to our exhibitors, a heartfelt gesture that reminded everyone why we do this work.

Some sessions included:

- Hands-on CPR training
- Women's and men's gatherings
- Teen sessions and teen outings
- Inhibitor discussions
- Live patient panels, offering raw, honest stories about new therapies











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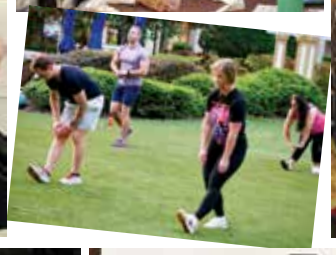




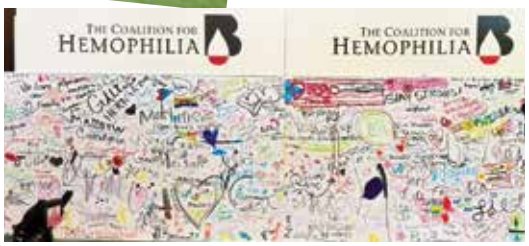
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"Processed Foods"
RESULTS





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Every morning began with tai chi and family fitness sessions, keeping mind-body wellness at the center. And our first-ever Advocacy Booth, Women's Booth, and Nutrition Booth were major highlights, offering targeted resources and support.

A Community of Leaders, Teachers & Healers

We were proud to honor Chad Stevens, Laura Echandi, and Leslie Starks during our *Volunteer Recognition Ceremony*, three individuals whose devotion and kindness have strengthened this community over the years. Thank you for all you do!

The weekend also featured an incredible roster of speakers and thought leaders:

- Dr. Chris Walsh, our beloved keynote, shared a brilliant overview of treatment advancements, describing today's options as a "menu" and reminding us just how far we've come.
- Brian O'Mahony flew in from Ireland to moderate a powerful gene therapy session, offering information, perspective and inspiration.
- Jeron Hill, always a community favorite, delivered his iconic *Brave is in Our Blood* session along with an important discussion on Carriers Count, highlighting bleeding symptoms and advocacy for women and girls.
- Wendy Wollner led an uplifting session on wellness, teaching us to find meaning in life's cracks and embrace the journey.

- Catherine Canadeo gave an energetic and informative session on nutrition, reminding us that how we fuel our bodies matters for long-term health and healing.
- Douglas Stringham hosted a powerful kinesiology session focused on caring for your joints and pain.
- Hope Woodcock-Ross guided attendees through *Infuse with Hope*, a beloved session on the emotional and ritual aspects of infusion.
- Donnie Akers led a crucial talk on financial pitfalls and smart strategies, giving attendees real-world tools to stay on solid ground.
- Mark Skinner provided a thoughtful perspective on the future of hemophilia from lived experience and leadership at the international level.
- Jim Romano, Congressman Erik Paulsen, and Dane Christensen teamed up for a jam-packed advocacy session titled *Voices in Action: From Congress to Community*.















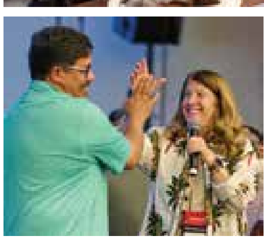

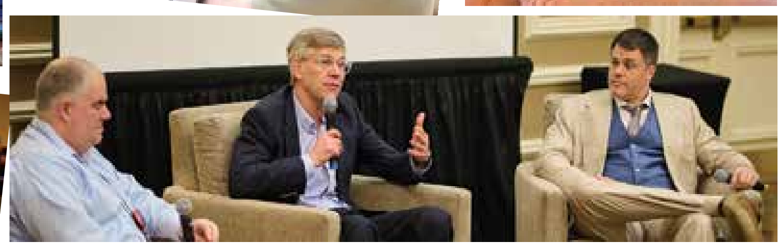



More Highlights That Made It Unforgettable

- Dr. David Clark moderated a key panel on the treatment landscape, giving attendees a clear picture of what's available now and what's next.
- Dr. April Willis delivered a dynamic session on career development and networking, helping people expand their professional horizons with confidence.
- Deena Lipinski empowered the audience with a step-by-step guide to becoming an educated and effective advocate.
- Natalie Sayer, a gifted storyteller and leadership coach, helped us navigate uncertainty with grace in her session Finding Light in the Forest.
- Karen Boyd and David Rushlow co-led a joyful and practical session on *Embracing Aging: A Positive Approach to Life After 50*.
- Kimberly Haugstad, Nelly Miranda, Jim Romano, Phil, and Shadae came together for a heartfelt talk on walking side by side to address barriers and improve fair access across hemophilia B care.
- Parker Faghan, one of our community's shining young stars, gave a standout talk on *Dream It, Build It: Entrepreneurship Without Limits*, a message of empowerment for the next generation.
- Kevin Harris led a hands-on session on wilderness survival and first aid, teaching vital, real-world skills with humor and heart.

There were so many wonderful speakers, each bringing their own energy, insight, and inspiration. We truly wish we could highlight them all here, every single one made a lasting impact. And for those who couldn't attend in person, we kept our promise and offered a full livestream so that everyone, no matter where they were, could still be part of this special experience.

Connection, Celebration and Pure Fun

Beyond the breakout sessions and panels, the symposium was filled with joy:

- The Bleeders Band brought down the house during Friday night's community hangout.
- Elec Simon kicked things off by teaching rhythm and teamwork to young attendees with his energizing drum jam session.
- The ice cream social was a sweet treat for everyone,












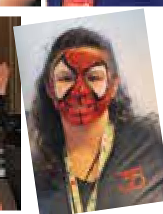

















LIKE A PRO

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bringing together families, teens, kids, and adults for a fun and relaxed time to connect.

The teen program was a highlight of the weekend and reflected our belief that every city we visit deserves something in return. The teen trip included volunteer work, because we believe in giving back wherever we go. This year's adventure began with a hands-on service project at *Keep Orlando Beautiful*, where teens joined forces to protect and restore the local environment.

Then it was time to launch into discovery at *WonderWorks*—a space where science, creativity, and adventure collide! Teens stepped into the shoes of astronauts, challenged their senses with mind-bending illusions, and defied gravity through interactive exhibits. From hurricane-force winds to astronaut training simulations, it was a day of exploration, teamwork, and unforgettable fun. This wasn't just a trip, it was a mission to make an impact, push boundaries, and experience the extraordinary.

For Saturday's *Final Night* event, we turned back the clock for a *Totally '80s Dance Party*! From Michael Jackson and Madonna impersonators to neon outfits, leg warmers, and disco lights, it was the perfect way to end a powerful weekend. Laughter echoed, dance floors filled, and community shone.

Gratitude & Looking Forward

We are so grateful for every single person who joined us, patients, caregivers, speakers, exhibitors, sponsors, staff, and volunteers. This year wasn't just about celebrating 35 years, it was about building on that legacy to create something even stronger, more inclusive, and more empowering for the future.

Whether you joined us to learn, lead, share, connect, or just feel a little less alone, you were in the right place. And we will always meet you where you are, with compassion, community, and care.

Save the Date: **Symposium 2026, April 9-12, 2026**
Viva Las Vegas! Nevada

We'll see you there!

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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

LET'S PLAY NINE

BY KIM PHELAN

The day before our Symposium our team hosted a wonderful golf fundraising event to raise funds that help equip children with golf clubs and lessons in their hometowns. We believe golf is a healthy and empowering sport for individuals with hemophilia and this event brought that event. We're especially grateful to Perry Parker, who shows up year after year to share his time, energy and clinic with expert golf tips (not to mention excellent group selfie taker) with our community. Forty-four patients, families, and sponsors gathered from across the country, making this event not only impactful, but truly special. A heartfelt thank you to our incredible volunteer, Hope Woodcock-Ross, whose dedication and presence each year help keep everything running smoothly, and to all of our volunteers: we truly couldn't do this without you!

Thank you to our sponsors for making this event possible!








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MAY MEETINGS ON THE ROAD: BUILDING TRUST AND TEAMWORK IN ST. LOUIS AND SAN ANTONIO

BY ROCKY WILLIAMS

This May, The Coalition for Hemophilia B brought our Meetings on the Road to two incredible cities: St. Louis on May 3rd and San Antonio on May 17th. These gatherings were more than just informative. They were filled with laughter, connection, teamwork, and unforgettable moments that reminded us why coming together matters so much.

We kicked off with a jolt of joy thanks to Dr. Robert Friedman's "Shake, Rattle & Roll" session. It got everyone up and moving, dancing, shaking off nerves and starting the day with big smiles and good energy. It was exactly what we needed to dive in.

One of the most powerful parts of the day was the Rap Circle. I had the honor of leading and the conversations that unfolded were incredibly moving. This space gave families the chance to truly connect, sharing stories, experiences, and insights that deepened our sense of community.

There were so many great educational sessions throughout the day at both locations. We heard fantastic presentations from Dr. Guy Young and Kim Schafer on which sparked thoughtful questions and gave families a clearer understanding of the evolving treatment landscape. We also learned about bleed control and infusion frequency and spent time focusing on physical wellness. After lunch, we kept that momentum going with energizing stretch sessions that had everyone moving, loosened up, and recharged for the rest of the day.

The tweens/teens had some unforgettable adventures of their own. In St. Louis, they spent the afternoon exploring the Science Center, diving into interactive exhibits and enjoying time to connect beyond the main event. In San Antonio, the group headed to the Aquarium, where they laughed, learned, and made memories among stingrays and sea creatures. These outings brought an extra spark to the weekend, giving them a fun and meaningful experience together.

Meanwhile... the game was afoot! Throughout the day, we leaned into our inner detectives with a Sherlock Holmes inspired adventure. The mystery continued with ongoing Clue Challenges that had everyone piecing together puzzles, following leads, and cracking codes. It was clever, fun, and full of "aha!" moments. More importantly, it brought people together in the best way. Watching teams encourage one another and celebrate each breakthrough was a highlight of the entire day..

We closed with wellness sessions that left a lasting impact. In St. Louis, Alesia Clardy offered simple, meaningful strategies for building a more balanced lifestyle. In San Antonio, Bill Patsakos shared valuable insight into nutrition and supplements for bleeding health. Both sessions gave everyone practical tools and a sense of empowerment to take home.

We appreciate the amazing speakers, exhibitors,

St. Louis, MO

San Antonio, TX

volunteers, and families who brought their energy and heart to the day. We're also incredibly grateful to the local chapter organizations who joined us, your presence helped make the day more enriching and engaging for everyone.

A heartfelt thank you to CSL Behring for making this possible. Because of your support, we're able to travel across the nation, meeting patients where they are, providing education, and bringing the community together to grow and thrive. We are truly grateful for your belief in the strength and spirit of our community.

From Missouri to Texas, these gatherings showed us that when we connect, we grow. We learn. We laugh. And sometimes, we even solve a few mysteries along the way.

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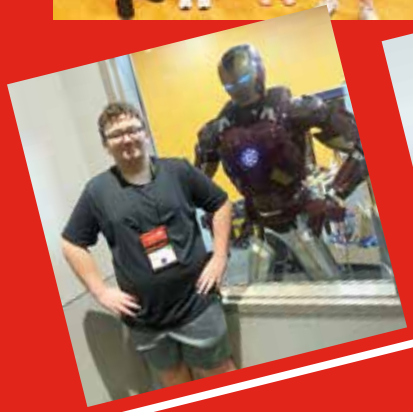
ST. LOUIS, MO


SAN ANTONIO, TX




HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

Real-World Unmet Needs of Hemophilia Patients

5/5/25: The explorer6 study was set up by Novo Nordisk to collect information about the current state of hemophilia patients around the world. Novo used the data as a comparison baseline for their Phase III study of concizumab (now Alhemo), but it also gives the community some reliable information about the experiences of hemophilia A and B patients, with or without inhibitors, in today's world. A group of the explorer6 researchers recently published the findings.

