THE COALITION FOR HEMOPHILIA B

SPRING 2023

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

SYMPOSIUM 2023: POWERFUL CONNECTIONS AND UNFORGETTABLE MOMENTS

NAVIGATING LIFE: A JOURNEY OF DIAGNOSIS, CARE, AND PERSONAL GROWTH

CHB LET'S PLAY NINE GOLF OUTING: CONNECTING PASSION AND GENEROSITY

MINING FOR GOLD IN THE "WHAT IFS?" AN INTERVIEW WITH KRISTIN SHELTON

CONTENTS

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.

SYMPOSIUM 2023: POWERFUL CONNECTIONS AND UNFORGETTABLE MOMENTS

BY ROCKY WILLIAMS

The 2023 Hybrid Symposium held in Orlando, Florida March 16-19th saw an impressive turnout, with more than 800 attendees in person and an additional 120 virtual attendees from all over the United States! Not only did we have community members join in from nearly every state, but we also had participants come from other countries including Ireland, Puerto Rico and Australia! We were so happy to see you all!

Coming together at Symposium is powerful, and the experiences of attendees makes the impact clear. According to Jenifer Fraker, "One of the highlights of my time at the Symposium was collaborating with Dennis (videographer) on a remarkable video project aimed at shedding light on this rare bleeding disorder and its impact on the lives of those living with it."

Jen underscores, "My involvement in the video project provided me with a unique opportunity to witness the power and significance of the Coalition for Hemophilia B firsthand. As I worked alongside Dennis, I had the privilege of witnessing members of the hemophilia B community sharing their personal stories and experiences, showcasing the profound impact that the Coalition has had on their lives. Their heartfelt testimonials not only showed the importance of this organization, but also served as a powerful reminder of the resilience and strength displayed by those living with hemophilia B."

The programs and educational sessions were topnotch. We had incredible sessions on sewing for self-care, gene therapy, emerging therapies, 504s, finances, advocacy, and so much more. We held rap sessions for our men and women, held a hemophilia B aging panel, sessions on infusion and kinesiology, and had an awesome exhibit hall. We even had a Science Fair for adults and kids alike!

This year's tween and teen programs were through the roof! They had a chance to spread their wings–and/or relax–at this year's Symposium. According to Michelle Bunk, "The teen lounge was a calming place for teens to socialize or decompress from the bustle of the Symposium. It offered a place for games, crafts, movies, and a low-stress environment. It was also a place to meet for the various activities planned for those who wanted more adventure. The teen experience offered various programs including a scavenger hunt, empowerment sessions, music, dance, physical


ORLANDO, FL 2023

therapy in the pool, a science fair, career mentorship, mental health, community service, and a wildlife park experience. Teens were able to choose the experiences that best fit their needs. As a volunteer, it was rewarding to spend time with the teens and learn about their experiences with hemophilia B."

The Symposium also provided a platform for showcasing talent. We held our annual talent show contest at Symposium, and I am proud to say that there was an amazing abundance of talent that poured into making a terrific talent show video for us to witness and to vote on. Winners were celebrated in both the youth and adult categories. Congratulations to our first-place winners, Robert on the piano and Shardonnae for her rendition of *If The World Was Ending*.

We also had the pleasure of witnessing folks getting to know CHB's Perspectives of Patients project, in partnership with Upequity. Kimberly Haugstad leads Upequity, which works to drive improved access to quality, affordable healthcare for underserved populations with rare and serious health conditions.

The Coalition for Hemophilia B and Upequity are conducting a project to hear from you about the current realities, experiences, and outstanding needs of caregivers and people with hemophilia B from underserved communities. The project prioritizes the inclusion of communities of color, women, and those living in rural areas. By gathering answers and insights, we aim to better comprehend and address barriers or gaps in healthcare access, patient-provider communications, patient advocacy organizational services & support, and patient engagement.

Kimberly commented, "We initially hoped to host three or four focus groups with a dozen participants, and we were thrilled to have conducted nine focus groups with over 40 individuals at the CHB Symposium, including one group conducted entirely in Spanish! In a project of this nature, the goal is to engage with individuals and listen deeply. You know you are on the right track when you start to hear similar statements again and again. By the end of Symposium, we were starting to get there. For me, this was a humbling and powerful experience to be a part of, and my heart was full knowing that all of this surrounded my own hemophilia B community. I am so lucky to work in rare disease!" We are grateful to our Health Equity Project sponsors CSL Behring, Pfizer and Sanofi.