During the study, the participants maintained whatever treatment regimens they had been on, either prophylaxis or on-demand treatment. They had 72 hemophilia B subjects without inhibitors (19 on-demand; 53 on prophylaxis) who had factor IX levels up to 2% plus 31 B inhibitor patients of any severity (14 on-demand; 17 on prophylaxis). They intentionally increased the number of inhibitor patients in the study because a random selection would have resulted in very few B inhibitor patients. One of Novo's primary aims in developing Alhemo was to develop better treatments for inhibitor patients.

The average annualized bleeding rates (ABRs) for the non-inhibitor hemophilia B patients was 2.2 for prophylaxis and 10.5 for on-demand treatment. The average ABRs for the hemophilia B patients with inhibitors was 12.4 for prophylaxis and 9.3 for on-demand treatment. These results agree with the idea that prophylaxis is far superior to on-demand treatment for non-inhibitor patients. Interestingly, though, the on-demand inhibitor patients had a lower average ABR than those on prophylaxis. For comparison, the averages for the hemophilia A patients were 4.7 and 21.5, respectively, for non-inhibitor patients for prophylaxis and on-demand treatment. For the A inhibitor patients, the ABRs were 10.3 and 15.2, respectively.

The authors also looked at physical activity and joint health. As expected, physical activity scores were lowest for the hemophilia B patients with inhibitors. About two-thirds of the non-inhibitor patients on prophylaxis reported engaging in sports activities. Joint scores were similar among the various hemophilia types and treatments, but overall scores were lower (better) for those on prophylaxis. The results show that the greatest unmet needs are for patients with inhibitors and those using on-demand treatment. [Wheeler AP et al., Haemophilia, online ahead of print 5/5/25]

Less Bleeding in Hemophilia B Patients on EHL Prophylaxis

2/14/25: Current information on bleeding in patients with severe hemophilia B who are on prophylaxis is scattered and inconsistent. A group of Italian researchers performed a systematic literature review and meta-analysis to better understand their outcomes. Starting from 2440 possible studies, they winnowed the group down to 42 studies that contained good information on bleeding in patients with severe hemophilia B. Meta-analysis is a statistical method to pool results from a number of independent studies to produce a better overall picture of a question than any of the single studies can provide.

They looked at ABRs for patients on prophylaxis with either standard half-life (SHL) factor IX products or extended half-life (EHL) products. EHL products were originally developed with the aim of reducing the burden of infusions, but their most important benefit has been the ability to maintain higher factor IX levels between infusions. The authors found a significantly lower average ABR of 1.29 for patients on EHL products compared to an ABR of 3.12 for those on SHL products. They also found that 53% of patients on EHL products had zero bleeding events compared with 24% of those on SHL products. In a subgroup analysis, they found no significant difference in ABRs according to age. Thus, EHL products appear to offer more bleed protection than SHL products, along with less-frequent dosing. [Franchini M et al., Haemophilia, online ahead of print 2/14/25]

Non-Inhibitor Antibodies May Affect Hemophilia Severity

10/28/24: Some patients develop neutralizing antibodies (NAs) against infused factor VIII or IX. NAs bind to the factor molecules and interfere with (neutralize) their activity, preventing them from working properly. In the hemophilia world, NAs are called "inhibitors." Patients with inhibitors have an especially difficult time with their hemophilia because typical factor replacement products for their type of hemophilia don't work for them. We know that the immune system also produces non-neutralizing

antibodies (NNAs). NNAs also bind to factor molecules but don't interfere with their activity – or at least we've always thought so.

A group from Scandinavia looked at NNAs in moderate A and B patients in their treatment centers and found some surprising results. In a study of 137 subjects, they found NNAs in 13% of their A patients and also in 13% of their Bs. The NNAs were found regardless of whether the patients were on prophylaxis or on-demand treatment and regardless of their baseline factor level. Most often the NNAs appeared after about 150 exposure-days to their product. All patients were negative for actual inhibitors.

Bleeding rates were similarly low in all patients, but joint health scores were significantly higher (worse) in the patients with NNAs. The NNA-positive patients also had undergone more frequent joint surgeries. This suggests that although NNAs don't interfere directly with clotting behavior, they may be binding at locations on the factor molecules that are important for maintaining hemostasis in the joints. The authors recommend that these findings be followed up by larger, more in-depth studies. [Maseide RJ et al., Res Pract Thromb Haemost, online ahead of print 10/28/24]

Infants with Hemophilia Lag in Gross Motor Development

1/27/25: Infants with hemophilia have difficulty with full motor development (ability to move the body) compared with non-hemophilic infants. This is thought to be due to overprotection by the parents. A group from Greece looked at infants with hemophilia compared with non-hemophilic infants who had either partial- or full-term births. They used the Alberta Infant Motor Scale (AIMS), which rates 58 motor skills performed by an infant from birth to independent walking (19 months). The results were striking.

The full-term infants (39 weeks; Group A, 1068 babies) had normal scores on average, as expected. The partial-term infants who were born more than 32 weeks after conception (Group B, 150 babies) lagged significantly behind. The partial-term infants who were born 32 weeks or less after conception (Group C, 405 babies) lagged significantly behind group B. Finally, the infants with hemophilia (Group D, 15 full-term babies) lagged significantly behind Group C. The authors conclude: "Differences in AIMS scores could be attributed to the reduction of movement activity, since infants with haemophilia are often deprived of certain positions, being held and carried in the parents' arms, as well as from free play time on the floor." [Syrengelas D et al., Haemophilia, online ahead of print 1/27/25]

Spinal Stenosis May Be Part of Aging with Hemophilia

2/5/25: Spinal stenosis is a narrowing of the space in the backbone that the spinal cord runs through. This puts pressure on the nerves of the spinal cord, which can have effects ranging from no symptoms to debilitating pain. It is a common condition of aging affecting about 4% of the general population. At EAHAD, a group of Irish researchers presented what is thought to be the first study of spinal stenosis in hemophilia patients.

In a group of 100 males, 40 years or older, with moderate or severe hemophilia, the authors identified 13 (13%) subjects with spinal stenosis, over three times the rate in the general population. They saw no difference between the moderate and severe patients but did see an increase with age. The 40- to 59-year-old group had a rate of 3.5%; the 60- to 69-year-old group had a rate of 19.2%, and the over-70 age group had a rate of 35.3%. Interestingly, they saw no significant difference between the non-stenosis and stenosis patients according to the Hemophilia Joint Health Score (HJHS), which mainly measures the condition of the large joints, such as the ankle, knee and elbow. Therefore, the HJHS, which many patients receive during their annual exams, doesn't appear to be useful for diagnosing spinal stenosis.

Although there was no significant difference in orthopedic surgeries between the two groups, the group with stenosis had a significantly older age at first surgery than the non-stenosis group. This might suggest that delaying joint surgeries may promote stenosis because the patient may be putting more stress on the back trying to limp along on bad joints.

One question this brings up is whether this has anything to do with bleeding into the joints between the vertebrae in the back. That hasn't been studied in hemophilia, but it's possible that a lifetime of subclinical (micro) bleeds in the spine could cause damage that leads to stenosis. In the general population, arthritis is a known risk factor for spinal stenosis. Hemarthrosis, which is caused by bleeding into the joints in hemophilia, has many similar characteristics to arthritis. It could similarly be a risk factor for stenosis.

This could be one more of the conditions that we're going to have to deal with as the hemophilia population ages. [European Association for Haemophilia and Allied Disorders (EAHAD) annual meeting, February 4–7, 2025, abstract PO036]

Bone Health in Hemophilia Patients

Several recent studies have looked at bone health in hemophilia patients. Both hemophilia A and B are associated with reduced bone mineral density (BMD). However, the reasons for this are not well studied and neither are the consequences. Until the 1970s, hemophilia patients were discouraged from physical activity due to concerns about increased bleeding risk. However, with today's plethora of treatment options

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making it possible, we now know that physical activity is absolutely indispensable both for general health and for minimizing joint bleeding.

3/18/25: One question is whether reduced BMD increases the risk of fractures in hemophilia patients. A group of researchers from Greece did a comprehensive review and meta-analysis of past studies. In 18 studies spanning 2007 to 2022, which included 13,221 subjects, they found that people with hemophilia had a 4.56 times greater risk of fracture than the general population. [Divaris E et al., Haemophilia, online ahead of print 3/18/25]

3/26/25: We know that regular physical activity helps to strengthen the muscles around the joints and minimize joint bleeds. A group from Germany looked at the effects of physical activity on bone and muscle health in terms of handgrip strength and hemophilia severity. Handgrip strength is a good indication of overall muscle strength. Interestingly, they found that physical activity levels do not vary significantly across the three hemophilia severities. That's actually not good news since patients of all severities only performed low-intensity activity, which is probably not enough to improve BMD. Handgrip strength was positively correlated with improved BMD, bone health and muscle mass. [Ransmann P et al., PLoS ONE, online ahead of print 3/26/25]

3/21/25: Kinesiophobia, literally the fear of movement, refers to an excessive and irrational fear of performing physical movements due to a perceived vulnerability to painful injury or re-injury. As mentioned above, until the 1970s, kinesiophobia was actually encouraged in hemophilia patients. A group from Turkey looked at kinesiophobia in terms of hemophilia management. They found that treatment adherence and physical activity tended to reduce kinesiophobia.

That physical activity reduces kinesiophobia, the fear of physical activity, may seem somewhat circular, but it reflects the opposite situation, which is that avoidance of physical activity can start a downward spiral in which avoidance of something tends to make us more afraid of that something. It has been understood in human psychology for decades that the best way to change a person's attitude is to change their behavior. [Akan DC et al., Haemophilia, online ahead of print 3/21/25]

3/7/25: Despite the apparent importance of bone health for people with hemophilia, a study by U.S. researchers shows that screening of bone health is not consistently performed at U.S. Hemophilia Treatment Centers (HTCs). With responses from 66 of the 147 current HTCs, they found that only 21 HTCs (31.8%) routinely screened for vitamin D deficiency and only nine (13.6%) regularly performed dual-energy X-ray absorptiometry (DEXA) scans on their patients. Vitamin D is important for bone health and studies have shown that not only hemophilia patients (see below), but also the general population, tend to have low vitamin D levels. A DEXA scan measures bone density by determining how much x-ray energy is blocked by the minerals in the bones. The more blockage, the higher the BMD. The authors emphasize the need for standardized screening guidelines for bone health among all the HTCs. [Citla-Sridhar D et al., Haemophilia, online ahead of print 3/7/25]

3/6/25: Another group from Turkey looked at bone health in 62 hemophilia patients at their center. They looked at DEXA scans as well as various chemical markers like vitamin D levels and calcium levels. The study included 42 (67.7%) patients with severe hemophilia, 13 (21%) moderates, and seven (11.3%) milds. A total of 85.5% were on prophylaxis and 75.4% had a target joint.

They found that 75% of the subjects had a vitamin D deficiency and 62.9% had a lower-than-normal BMD. Among the chemical markers, they found that the ratio of parathyroid hormone (PTH) level to calcium level (PTH/Ca) correlated well with BMD. Thus, the PTH/Ca ratio may be an accessible cost-effective and practical test for BMD in hemophilia patients. [Ersal T et al., Diagnostics, 15, 638 (2025)]

One final comment… There may be an interplay between physical activity and vitamin D. Our bodies make vitamin D in response to sun exposure. The lack of physical activity may mean that hemophilia patients are also not spending as much time outside in the sun. Although nowadays people can take vitamin D supplements and many exercise indoors, a prescription for good bones might be to "go play in the sun" (with sunscreen, of course).

Iron Deficiency in Women with Bleeding Disorders

3/18/25: Iron deficiency is an under-diagnosed, under-appreciated concern for many women and girls with bleeding disorders (WGBD). Physical effects of iron deficiency include reduced exercise capacity and performance, fatigue, alopecia, and restless legs. It has also been linked to anxiety and depression. In school-age children, it is linked to poor school performance and cognitive difficulties. During pregnancy, it is associated with higher rates of miscarriage, postpartum depression, and suboptimal brain development in the fetus. Women with heavy menstrual bleeding (HMB) are at greater risk for iron deficiency. One study found

that 87.5% of adolescent females with HMB had low iron levels. In spite of all this, there are no specific recommendations for screening WGBDs for iron deficiency.

A group of U.S. researchers looked in more depth at iron deficiency in WGBD. They found that only 70% of HTCs routinely screened female patients for iron levels. Only 3.6% of the patients in the ATHN data set had been tested for iron deficiency, even though 71.9% of those tested were iron deficient. They also found that Black or African-American women, women with platelet disorders, and women with HMB were most at risk.

Another issue is that many WGBD are only tested using CBC (complete blood count) and/or hemoglobin assays, which may miss an iron deficiency. They found that 41.7% of WGBD who were not anemic, had iron deficiencies that would only have been identified with more extensive testing. Finally, they found that many physicians are not up-to-date on the best methods for iron supplementation, which have been changing. Interestingly, recent studies have shown that every-other-day dosing may improve iron absorption in women compared with daily dosing. [McCormack M et al., Haemophilia, online ahead of print 3/18/25]

Lick Your Wounds!

12/19/24: When you cut yourself, do you feel a compulsion to lick the wound? Do it! Recent research has shown that saliva contains tiny particles that promote clotting. Extracellular vesicles (EVs) are tiny, hollow particles that cells release to communicate with other cells. EVs may contain proteins, nucleic acids (the building blocks for DNA and RNA) and other molecules. A European group has recently shown that some EVs in saliva contain tissue factor and factor VII, which together activate factor X to promote clotting. Factor VII activation of factor X is the first step in the normal clotting cascade, and it is independent of factor VIII or IX. That's one of the reasons that inhibitor patients are given activated factor VII (tissue factor activates factor VII) to treat bleeds, when they can't take factor VIII or IX.

An earlier study by some of the same researchers showed that pro-coagulant EVs are also present in human milk, urine, semen, and amniotic fluid. The reasons they are there are currently unknown, but don't let that stop you. We seem to have an instinctual desire to lick our wounds, and now we know why. [Thaler J et al., Blood, 144(25) 2666-2677, 2024]

HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Spring 2025
A huge amount of new product development is 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.

A number of the items below were presented at the European Association for Haemophilia and Allied Disorders (EAHAD) annual meeting, February 4–7, 2025 in Milan, Italy. Copies of the abstracts (summaries) for the presentations are available for free at https://eahadcongress.com/abstract-publication/.

Additional items were presented at the World Federation of Hemophilia (WFH) Comprehensive Care Summit: New Developments in Bleeding Disorders and MSK, April 23–25, 2025, in Dubai, U.A.E. Copies of the abstracts for the presentations are available for free at https://wfh.org/ccs2025/abstracts/.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using, including products for inhibitor treatment. The improvements include longer half-lives and delivery by subcutaneous injection. This section also includes updates on some of the current products on the market.

Medexus Reports on Ixinity Use in Children

2/4/25: Medexus markets Ixinity, a standard half-life factor IX product for treatment of hemophilia B. They recently published the results of their Phase III/IV study on the use of Ixinity in children 12 years of age and under. The study looked at pharmacokinetics, efficacy, and safety in 21 previously-treated severe or moderately-severe patients without inhibitors. Ten subjects were under 6 years of age, and 11 were between ages 6 and 12. None of the subjects under age 6 had target joints, but two of the 6 to 12 age group already had target joints. Overall, the group had an average annualized bleeding rate (ABR) of 14 bleeds per year, with one subject having had 16 bleeds in the 6 months leading up to the study.

They found an average half-life of 16.3 hours with no significant difference between the two age groups. This was lower than the 24.2-hour half-life seen in a previous study of 32 children and adolescents above 12 years of age, but it was consistent with the higher clearance rates seen for other products in young children. The average ABR for the subjects on Ixinity was reduced to 2.34 bleeds/year, with a third of the subjects having zero bleeds. The safety data were consistent with that previously seen in adolescents and adults. [Vilchevska V et al., Res Pract Thromb Haemost, online ahead of print 2/4/25]

Novo Reports on 11 Years of Experience with Rebinyn in Children

2/5/25: Novo Nordisk markets Rebinyn, an extended half-life recombinant factor IX product for treatment of hemophilia B. Rebinyn achieves its extended half-life by conjugation with polyethylene glycol (PEG). At EAHAD, they presented final results from long-term follow-up of their clinical studies in previously treated patients (PTPs) (79 subjects) and in previously untreated patients (PUPs) (54 subjects). The average age of the PTPs at the beginning of the study was 7.0 years (range 1–12). The average age of the PUPs at the beginning of the study was 0.5 years (range 0–8). All of the subjects had severe or moderately-severe (<2% FIX) hemophilia B.

The subjects achieved median ABRs of 0.47 (range 0.28–1.68) in the PTPs and 0.33 (0–1.17) in the PUPs. Zero bleeds were experienced by 16% of the PTPs and 24% of the PUPs. Adherence to the prophylactic regimen with the product was >96% in the subjects.

The product was well-tolerated. None of the PTPs developed inhibitors, while 7.9% of the PUPs developed inhibitors. No other safety concerns were identified, including in the central nervous system, liver, or kidneys. No neurological or cognitive concerns were

observed. An early concern was the effect of chronic administration of PEG from the product, but PEG levels in the subjects reached a constant level and no PEG-related concerns were observed. [EAHAD abstract PO041]

Low Incidence of Rebleeding with SEVENFACT in Inhibitor Patients

12/15/24: HEMA Biologics distributes SEVENFACT, an activated factor VII treatment for hemophilia A and B patients with inhibitors made by LFB, a French pharmaceutical company. An international group of researchers who had participated in the PERSEPT 1 study supporting licensure of the product did an after-the-fact analysis of the effect of SEVENFACT treatment on rebleeding. Rebleeding varies from patient to patient, but some studies report up to a 19% incidence over 24 hours.

The researchers looked at 465 bleeding episodes in 27 subjects over the first 24 hours after the initial treatment. They found no rebleeds in subjects who had received a 75 µg/kg initial dose, and there was a rebleed rate of 0.5% for those receiving an initial dose of 225 µg/kg. After 48 hours, the rebleeding rates were 3.2% and 5.6%. The rebleeds were treated with SEVENFACT and most were resolved with a single dose. No subjects with rebleeds required hospitalization. Although it appeared that the higher initial dose produced a higher rebleed rate, the differences were not significant. Both initial doses proved effective. [Dunn A et al., Haemophilia, online ahead of print 12/15/24]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so that 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 clotting. In a person without a bleeding disorder, the system is in balance, so it 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.

e-therapeutics Developing Rebalancing Agent Targeting ZPI

2/5/25: e-therapeutics plc, a British company, is developing ETX-148, a small interfering ribonucleic acid (RNA) (siRNA; the "s" may also stand for short or silencing) rebalancing agent to reduce the levels of the anticoagulant protein Z-dependent protease inhibitor (ZPI). siRNAs are small pieces of RNA that bind to messenger RNAs (mRNAs) to suppress production of specific proteins in the body.

In the nucleus of cells, genes are copied into mRNA, which is a template that tells the cell how to make a specific protein. The mRNA is then secreted from the nucleus into the main body of the cell where it is picked up by the protein-making machinery (ribosomes). The ribosomes use the mRNA as a pattern to tell it the order in which to string together amino acids to form a protein. siRNAs bind to the mRNA to block it from binding to ribosomes, thus preventing the protein from being made. This is the same method used by Qfitlia (see below) to suppress the production of antithrombin (AT).

At EAHAD, e-therapeutics presented results from their pre-clinical studies of ETX-148 in mice and non-human primates (NHPs). The NHP studies suggested that ETX-148 could be effective on a quarterly dosing schedule (once every 3 months) with subcutaneous injection. The mouse studies showed that it could be effective in preventing joint damage. The product was well-tolerated and showed no obvious safety concerns.

We now have yet another company targeting yet another anticoagulant. This is important, not just because we might have another product that could help a group of hemophilia patients. We may potentially learn a lot more how the various anticoagulants affect not only clotting but other body processes. Other companies are currently looking at modified versions of ZPI as potential anticoagulant drugs for use in patients with heart disease and thrombosis. The more we learn about clotting and thrombosis, the better we can help all patients with bleeding/thrombosis problems. [EAHAD abstract OR10]

Sanofi's Qfitlia (Firusiran) is Approved by FDA

3/28/25: The U.S. Food and Drug Administration (FDA) approved Sanofi's Qfitlia (fitusiran), a rebalancing agent for treatment of hemophilia A or B patients 12 years or older, with or without inhibitors. Qfitlia is an siRNA treatment that inhibits the production of the anticoagulant AT. Fitusiran is a monthly or every-other-month subcutaneous injection.

Qfitlia differs from the other two recently-licensed rebalancing agents in several ways. First, it is the only rebalancing agent currently licensed for use in both inhibitor and non-inhibitor patients. Second, it works by a different mechanism. Both HYMPAVZI (marstacimab) and Alhemo (concizumab) are monoclonal antibodies that bind to and inhibit the anticoagulant tissue factor pathway inhibitor (TFPI). Qfitlia is an siRNA that prevents the production of the anticoagulant AT. Both approaches appear to work in lowering the anti-clotting activity in the blood.

Qfitlia has a variable dosing scheme that aims to reduce the level of AT to 15–35% of normal, which requires periodic tests for AT activity using an FDA-approved assay. The dose can be adjusted by varying the amount of product injected or by varying the dosing interval to either once-monthly or once every-other-month.

New patients are tested before treatment and at 1, 3, and 6 months afterward. Once a dose is established, patients are tested annually for AT level. Breakthrough bleeds are treated with reduced doses of either clotting factor, for non-inhibitor patients, or with bypassing agents for inhibitor patients. Following the guidelines for dose levels and for breakthrough bleed treatment are important to prevent thrombosis and other safety issues.

For inhibitor patients, Qfitlia produced a median annualized bleeding rate (ABR) of 1.9 bleeds/year compared to a median ABR of 3.9 for the non-inhibitor patients. The difference occurs because the non-inhibitor patients had more traumatic bleeds, that is, they were probably more active and experienced more injuries. The difference goes away if we look only at spontaneous bleeds (bleeds with no known cause). In that case, inhibitor and non-inhibitor patients had ABRs of 2.00 and 1.86, respectively. Overall, 31.5% of subjects had zero bleeds.

For the initial clinical studies of Qfitlia in which patients were on a fixed dose, several subjects exhibited gallbladder disease or liver toxicity (elevated liver enzymes). However, in the later studies where the doses were lowered to target 15–35% AT levels, those issues were minimized. In the variable-dose studies, 3.8% of 286 patients exhibited gallbladder issues. Also in the variable dose studies, 3.4% of patients showed at least one instance of elevated liver enzymes. Physicians are warned to avoid use of Qfitlia in patients with liver impairment.

As with the other two licensed rebalancing agents (and many other hemophilia treatments), Qfitlia was only studied in men with severe or moderately severe hemophilia. However, the indication covers most people with hemophilia, male or female, of any severity. Until we know more, women and anyone with mild or moderate hemophilia should approach this with caution, working closely with their physician.

The same holds true for use of the three rebalancing agents in hemophilia patients with any kind of thrombotic disorder, since they have a potential risk for thrombosis. All three carry a warning of possible thrombotic events. The prescribing information (PI, also called the package insert) for Qfitlia states that it should not be used in patients who have natural AT levels of less than 60% of normal but does not address any other thrombotic disorders.

Qfitlia adds another product to our arsenal of rebalancing agents, two for inhibitor patients and two for non-inhibitor patients. As we found with the extended half-life products, not every product will work optimally for every patient, but the more products available, the more likely at least one will. These products should be especially attractive for inhibitor patients who have lacked good options for years. Other patients will be attracted by their ease-of-use with monthly or fewer subcutaneous injections. [Sanofi press release 3/28/25 and Qfitlia PI]

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.

Belief Biomed/Takeda Gene Therapy Approved in China

4/10/25: Belief Biomed (BBM), a Chinese company, and Takeda China announced the approval by the Chinese National Medical Products Administration of BBM-H901, BBM's gene therapy

for hemophilia B. Takeda will commercialize the product in China, Hong Kong, and Macau. BBM-H901 uses a proprietary adeno-associated virus (AAV) vector to deliver a Padua-variant factor IX gene. After 1 year of follow-up, the average ABR for the 26 patients in the Phase III study is 0.6 with an average factor IX level of 55%.

After treatment, 21 of the 26 patients had no bleeding events, and none of the subjects had any serious adverse events. BBM may pursue licensure in the U.S. or Europe, having received Breakthrough Therapy and Orphan Drug designations from the FDA and an Advanced Therapy Medical Products designation from the European Medicines Agency (EMA). [Belief Biomed press release 4/10/25]

Biocad Applies for Approval of Their Hemophilia B Gene Therapy in Russia

11/27/24: Biocad, a Russian biotech company, is developing ANB-002, an AAV5-based gene therapy for hemophilia B. They have now applied to the Ministry of Health of the Russian Federation for conditional licensure of the product based on successful Phase I/II studies. Conditional licensure allows for faster access to therapy based on sufficient clinical data. Biocad will continue with their Phase III studies aimed at achieving full licensure in the future.

Unlike most western companies who only want "perfect patients" in their clinical studies, Biocad is including subjects with risk factors that may diminish the effectiveness of the gene therapy, such as antibodies to AAV5 and/or a history of hepatitis. They hope to be able to reach more patients, many of whom have been unable to receive gene therapy. No further information is available at this time. [GXP News article, 4/17/25]

CSL Behring Reports 4 Years of Data for HEMGENIX

2/7/25 CSL Behring markets HEMGENIX, a gene therapy for hemophilia B that is delivered by an AAV vector and uses the Padua high-activity factor IX gene. At EAHAD, they presented 4-year data from their Phase III clinical study. Of the 54 original subjects, 51 completed 4 years of follow-up. Average factor IX levels were 41.5%, 36.7%, 38.6% and 37.4% at years 1 through 4, respectively. The average ABR after 4 years was 0.40, compared with 4.16 for the 54 patients prior to treatment. The ABR for joint bleeds fell from 2.34 prior to treatment to 0.09 during year 4. No patients returned to prophylaxis during the fourth year, so 94% of the subjects still did not require prophylaxis. The treatment remained well-tolerated with no new adverse reactions. Thus, the results continue to look favorable. [EAHAD abstracts PO037 and PO040]

Pfizer Discontinues BEQVEZ Gene Therapy

2/21/25: Pfizer announced that they are pulling their hemophilia B gene therapy, BEQVEZ, from the market. This is less than a year after it was approved in April 2024, but in that period of time they have reportedly received no patients for the treatment. With this step, Pfizer has now completely left AAV gene therapy, but they are collaborating with Beam Therapeutics on mRNA-delivered gene editing.

This leaves only CSL's HEMGENIX as a gene therapy for hemophilia B. Two studies, one using traditional statistics and one using artificial intelligence (AI), have shown no significant difference in efficacy between HEMGENIX and BEQVEZ. BioMarin's Roctavian gene therapy for hemophilia A has also received a tepid reception, and BioMarin has pulled back their marketing efforts to just a few countries. The reasons for the lack of interest in hemophilia gene therapies are currently being debated. [Fierce Pharma article 2/21/25]]

Ultragenyx Discontinues DTX101 Gene Therapy

4/8/25: Ultragenyx Pharmaceutical has been developing DTX101, an AAV gene therapy for hemophilia B. They recently published the results of their Phase II clinical study, which failed to achieve a 20% factor IX level in six patients. They have subsequently decided to stop the project, but state that what they've learned in their studies should help in designing future products. [Pipe S et al., Blood Adv, online ahead of print 4/8/25]

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What is HYMPAVZI?

HYMPAVZI is a prescription medicine used to prevent or reduce the frequency of bleeding episodes in adults and children 12 years of age and older with hemophilia A without factor VIII inhibitors or hemophilia B without factor IX inhibitors.

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

IMPORTANT SAFETY INFORMATION

Important: Before you start using HYMPAVZI, it is very important to talk to your healthcare provider about using factor VIII and factor IX products (products that help blood clot but work in a different way than HYMPAVZI). You may need to use factor VIII or factor IX medicines to treat episodes of breakthrough bleeding during treatment with HYMPAVZI. Carefully follow your healthcare provider's instructions regarding when to use factor VIII or factor IX medicines and the prescribed dose during your treatment with HYMPAVZI.

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

- have a planned surgery. Your healthcare provider may stop treatment with HYMPAVZI before your surgery. Talk to your healthcare provider about when to stop using HYMPAVZI and when to start it again if you have a planned surgery.
- have a severe short-term (acute) illness such as an infection or injury.
- are pregnant or plan to become pregnant. HYMPAVZI may harm your unborn baby.

  Females who are able to become pregnant:
  - Your healthcare provider will do a pregnancy test before you start your treatment with HYMPAVZI.
  - You should use effective birth control (contraception) during treatment with HYMPAVZI and for at least 2 months after the last dose of HYMPAVZI.
  - Tell your healthcare provider right away if you become pregnant or think that you may be pregnant during treatment with HYMPAVZI.
- are breastfeeding or plan to breastfeed. It is not known if HYMPAVZI passes into your breast milk.

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

What are the possible side effects of HYMPAVZI?
HYMPAVZI may cause serious side effects, including:

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

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

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

Please see the following page for Important Facts about HYMPAVZI.

Pfizer

IMPORTANT FACTS

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marstacimab-hncq
injection | 150 mg/mL

Important information: Before you start using HYMPAVZI, it is very important to talk to your healthcare provider about using factor VIII and factor IX products (products that help blood clot but work in a different way than HYMPAVZI). You may need to use factor VIII or factor IX medicines to treat episodes of breakthrough bleeding during treatment with HYMPAVZI. Carefully follow your healthcare provider's instructions regarding when to use factor VIII or factor IX medicines and the prescribed dose during your treatment with HYMPAVZI.

What is HYMPAVZI used for?

HYMPAVZI is a prescription medicine used to prevent or reduce the frequency of bleeding episodes in adults and children 12 years of age and older with hemophilia A without factor VIII inhibitors or hemophilia B without factor IX inhibitors.

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

What should I tell my healthcare provider before using HYMPAVZI?

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

- have a planned surgery. Talk to your healthcare provider about when to stop using HYMPAVZI and when to start it again if you have a planned surgery.
- have a severe short-term (acute) illness such as an infection or injury.
- are pregnant or plan to become pregnant. HYMPAVZI may harm your unborn baby.
  - **Females who are able to become pregnant:**
    - Your healthcare provider will do a pregnancy test before you start your treatment with HYMPAVZI.
    - You should use effective birth control (contraception) during treatment with HYMPAVZI and for 2 months after the last dose of HYMPAVZI.
    - Tell your healthcare provider right away if you become pregnant or think that you may be pregnant during treatment with HYMPAVZI.
- are breastfeeding or plan to breastfeed. It is not known if HYMPAVZI passes into your breast milk.

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

How should I use HYMPAVZI?

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

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

What warnings should I know about HYMPAVZI?

HYMPAVZI may cause serious side effects, including:

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

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

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

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

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

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

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

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

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women & girls with hemophilia

articles to support, educate, and empower

Award-Winning Educator Rebecca VanSant Reflects on Teaching, Family, and Hemophilia

BY SHELLY FISHER

Congratulations to Middle School Math Teacher Rebecca (Becky) VanSant for receiving the President's Award for Excellence in Mathematics and Science!

If the number line above the white board, colorful educational posters, and student work displays hadn't immediately clued me in to Becky's profession, her easy-going, soft-spoken nature and heart for middle schoolers would have given her away. In fact, the only thing we talked about more than her students were her daughters. With a board meeting just minutes away to celebrate Becky's receipt of the Presidential Award for Excellence in Mathematics and Science, she sat down with me to talk about two of the most important roles in her life: mom and educator.

Equally proud of all three daughters, Becky enjoyed sharing a little about each one of them. Her youngest is a soon-to-be college graduate following in her mother's footsteps and about to start a semester of student teaching. As a nurse, her middle daughter chose to work in the Cardiovascular Intensive Care Unit of their local hospital. The oldest of the three is currently a music therapist and owner of her own clinic offering care to neurodivergent clients of all ages from toddler to senior. Though her teaching career has been full of accolades, it was clear that Becky considered her role as mom to be the most important by far.

Speaking of those accolades, the most recent one that Becky received is actually the highest recognition in the nation for a K–12 science, technology, engineering, or math (STEM) teacher. The Presidential Award for Excellence in Mathematics and Science recognizes up to 110 teachers each year for outstanding teaching in their field of study, and Becky was among a small group of educators acknowledged by the National Science Foundation when the winners for 2021, 2022, and 2023 were announced in January of this year.

Nominated by a coworker in 2021, Becky humbly started the 3-year application process that included the creation of a teaching demo, proof of content knowledge and pedagogy, and a demonstration of her ability to meet the needs of all students. She was announced as the 2022 math winner, recognized at her state capital on STEM day, and she's hoping to attend a gala in Washington, D.C.

A educator, Becky has taught math to mostly 4th, 5th and 6th graders. "Teaching math is my jam. When I was in college, the math standards had just been published and a lot of our staff had been instrumental in their creation. We had a math grant to teach pre-service teachers the 'the new math,' so I specialized in math from the very beginning."

When an opportunity to teach math all day at a new middle school in her district opened up, she jumped at the chance. "I love elementary, but the idea of getting to teach math all day seemed great!" In a recent publication from her school district, Becky shared, "Our kids have such big ideas. They are problem solvers in so many ways," she says. "I just love them. I know a lot of people think middle schoolers are difficult, but I just love them. They are still kids. Think about it. They are only 11 or 12 years old. We expect so much of them, and all they want is for us to laugh at their goofy jokes or listen when they talk. Sometimes, maybe a hug."

Additionally, Becky said, "I just love the way they think about things, and they are so eager to share. I wish people could spend a day in the classroom and watch how the kids figure things out." As a gifted educator, it was no surprise when Becky felt that her friends would consider her strength to be compassion. "It's the center of who I am."

When she's not teaching in the summer she heads to a St. Louis Cardinals game, sews, enjoys her family and nephew, and reads historical fiction. In addition, her nest has recently become full again with her youngest back at home to complete her student teaching with a

goal to teach during the next school year. When I asked if she would be teaching at the same school as Becky, she jokingly said her daughter felt like there was only room for one teacher from their family per campus.

Becky has some future goals of her own as well. "I want to work with pre-service teachers. My classroom is an open-door, model classroom, and I love that. I also love doing professional development as well, so I think maybe my next thing will be to work at the college level. But, I am definitely not leaving this behind."

When our conversation turned to hemophilia B, Becky shared that her oldest daughter was diagnosed as an individual with severe hemophilia B at 3 months old. After a period of denial, she said that some very special people were placed in her family's path. "It was a long struggle, and I don't know what I would have done without our hemophilia people and the Coalition. All my friends within the coalition are just another family to us."

After noticing some bruising on her ribs and apparent hematomas, Becky took her infant daughter to a doctor who poked her finger to run some tests. The bleeding wouldn't stop, and the pediatrician eventually diagnosed her with a spontaneous gene mutation called a balanced translocation. With her daughter's factor IX gene intact, but in the wrong place, Becky indicated how grateful she was for the early diagnosis. At 18 months of age, her daughter started prophylaxis and has only been on recombinant factor due to her participation in the PUP study. "We got very lucky."

Grateful for the early intervention of prophy treatment for her daughter, Becky also counts the constant involvement at the Coalition for Hemophilia B (CHB) symposia as one of the main avenues of support for her family. Her favorite part about being at the CHB symposiums is "being with her people," and she recalled individuals like Carl, who let her infuse him so that she would know what it was like before infusing her daughter, and Hope, who helped her get her daughter to Camp Little Oak just for girls in upstate New York where she "learned to self-infuse and loved everything about camp."

In addition to her daughter making friends for life just months into her diagnosis, the CHB symposia allowed Becky to make connections with other parents who would be "instrumental" to her, as she sought to support her daughter and life with hemophilia B.

Becky and her family enjoyed being involved and volunteering at the symposiums as her daughter grew up. She volunteered with outings for the young children, helped with the women's group, set up the babysitting room, checked people in, and led a new parents group. "It's scary when you're diagnosed, but you have this huge group of people who are there to help you, and people are living very great, healthy, and productive lives. My daughter would tell you she doesn't love having hemophilia, but she wouldn't not have it because it's made her who she is."

When asked what advice she would give to someone who had just been diagnosed, or had a child just diagnosed, the teacher offered, "Trust your gut. You're a mom and you know best. You get to choose what works and makes sense for you. You can change your mind and that's okay. Gather all the info you can and know that your child is going to have a very full life. Don't be afraid to ask questions. Don't be afraid to address the emotional aspect with therapy if needed."

Becky wanted to mention Jill Lathrop as another mom who "rode this journey with me. I don't know what I would have done with her." After connecting on-line and emailing each other, Becky truly can't remember a time without her, or the CHB. She also mentioned Wayne, Carl, Chad, Nina and Matt as being especially encouraging and support from Kim who was there when we needed her ready to give support and Dr. Dave who gives us so much information. She noted that as a group, the CHB is filled with people who are there to help. "Always know the CHB is not an organization. It's a family. I never hesitate to call the people I meet in the middle of the night, and I hope, I am that person for others. We're all better for it."

ADVOCACY NEWS

MAJOR HEALTH POLICY CHANGES AHEAD:

WHAT THE 2025 BUDGET RECONCILIATION ACT MEANS FOR PEOPLE WITH HEMOPHILIA B

BY LEE HALL

This past May, I joined the CHB Advocacy team during a pivotal moment in health policy. Both political parties were fiercely debating H.R. 1—now known as the 2025 Budget Reconciliation Act—a sweeping piece of legislation that was signed into law on July 4th. This law brings significant changes to Medicaid, Medicare, and Marketplace health coverage that everyone in our community needs to understand.

For those who may not know me yet, I am a lifelong member of the bleeding disorder community. I grew up in an empowered home during some of the most challenging years in our history—the era of Ricky Ray and Ryan White, two young men from our community who lost their lives to HIV, contracted through contaminated factor products. Leaders like Val Bias, Dana Kuhn, Corey Dubin, Ed Burke, and Kim Bernstein inspired me to channel my passion into advocacy, working to protect America's blood supply and ensure that the sacrifices of so many were not in vain.

I cherish every member of our large, vibrant bleeding disorders community. When I first read the House and Senate versions of H.R. 1, I couldn't believe the proposed healthcare cuts would pass. Sadly, Congress did pass the bill, and the reality is sobering:

- Medicaid will face $1 trillion in cuts over the next decade.
- New work requirements will be implemented for many Medicaid recipients.
- The ACA open enrollment period will be shortened to occur from November 1 to December 15.
- Citizenship or legal status verification will be required, adding more bureaucratic hurdles.

Since then, I've participated in countless policy calls with healthcare experts and advocates. Many are calling this the largest loss of healthcare coverage in American history. These changes will impact millions, including those with hemophilia B and other rare or chronic conditions, regardless of gender, race, religion, or political affiliation.

I can't help but recall the difficult days our community has faced before, and I worry about what could happen if any of our friends or CHB members lose access to the life-saving treatments they need.

That's why I'm writing to you now, not to alarm, but to urge action. If you are negatively affected by this new law, do not stay silent. Share your story, post it on social media, tag CHB, or send it directly to us at advocacy@hemob.org, Email your representatives. Your voice matters, and there are still actions we can take as a community.

Stay connected with CHB and check emails, texts and our website for advocacy updates. Be aware of key dates: many changes from the 2025 Budget Reconciliation Act will begin as soon as August 2025. Also, monitor your state's healthcare policies, as local changes may further impact your coverage.

We will continue to provide updates and resources to help you understand how these policy changes may affect your access to care, affordability, and the stability of your health coverage.

Together, we can face these challenges and continue to advocate for the health and well-being of everyone in the hemophilia B community.

You are not alone. We are here with you through these challenging times, so please, do not stay silent. Reach out and connect with one of our dedicated staff members whenever you need support. Get Involved and Stay Informed: Register for our upcoming events at hemb.org.

We'll provide up-to-date information and resources to help you navigate these changes. If you have questions or needs before our next meeting, please don't hesitate to email or call us. Your voice and your well-being matter to us. Together, we are stronger. Let's continue to support one another and make sure every member of our community feels heard, informed, and empowered.

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

Together, they're the Heroines of Hemophilia B.

Share your stories at

HeroinesofHemophiliaB.com

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NO BULL: WISDOM FROM A HEMO FARMER

AN INTERVIEW WITH CHRIS TEMPLIN

BY RENAE BAKER

"I would rather spend time with my family at an animal show, covered in animal smell and dirt than at a black-tie event or a fancy restaurant," says Chris Templin.

That's not hyperbole. This chicken cook-off winner is speaking from his experience working the family farm with his wife, Amy, and children Colby and Allyson. The farm, which boasts chickens, Ayreshire cows for dairy and beef, swine, rabbits, and goats, is located in eastern Pennsylvania.

Although Chris lives and works on the farm, which alone amounts to a full-time job, he is also a consultant within the chronic disease world. He does not shy away from complicated conversations. He's on a mission to improve the quality of life for all in the bleeding disorders community. To that end, he sows seeds of reform within the leadership councils, which has reaped him a reputation as a rabble-rouser. He's willing to take the heat. "Words are just words," he says.

"I call myself a concerned, compassionate person with hemophilia, because I live with it." He feels that its important to be compliant with your treatment regime.

Chris likes to draw attention to situations and offers solutions at government and community levels. "I consider myself privileged to have the opportunity to travel around the country where I meet, educate, and advocate for people with bleeding and other disorders requiring plasma-derived medicinal products."

Meanwhile, back at the ranch, for our interview, Chris took time out from getting the family's animals groomed and a new trailer outfitted with lighting and rubber matting for his kids to take to the Pennsylvania Farm Show. Colby will be the first to head out. At 19, he is a farmer and truck driver. Chris, Amy, and Allyson, who is also a farmer and belongs to five 4-H clubs, will follow.

"It's supposedly the largest agricultural event in the country to take place under one roof," explains Chris. "It's a big 11-day deal—like a state fair without the rides." Animals, exhibits, and locally-grown foods are the main events. "The big thing is the milkshakes," he adds with a telltale twinkle in his eye.

When Chris, who was diagnosed with moderate hemophilia B at age 2, considered marrying Amy, he wasn't sure he wanted a big church wedding. He told her, "If we have huge medical bills, we may have to get divorced. You're not gonna go down with the ship. It's gonna be women and children first. You're gonna be the woman from Titanic on the life raft, and I'm gonna sink down to the bottom."

Thankfully, it hasn't come to that. Although Chris has had insurance insecurities and financial hardships that can accompany life with hemophilia B, the family is, blessedly intact. Chris has survived two bankruptcies due to cataclysmic medical bills."That was really tough. I have a very expensive condition," Chris says soberly, referring to the difficulty of retaining health insurance.

"I have had good health insurance most of my life. But," he qualifies, "you've got to be a doctor, a lawyer, and an insurance expert to read the insurance contracts and know what the stuff does."

When Chris and Amy were blessed with two children, they understood that their daughter might be an obligate carrier, but they were surprised when she was diagnosed with mild hemophilia B. "I'm moderate, but I bleed like a 'severe.' She's mild and bleeds like a true 'mild,'" Chris relates. He can talk up a storm about phenotypes and how they affect individual bleeding patterns, despite one's diagnosis. The point is that everybody is different and needs their own, unique plan. The education is available through events offered by the Coalition for Hemophilia B (CHB) and other organizations.

Chris shares he had an accident and ended up in the trauma center. They wouldn't let my wife bring the medicine in. They said, 'We can't allow you to bring it in. We'll have to find some on our own shelves.' Well, they didn't have any, and there wasn't any within 80 miles of the place. Knowing the importance of infusing right away Chris contacted his hematologist to work with the trauma center to educate them and he infused himself.

"Every situation is different sometimes you can go to a ER and they don't mind if you brown bag it (bring your own factor) other times you have a situation where clearly only education is gonna to help get things done timely." He said that there are people who need more education, and he wants them to experience the same relief he enjoys due to informed emergency planning.

"My support system around me knows what to do in the event I can't speak for myself. Or if my daughter gets hurt, and somebody has to take her to the hospital, they know what medicine to give, how much to give, where to go, and what to do. But even knowing all of that doesn't guarantee that the people on the other end are gonna to listen to you. If you find yourself in an ER where the doctor won't listen to you, you're sort of out of luck. You have to advocate and either call your regular doctor and get someone who's in charge of the place to come figure it out." Ideally it's best if they work with your hematologist.

Amy makes it a habit to attend educational meetings, so she knows how to manage an adolescent girl as well as a full-grown man with hemophilia. Regarding Allyson, Chris says, so far, Allyson hasn't let hemophilia slow her down. In addition to farming and keeping up with her flock of 4-H clubs, she is a cellist and clarinet player who attended her first CHB Beats Music Program in 2023. At 13 years old, she is already eyeing a career as either a veterinarian or a teacher.

Chris takes pride in both Allyson's and Colby's aspirations and hard work. He wants his children to enjoy the fruits of their labors and the farming lifestyle by attending agricultural events where they can show off their prize-winning livestock and participate in tractor parades. "That is, when they're not out plowing down the back 40!"

As our chat ends, there is a smile on Chris' face. Sometimes family planning involves milkshakes.

YETI 2025

BY ROCKY WILLIAMS

In February 2025, it was great to attend the Youth Effectively Transitioning to Independence (YETI) conference in Portland, Oregon, hosted by Pacific Northwest Bleeding Disorders and team at GutMonkey. YETI is widely recognized as one of the best leadership and transition programs for the bleeding disorders community, setting the standard for impactful, youth-focused engagement.

This dynamic train-the-trainer weekend brought together chapter representatives, hemophilia treatment center professionals, and teens from across the country. All were united by a shared goal: to empower youth with bleeding disorders as they transition into adulthood. This year's theme focused on *Transition Plans*.

From rewriting and performing songs about teens' real-life transition journeys to working together on high ropes challenges, the weekend was filled with meaningful experiences and connection. Every activity emphasized the importance of shared decision making, clear communication, and youth leadership in managing health and independence.

More than just a training, YETI was a powerful experience that sparked fresh ideas, built real connections, and equipped us to better serve our community's future leaders.

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.

READY FOR ANYTHING: EMPOWERING FAMILIES THROUGH EMERGENCY PREPAREDNESS

BY ROCKY WILLIAMS

When the unexpected strikes, being prepared isn't just helpful, it's essential. This spring, The Coalition for Hemophilia B presented Ready for Anything: Emergency Preparedness, a virtual series designed to give families the tools, knowledge, and confidence to stay safe during any crisis. Held on February 18 and March 18, 2025, the sessions featured the passionate and highly experienced William Patsakos, PharmD, whose insights left a lasting impression on all who attended.

With his background in pharmacy, firefighting, the military, and advocacy, Bill brought a powerful and approachable perspective to emergency readiness. His message was clear: preparation saves lives. He shared practical strategies that families could act on immediately, from using fireproof safes for medications and documents to identifying vulnerabilities in their homes and routines.

The first session encouraged participants to reflect on their own preparedness. Bill explained how health, home, and safety systems are all connected and encouraged families to build personalized plans that reflect their unique needs, especially for those living with bleeding disorders.

The second session focused on safety. Bill addressed everyday risks like unattended cooking, overloaded power strips, and improperly stored flammables. He emphasized the importance of routine maintenance, such as checking smoke detectors and practicing home escape plans. His tips were simple, practical, and eye-opening.

One of the most powerful moments came during Bill's discussion of active shooter situations. Using an FBI training video, he guided families through the "Run, Hide, Fight" approach. It was a sobering topic, but one that helped families feel more mentally and physically prepared for extreme emergencies.

The third and final session in the series was held in person at this year's Symposium, bringing the conversation full circle. Attendees had the opportunity to engage face-to-face, ask questions, and continue building their preparedness skills in a hands-on environment. The in-person format added a deeper level of connection and sparked valuable discussions within the community.

What truly made the series stand out was the connection among participants. Attendees shared personal stories, asked thoughtful questions, and supported one another. As one participant put it, "This event gave me so much to think about and real tools to take action. Thank you, Bill, for all the awesome information!"

This impactful series was made possible by the generous support of CVS. Their ongoing commitment to the bleeding disorders community continues to bring important, empowering programs like this to life. We are incredibly grateful for their partnership.

If you missed the sessions or want to revisit the information, visit the CHB Education Hub (https://connect.hemob.org/) for resources and links shared by Bill. Emergency preparedness starts with awareness, and together, we're helping families feel more ready, more resilient, and more confident for whatever comes our way.

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ADVANCING INHIBITOR CARE

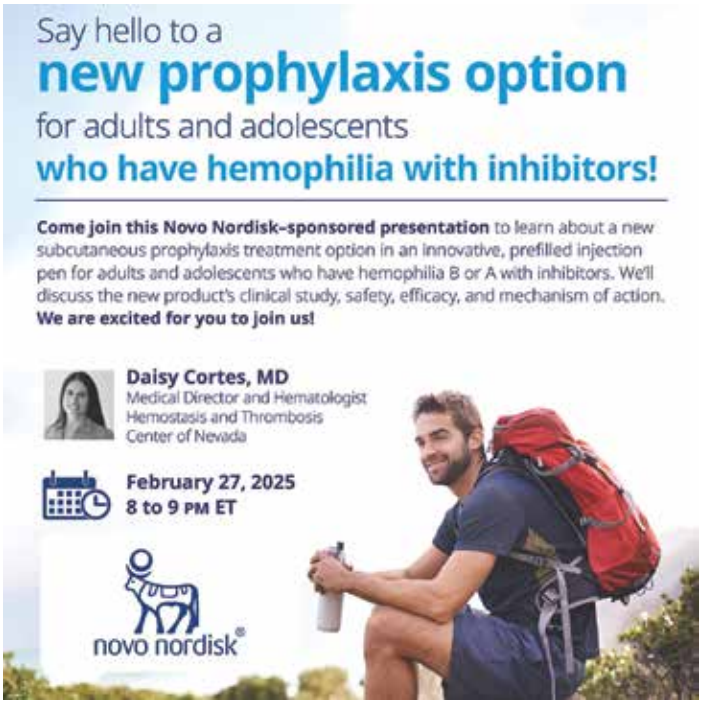
BY MARTA THOMAS

On February 27th, members of the hemophilia community came together for a sponsored event by Novo Nordisk. This was an engaging and informative session for those living with inhibitors. Presented by Dr. Daisy Cortez, a board-certified pediatric hematologist and oncologist who currently leads the Hemostasis and Thrombosis Center of Nevada.

Dr. Cortez brought a relatable and down-to-earth energy to the session, sharing both clinical knowledge and real-life insight from her years of treating patients across all age groups. The focus of the evening was Alhemo, a newly FDA-approved treatment for people with hemophilia A or B who have inhibitors.

Alhemo is a daily prefilled pen that's injected under the skin. Dr. Cortez explained how Alhemo works using the clotting cascade as a visual, like a line of dominoes where each one needs to fall in the right order. Alhemo helps remove the blocker that keeps the dominoes from falling, making it possible for clotting to happen even without factor VIII or IX.

During the Q&A, community members asked thoughtful and specific questions about missed doses, combining Alhemo with other treatments, and the possibility of it being expanded for people without inhibitors. Dr. Cortez was open and honest, reminding everyone to speak directly with their hematologist about what's best for their personal care plans.



The night ended with gratitude and excitement. There was a clear sense of hope in the air as people shared their thoughts in the chat and stayed on after the presentation to connect with one another. This session that was a great reminder of how powerful it is when education, innovation, and community come together in one space.

Many thanks to Novo Nordisk for generously sponsoring this event!

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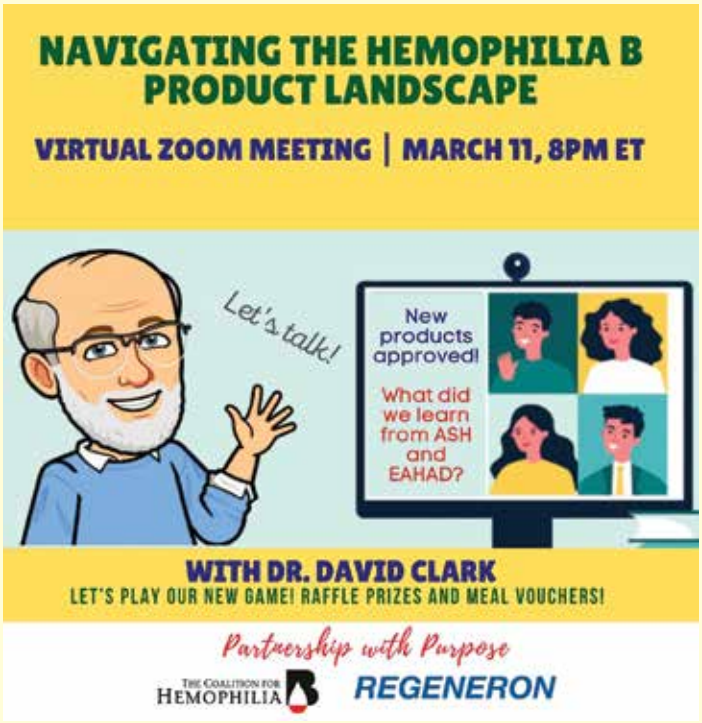
NAVIGATING THE HEMOPHILIA B PRODUCT LANDSCAPE

BY MARTA THOMAS

It's always a full house (virtually) when Dr. Dave Clark presents! His updates on hemophilia b therapies are a must see. What's new?, What's coming, How does it work?

We are incredibly grateful for his ability to take complex, hard-to-interpret data and break it down in a way that's clear, relevant, and empowering for our community.

This session on March 11 was sponsored by Regeneron, who gave a short talk. We heard from Virginie Delaware, Regeneron's Advocacy Lead, and Slate Schneider, a scientist involved in hemophilia B therapy development. They shared updates on their CRISPR-based gene insertion approach, and introduced the "Honeybee Study," which starts with an observational phase before exploring interventional gene therapy. The session highlighted how community input helped shape the trial design, underscoring the essential role of patient voices in advancing research.



The night ended on a fun and interactive note with a trivia game titled *"Are You Smarter Than Your Hemophilia B?"* written by Dr. Clark. It brought laughter, learning, and a bit of friendly competition. A raffle added a celebratory close to an evening packed with valuable insights.

From cutting-edge science to open community conversations, this session offered a little bit of everything. It was a clear reminder that while innovation drives progress, it's the people and the connections that truly shape the future of care.

Much appreciation to Regeneron for supporting this educational experience!

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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.

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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.

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Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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NEW MEXICO FAMILY EDUCATION WEEKEND

BY ROCKY WILLIAMS

In March 2025, the Coalition for Hemophilia B (CHB) was proud to exhibit at the New Mexico Family Education Weekend, held March 21–22 in Albuquerque, New Mexico. Hosted by Sangre de Oro, New Mexico's BleedingDisorders Foundation, this event brought together individuals and families from across the state's bleeding disorder community for a weekend of learning, connection, and empowerment.

Chris Villarreal and I had the pleasure of representing CHB and connecting with attendees throughout the event. It was a fantastic opportunity to share helpful resources and highlight exciting upcoming events for the hemophilia B community.

One of the highlights of the weekend was a featured presentation by our Chairman, Dr. David Clark, who gave an insightful talk on current treatments and emerging therapies for bleeding disorders. His session sparked thoughtful discussions and gave attendees a clearer understanding of what's on the horizon in care and innovation. We're grateful to Sangre de Oro and for their continued dedication to serving the bleeding disorder community in New Mexico.

HFA SYMPOSIUM 2025

BY ROCKY WILLIAMS

The 2025 Hemophilia Federation of America (HFA) Symposium took place this March in San Representing the Coalition for Hemophilia B, Dr. David Clark, Farrah Muratovic, and I had the opportunity to exhibit and engage with community members.

One of the standout moments of the weekend was the highly anticipated "Therapies on the Horizon" session on Saturday, March 29th. We were proud to see our very own Dr. David Clark take the stage as a co-presenter alongside Dr. Robert Sidonio.

Dr. Clark shared an incredibly informative presentation on the current treatment landscape for bleeding disorders, sharing knowledge that helps our community stay informed and empowered about evolving treatment options.

LEARN ABOUT HYMPAVZI™ (MARSTACIMAB-HNCQ)

BY MARTA THOMAS

On May 6th, we held a virtual event sponsored by Pfizer. The conversation around rebalancing therapy took center stage during the virtual educational session. Community members tuned in to learn more about HYMPAVZI™ (marstacimab-hncq), a newly approved treatment for hemophilia A and B, and to hear directly from Brad Schoenfeld, Director, Advocacy & Professional Relations, Rare Disease North America at Pfizer, who walked us through how the therapy works and answered questions from the group.

HYMPAVZI is approved for people with hemophilia A or B, age 12 and older, who do not have inhibitors. Brad explained how HYMPAVZI works. It blocks a protein called tissue factor pathway inhibitor (TFPI) that slows down clotting. By blocking TFPI, the body is able to form clots more effectively. It's given once a week through a subcutaneous (directly under the skin) injection using a prefilled auto-injector pen and comes in a fixed dose based on age group rather than weight.

Participants had several questions throughout the session. One person asked what happens if you miss a dose. Brad shared that while the recommendation is to stay on a regular weekly schedule, missing a dose by a day or two doesn't mean the medication stops working. However, it's best to speak with your doctor about how to proceed, and it's not recommended to double up to make up for a missed dose.

One question asked was whether HYMPAVZI can be used before surgery. Brad explained that this therapy is designed for prophylaxis, not for managing active bleeds or high-risk procedures. Anyone scheduled for surgery should talk with their treatment team ahead of time, as additional clotting support may be needed.

When asked about pediatric use and availability for people with inhibitors, Brad noted that trials are currently focused on adults and adolescents age 12 and older without inhibitors. However, additional studies are planned or already underway to expand the indication in the future. He encouraged attendees to stay connected with their care teams and advocacy organizations for updates as more data becomes available.

What stood out most was the honest and supportive tone of the conversation. Brad kept things clear and approachable while still providing the scientific detail people needed to feel informed. It wasn't just about one product—it was about helping people understand their options and feel confident when speaking with their doctors. Sessions like this continue to remind us that education and community go hand in hand.

Thanks to Pfizer for supporting this learning experience!

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FROM NEWBORN TO EMPTY NEST, PARENTING SUPPORT

BY MARTA THOMAS

The *Parenting with Hemophilia* session held on May 8th brought together caregivers from all walks of life to share their journeys and learn from one another. Thanks to CSL Behring for sponsoring this session.

What made this virtual gathering special wasn't just the information exchanged; it was the sense of solidarity that came through every story, every question, and every moment of shared vulnerability.

The evening began with Megan Carty, a seasoned caregiver and CSL Behring representative, who shared her personal journey raising two children with hemophilia. Logan, her son, lives with severe hemophilia B, and her daughter Addison has mild.

Megan walked us through the highs and lows of parenting through diagnoses, hospital visits, inhibitors, surgeries, and eventually learning to let go when it was time for college. Her honesty about the emotional toll, paired with the practical tips from years of experience, set a heartfelt and grounded tone for the night. Her family's motto, "tough times don't last, tough people do," resonated deeply with everyone listening.

From there, the conversation moved into open sharing. We heard from Nick, who adopted his son with severe hemophilia and recently learned about a rare genetic variant that shifted his son's treatment plan. Nelly talked about advocating for her son and daughter, both with mild hemophilia, and the importance of pushing past labels like "just a carrier." Debbie reflected on preparing her son, who has an inhibitor, for more independence as he transitions to a new school.

As the discussion continued, caregivers traded tips on managing bleeds, adjusting medications, and coping with the emotional hurdles that come with treatment decisions. Many spoke about the challenges of infusing their children, especially when the need for an infusion isn't apparent to the child. Others shared the relief and progress they saw once starting prophylaxis, even when it was a difficult decision to make.

One of the most uplifting parts of the night was hearing how community and camp experiences shaped these families. Parents talked about the confidence their children gained from being around others with hemophilia and how much those connections helped them feel understood and less alone. Some adults reflected on their own childhoods without that support and how determined they are to make sure their children never feel that same isolation.

Whether a child is newly diagnosed, preparing to head off to college, the takeaways were clear: trust your gut, lead with love, and don't be afraid to speak up. There's no single roadmap for parenting with hemophilia, but what this session proved is that the support and wisdom we share with each other can make all the difference.

Thank you to CSL Behring for sponsoring this important parenting session!

CSL Behring

MENTAL HEALTH ACTION DAY

BY MARTA THOMAS

On May 15th, we came together for the first session of a new series called *Mental Health Mondays*. The virtual workshop was led by somatic healing practitioner and movement therapist, Renk Koçtürk, who guided participants through simple, body-based techniques designed to ease stress and bring the nervous system back into balance.

Living with or caring for someone with hemophilia B can bring both visible and invisible challenges. While medical treatment often takes center stage, many of us carry emotional strain quietly. This session reminded us that there are small, meaningful ways to care for ourselves too, and that support can begin with a single breath.

Renk opened the space by encouraging everyone to slow down and notice how they were feeling in the moment. From there, she introduced grounding exercises like noticing points of contact with a chair, using movement to reconnect with the body, and doing gentle wrist circles or self-holds to calm the system.

These techniques were paired with breathwork like the "5-4-3-2-1" grounding method and "Take Five" breathing, giving participants tools to use during moments of anxiety, overwhelm, or mental fatigue.

One of the most powerful parts of the evening was how Renk framed the work. These practices don't need to be long, perfect, or done in silence to matter. Even a 60-second breath break in the middle of a hectic day can bring you back to yourself. As one participant shared, the session felt like a reminder that it's okay to take up space for your own healing.

This event also marked the beginning of the Mental Health Mondays series, a new offering within the B Education Hub created specifically to support emotional well-being in the Hemophilia B community. Whether you've been exploring healing practices for a while or are just starting to think about the connection between body and mental health, these sessions are designed to meet you exactly where you are.

By the end of the workshop, many participants reported feeling more grounded, relaxed, and present. And more importantly, they felt seen. Mental health often exists in the background of conversations about chronic illness, but this session brought it to the center where it belongs.

Check out recordings in our B Hub and try the Bumblee B Breathwork! Remember *Self Care is not Selfish!*

GENE THERAPY: PEER SUPPORT GROUP

BY BOBBY WISEMAN

For over the past year, a uniquely interesting peer-to-peer support group has taken place. This group has fundamental core elements: inquiry/investigation, discussion/discovery, and, dare I say, community/connecting. This particular group can be described as beyond compare, boldly brave, and blissfully balanced. This is the Gene Therapy Virtual monthly P2P gathering. A group that is quite like that well known specialty — gumbo.

Gumbo has several key ingredients, is made from a concentrated, specific process, and can be reproduced and enhanced with easily accessible ingredients. As a dish, gumbo has levels of flexibility, versatility, and adaptability that relate directly to the "cook, location, and ingredients." The monthly gene therapy group embodies gumbo in a few like and similar ways. Thanks to CSL Behring for sponsoring this program.

Each "cook/facilitator" brings to the meal his own ways and means of cooking. Each "ingredient/participant" hails from a variety of locations: rural, urban, suburban. Each "ingredient/participant" brings their unique, individual addition/contribution to the gumbo. Each "facilitator/participant" becomes a cook and an ingredient while in the supportive space. For a gumbo to "cook," a heating element, a cooking vessel, and time are needed. The heating element: conversations with. The cooking vessel: sharing experiences, expectations, and anticipation. Time: the ability to openly, safely share, exchange concerns, and foster real conversations and connections.

During the past monthly supportive gumbo gatherings there have been many "heating vessels," such as the following:

- Full belly laughs from impromptu hemophilia B comedic thoughts.
- Learning how members built functional devices/machines from "scraps and leftovers."
- Hearing of members creative insights relating to musical instruments, toothpaste (yes toothpaste), and story titles.
- Uncontrollable laughing in a "virtual space" as if physically sitting face to face in someone's home.
- Explicit sharing of one's determination and self-advocacy.
- Sharing and supporting both individual and collective thoughts and feelings.

This private monthly gathering for males 18 and over with hemophila B, continues with peers sharing and discussing the various factors, decisions surrounding gene therapy decisions.

For those who embarked on their decision to obtain gene therapy, the group was/is there along the way. For those who have received gene therapy, the supportive group was/is there. The group is a wonderful, flexibly creative, adaptable gathering that is so much more than the gumbo and provides a space to be in process, to be in decision making, to be *in* and *with* community.

SAVE THE DATE

2026 ANNUAL SYMPOSIUM

APRIL 9-12, 2026 IN LAS VEGAS

THE COALITION FOR HEMOPHILIA B

HEMOB.ORG

UPCOMING EVENTS

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

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

AUG 16, 2025
CHARLOTTE, NC
Meetings on the Road
Connect with your hemophilia B community!

AUG 26, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

SEPT 6, 2025
MILWAUKEE, WI
Meetings on the Road
Connect with your hemophilia B community!

SEPT 13, 2025
SAN DIEGO, CA
Meetings on the Road
Connect with your hemophilia B community!

SEPT 16, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

SEPT 18-21, 2025
TULSA, OK
Men's Education & Empowerment Retreat
A unique three-day experience during which men are given a safe space to share their journey with others and gain support

Sept 23, 2025 • Virtual
Hemophilia B Product Landscape
Understand your treatment options with Dr. Clark!

SEPT 27, 2025
MINNEAPOLIS, MN
Meetings on the Road
Connect with your hemophilia B community!

SEPT 30, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

OCT 4, 2025
PORTLAND, ME
Meetings on the Road
Connect with your hemophilia B community!

Oct 9, 2025
New York, NY
Eternal Spirit Awards Gala
Celebrate this year's award winners!

Oct 14, 2025 • Virtual
Strength in Connection: For those 50 and Over
A Physical Therapist Guide to Moving Better and Feeling Better

OCT 16, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

OCT 18, 2025
PHOENIX, AZ
Meetings on the Road
Connect with your hemophilia B community!

OCT 25, 2025
PITTSBURG, PA
Meetings on the Road
Connect with your hemophilia B community!

OCT 27, 2025 • VIRTUAL
Gene Therapy Rap Session
Men diagnosed with hemophilia B who are interested in, or are on gene therapy, 18 and over

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

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

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

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

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

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

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

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

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

BCares Patient Assistance Program **provides short-term, limited financial aid to our hemophilia B community members who encounter unforeseen emergencies, including COVID-19 related hardships. The charity and compassion of our BCares partners make this critical funding program possible. Thank you for your support.**

The Coalition for Hemophilia B is a national nonprofit serving the hemophilia B community for 30 years.

LEARN MORE hemob.org/bcares

PLEASE DONATE hemob.org/donate

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SCAN ME

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

WHY YOU SHOULD SAY YES TO BEING A TEEN LEADER

BY LANDON

On February 4th, I had the chance to be a teen leader for one of our B-Leader Virtual Teen Events—and it was one of the most fun and rewarding experiences I've had. From the very beginning, the energy was electric!

Everyone jumped right in during our icebreaker, where we each shared our favorite places and what we love doing there. People were laughing, opening up, and genuinely enjoying themselves. And leading it? Honestly, it was easier and more fun than I ever expected.

After the icebreaker, we moved into our rap session, always a highlight and Matt, as usual, brought great energy and joined right in. Then we jumped into some hemophilia trivia, which got everyone thinking and participating. You could really feel the teamwork and excitement building.

Next up was GeoGuessr, a game where you're dropped into a random location on Google Maps and have to use clues to figure out where in the world you are. It was the perfect mix of challenge and fun. Watching everyone work together, problem-solve, and cheer each other on made it even more enjoyable, and we did a really great job on it, too! Everyone brought their A-game, and it showed.

One of the best parts of these events is getting to see and talk with other teens from all across the country. You get to make real friends, people who understand what you're going through, and then actually see them again at other events! Every time we reconnect, it feels like picking up right where we left off. That's something really special.

If you're thinking about leading one of these events, or even just attending, my advice is simple: do it. I'd say yes again in a heartbeat. And the best part? Planning it was a breeze. All it took was two people and an idea to make something awesome happen.

B-Leader Virtual Teen Events are more than just fun. They give you the chance to connect with people you don't see every day, form real friendships, and be part of something that truly matters. And a big shoutout to Rocky—he made everything 10 times more fun and incredibly easy to pull off.

So if you're on the fence about jumping in, take the leap. You won't regret it.

FINDING STRENGTH IN COMMUNITY: A DAY OF CONNECTION, LAUGHTER, AND LEARNING

BY ISABELLE

On Thursday, May 1st, our virtual teen event brought us together from all across the county for a heart-warming, energizing experience that reminded us all why community matters so much. From the very first welcome to the final moments of laughter, this event was a powerful mix of learning, connection, and joy.

I've always believed there's something incredibly special about hearing someone else's story, and sharing your own. It's in those stories that we find belonging, strength, and the comforting truth that we're not alone. That belief rang true all day long.

Breaking the Ice

We began with an icebreaker that set the tone for the day and got to know new people. Meeting people who share similar challenges and triumphs was both comforting and inspiring. It was fascinating to hear stories from others who have navigated life with hemophilia, each tale a testament to resilience. These introductions built a sense of camaraderie and reminded us that we are not alone in our journeys.

Rap Session: A Taste of Life's Pleasures

The rap session with Matt was a highlight, featuring a lively discussion about fun and adaptable foods. Matt's enthusiasm for foods and ice cream put a smile on our faces. This session underscored how small adaptations can lead to great joys, turning meals into moments of celebration.

Trivia Trials

Next came the hemophilia trivia game, which was as humbling as it was fun. It was an opportunity to learn and laugh together. I walked away with new knowledge and an even deeper appreciation for how far our community has come. It also sparked my curiosity, especially around hemophilia history and current treatments, and turned a lighthearted game into a call to action.

The Bonding Game

The day ended on an energetic note with the James Bond-themed game. We played two games of Guesspionage. It requires two to eight players and is a percentage-guessing game. Each player, in turn, guesses what percentage of people have a certain quality or do a certain activity, such as texting while driving. Designed to test our quick thinking and teamwork, it brought out our competitive spirits in the best way. The game was not just about winning but about working together and building connections through shared laughter and strategy.

Key Takeaways

For me, the event reinforced the value of community. It was a reminder that hemophilia doesn't define us but makes us stronger and better people. It connects us in ways that can be powerful and uplifting. Sharing experiences and knowledge creates a support network that is invaluable. I know I would be nowhere near the person I am today if it were not for my bleeding disorder.

Why These Events Matter

Events like this one are more than just social gatherings; they are lifelines. They provide a platform for education, advocacy, and emotional support, helping us feel seen and understood. Before I started getting involved in events like these, I felt alone and unworthy. I based my worth on my bleeding disorder, not realizing it made me a stronger, better person. By fostering a sense of belonging, such events combat the isolation that can accompany chronic conditions. Sometimes, the first step is hard, but in the long run, you will make friends and family who change your life and give you so many opportunities.

With Gratitude

A heartfelt thank you to Sanofi for making this day possible. Your generous sponsorship helped create a space where connection, laughter, and learning could thrive. We're deeply grateful for your continued support and belief in the power of community.

sanofi

BEN'S GOT THE PEDAL TO THE METAL, AND HE'S WINNING THE RACE OF LIFE!

BY SHELLY FISHER

Ben was almost halfway into his seventh grade year, starting his sixth year of baseball and his second year of orchestra when we visited. He took time out from his basement motocross track to talk about all things Wheatland Ducks, violin, remote control cars, and what the Coalition for Hemophilia B (CHB) has meant to him and his family.

After sharing that his favorite teacher was his English language arts teacher, Mr. Husk, Ben went on to explain why, "He gives us doughnuts all the time." Turns out Mr. Husk assigns some pretty cool projects as well. "He let us design our own shoes and write a persuasive essay for a contest to win a free pair of shoes."

Ben was excited to tell me that his essay persuading someone to buy his multi-colored, marbled black and white Nike basketball shoes almost made it to the final round of the contest.

When he's not playing outfield for the Wheatland Ducks, he's practicing the violin for the school orchestra. When asked why he chose the violin, he confided, "My dad played violin." With a brother who plays the cello and a sister who will soon be old enough to start taking orchestra, the family is hoping his sister will play a stringed instrument as well. Ben shared that his favorite song to play on the violin so far is "Dragon Hunter," and he keeps Pandora on shuffle with an eclectic taste for all kinds of music including Green Day.

On any given Saturday, you might find Ben riding bikes to the gas station with his brother, tuning up his bike, or working on his remote-control cars. When we spoke, he was focused on a track that he was adding to in the family basement and dreaming of owning his own Ford Raptor one day.

Ben's answer wasn't a surprise when he was asked if he knew what he wanted to do when he got older. "I want to be a motocross racer. My grandpa in Utah is hoping to build a racetrack in his backyard." Ben is looking forward to helping his grandpa with this project as well.

He felt his friends would think of him as the "3D printer guy" because he is always making things, and he's the one who invites them to come over and race cars in the basement. In the summers, he tries to get a good game of Wiffle ball going in the backyard as much as possible. "And I win," Ben shared confidently with 6 years of baseball under his belt.

He felt he was known as the "silly guy" as well, and his favorite movie quote from Jack Sparrow in the Pirates of the Caribbean, "I have a jar of dirt," seemed to support that claim.

When asked about his diagnosis, Ben shared, "My mom is a carrier, and my

grandpa has hemophilia B so they tested me." Diagnosed at 9 months old, he didn't have any issues until he was about 3 years old. While running through the house with siblings, he ran into a door frame, hit his head, and had to ice a "goose egg" on his forehead. However, the swelling didn't subside and his mom knew it was time to get connected with the local HTC and find a doctor.

Most of the issues that Ben has experienced have been recent. About 3 years ago, he sustained a broken nose while playing shortstop for his baseball team. A player hit a line drive that dropped and bounced unexpectedly, hitting Ben in the nose. Thankfully, it stopped bleeding before he got to the emergency room where he took factor and dealt with substantial swelling.

Eventually, the doctors were able to go in and reset his nose, and Ben was cleared to play with a face mask during tournament play. Although he was not a huge fan of the face mask, he was proud to say that his team took home a second-place trophy that day. Then, just a few months ago, oral surgery complications and issues with a bracket on his braces posed a bit of a problem for Ben and he had to seek care.

With the majority of his hemophilia B related issues occurring later in Ben's life, he had some sound and timely advice for anyone who has been recently diagnosed. "Be active. Take care of your body." And he would give the same advice he gives his papa, "Wear your medic alert and don't be crazy."

Ben and his mom are no strangers to CHB events, and they had just participated in the virtual gingerbread contest when we met. Ben was proud to announce, "We got third." When I asked him if there was a theme for his house, he smiled and added, "My sister took over." Ben was also a fan of the Meetings on the Road and enjoyed a field trip to Dave and Busters where he cleaned up on the coin pushing machines and walked away with a giant Pikachu and a Space Invader stuffy for his little brother. Whether it's virtual or in person, it seems the CHB events are a family affair in Ben's house!

The CHB has impacted not only Ben and his immediate family, but his grandparents as well. In addition to meeting other families with a loved one with hemophilia B, listening to different speakers and adding to their knowledge base, Ben and his mom were most grateful for Ben's grandpa to attend. As a member of an older generation dealing with hemophilia B, it seems Ben's grandpa blamed a lot on old age that really could have been alleviated with prophylactic treatment, regular check up with a hematologist, and supporting his health more actively.

In closing, Ben wanted to give a shoutout to his mom and big brother for being there throughout his journey with hemophilia B. "She gets me to doctor appointments and helps me when I get hurt. She carried me when I couldn't walk." He also said his big brother was constantly reminding him to be careful when he was little and watching over him.

Whether it's a remote-control car racetrack, a baseball game, a string orchestra concert, or hemophilia B, Ben's got an amazing pit crew and he knows it!

CADE'S NOT SWEATING THE BIG WAVES, OR QUANTUM PHYSICS FOR THAT MATTER!

BY SHELLY FISHER

Cade visited with me from a classroom where he was nearing the end of his first semester as an engineering major, surfer, and a member of the math club. With an impending midterm a few hours away and a surf party afterwards, I was glad he took some time to visit with me about quantum physics, catching waves, and living life with hemophilia B without regret.

He shared that his coursework in applied math and computer science requires "about 12 hours a day on schoolwork," but that wasn't a surprise to Cade. "The math can be brutal, but it's also pretty cool." While currently unrelated to his major, the freshman shared that his favorite class was chemistry. "We just finished doing quantum mechanics, and I really enjoyed it. I am thinking of switching my major to engineering physics so I can work with high energy particles."

When I asked what career he was hoping to pursue, he shared that software engineering, or quantitative research finance might be options, but he'd like to stay for more academia and pursue his masters. Additionally, he is hoping to gain enrollment in a research lab opportunity.

With the beach nearby, it's not surprising that Cade somehow finds time to participate in a surf club. While intended as an opportunity to socialize, the members of this club do hit the surf regularly, and Cade has definitely honed his craft on the board. When asked to rate his surfing skills, he shared that he was "pretty good." He's no stranger to shredding, and he shared that he's conquered 6-foot waves so far. Cade humbly admits to some major wipeouts as well and counts one off the Galapagos Islands as the biggest since it landed him in the hospital. His only comment, "that was interesting," is an indicator of his laid back spirit and approach to life in general.

When he's not in search of the perfect wave, Cade enjoys a much slower-paced pursuit: math club. In addition to math seminars, his membership allows him to explore criteria for math puzzles and attempt to solve famous, unsolvable math problems. For example, finding the solution for the most widely known "P Equals No Problem (P=NP)" would earn him 1 million dollars and a free PhD. When I asked if there were particular speakers he looked forward to listening to, he shared that anything related to physics or applied math interested him.

On any given Saturday, you won't find Cade sleeping in until late. He's an early riser who likes to surf in the mornings, work on school for about 4 hours, and then play position 7, on the right wing, for an intramural soccer team.

When I asked him what he thought his friends might say about him, he offered that they might say he was outgoing, social, and, hopefully, kind, caring and honest. During our discussion about friends, Cade mentioned his chemistry teaching assistant, Billy, and wanted to give him a shoutout as someone who constantly encourages and supports other students.

Cade's mom is a carrier, so he was tested at birth and

diagnosed with moderate hemophilia B. He counts himself as pretty fortunate that his regular use of prophy prevented his need to infuse for incidents until later in high school even though he was an active participant in middle and high school sports. After years of participation in athletics, it was a "ski trick" near his home on a small hill that resulted in a broken wrist and the need for factor IX.

When I asked him what advice he would have for someone who had just been diagnosed, Cade had this to offer: "It's different for everyone, and everyone's medical needs are different. Don't be dumb with your health. Don't let it stop you. Take your prophy. Don't use it as an excuse. You'll only regret what you don't do. There are so many resources there for you. Ask questions. Get advice. Don't feel afraid to reach out to the hemophilia B community."

Cade speaks from experience when he talks about the powerful resources of the hemophilia B community and the Coalition for Hemophilia B in particular. He attended the symposium in Dallas, TX, and enjoyed the sessions, exploring downtown and catching a Ranger's game. Cade wishes he had attended one of the CHB events when he was younger. "They show you that it's normal and nothing to be embarrassed about at all."

The impact that the CHB has had on his life is evident when he sums up his experiences at the symposiums. "You meet people like you. You're part of a community. You learn a lot and there's people there that can answer your questions. The information you get on therapies is interesting."

Though Cade is adamant that the only regrets he will have are the things he doesn't do in life. He does consult with his favorite hematologists Guy Young and Wendy Leung regarding all of his physical pursuits. After making the rugby team in a level I division, both doctors asked him to consider the high potential of a concussion, which translates to 3 to 4 weeks of missed school for someone with hemophilia B, so he considered their input and weighed it against his desire to play, and both the medical and academic implications. He admired their approach and appreciated their honesty. "Basically, they said do what you want, but here's what you need to consider and plan for in terms of school."

When asked to share a song that he might consider an adequate representation of his life overall, Cade chose "Upside Down" by Jack Johnson from the Curious George movie. Evidently, he liked this song so much when he was little that his family called him "Curious Cade," and the lyrics are a fitting tribute to his view on life with hemophilia B and in general.

Who's to say I can't do everything,
well I can try
And as I roll along, I begin to find
Things aren't always just what they seem

I want to turn the whole thing
upside down
This world keeps spinning and
there's no time to waste

Well, it all keeps spinning, spinning
'round and 'round and
Upside down
Who's to say what's impossible
and can't be found?

Cade was filled with gratitude for his mom, dad, brothers, and grandparents when I asked him if there was anyone he wanted to include as being particularly supportive during his journey with hemophilia B and he said "both of my brothers know what to do to take care of me." Though he never met his maternal grandfather, he feels as though he knows him through stories told by his mom and the bond they share through their shared experience with hemophilia. "My mom has shared stories about him working feedlots and smashing his hand in a gate, or breaking his leg or back, and it was just never an option to opt out due to his having hemophilia. You just do what you need to do."

With his determination to ride every "wave" that comes his way and live his life without regret, Cade is definitely channeling his grandpa, and taking life as it comes. To use the iconic quote from Matthew McConaghey that Cade shared as his personal mantra, I think that's "Alright, alright, alright."

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Spring 2025

IN THIS ISSUE:

MEET CADE!

MEET BEN!

WANTED: TEEN CONTENT CREATORS!

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

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