The final night event was epic! We had magicians and a mime strolling through and amazing us while our Bleeders Band rocked the house! We had face painting for the kids. There was great food and lots of dancing. It was such a fun way to celebrate and capstone what has truly been one of the most incredible events ever!

Also, at the final night event, we were truly humbled to receive the Outstanding Organization Award from Danny's Dose. In recognition of Rare Disease Day, Danny's Dose honored key individuals and organizations who have worked alongside them to promote Special Medical Needs. This acknowledgment serves to highlight just how important it is to connect with each other, build community, and to learn together.

Mom and CHB volunteer Jennifer spent some time reflecting on what it all meant. "In addition to the inspiring video project, the Symposium provided an ideal setting for community members to come together and forge lasting connections. The venue itself offered designated areas for attendees to gather, exchange stories, and provide support to one another. These spaces became vibrant hubs of empathy, where individuals impacted by hemophilia B found solace and camaraderie in the company of others who understood their unique challenges."

Thank you to all our speakers, presenters, facilitators, and participants! We are grateful to our many sponsors including CSL Behring, Medexus, and Pfizer (Platinum Level), Novo Nordisk and Sanofi (Gold Level), HEMA Biologics, Takeda (Silver Level), and others. And thank you to our Virtual Sponsors!





Y.E.S.






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SYMPOSIUM COMMENTS

"I wanted to thank you and the entire CHB team for another fabulous, informative Symposium!!! We are so very blessed to be a part of this community. It was nice to meet so many new families and to be able to offer support and embrace them like we were supported when we were new to the community. This Symposium was the best ever!! You keep topping yourself!"

"Thank you so much CHB for this amazing event. The Symposium gives us joy, hope, and all the things that we need to move forward."

"I have no words to express how thankful we are for the opportunity to be at the Symposium this past weekend! We could meet other families, learn from them and enjoy so many sessions for adults & teens."

"Every family I met thought the same thing and this is all possible thanks to you and your amazing team. Thank you for all the hard work you do for this event. Thanks also for the opportunity that the Coalition gave me to participate as a volunteer."

"This is our 2nd year participating and it was incredible to see more Latino families at the event. Thanks to the opportunity you gave to Kimberly and Martha for the Latino meeting, we could exchange phone numbers to be in touch. Our Latino families suffer from the language barrier but with the help of the community, they feel a part of this big family. Please count with me in any volunteer activity I can help with."

"Thanks for such a wonderful program. My whole family has enjoyed it while picking up new information. Thanks for all the hard work!"

"Thank you for a wonderful time. I made many great connections and plan to attend again."

"Thank you so much for organizing a wonderful event! My family and I are having a blast meeting all the other community members!"

"Families living with hemophilia B will never have to repeat the words, "We just didn't know anyone with












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WILD FLORIDA

SELFIE STATION!
FAIR
SHOW

hemophilia when we were growing up," and that is due to the efforts of CHB."

"Thank you to The Coalition for Hemophilia B for a fantastic weekend of education, fellowship and recreation! We are so blessed to be part of a community that provides support, opportunity, and leading medical information. #chb23"

"When you climb in bed at almost 2:00 am with your 12-year-old and 10-year-old who had so much fun they want to wake up for breakfast to "savor every moment left," as Danny put it, you know it was a great conference!"

"We love the Symposium because we recharge our energy to continue this journey and realize that we are not alone."

"As always Kim, thanks to you and the WHOLE team for pulling together such a phenomenal meeting. So much thought and planning go into these events, and it shows. We love you all and are so thankful to have the best team around supporting our B community. Thank you!"

"We had a lovely time. To have had the pleasure to watch the coalition grow into this mega organization that we all have come to count on and learn with. What a week! Thank you all. Great to see you and everyone."
"I wanted to say from the bottom of my heart thank you for everything the last couple of days! This was our first time at the Symposium, and I left with a lot of impact on my heart. The people, the Coalition, the information, the activities, etc. I needed this and it is so much appreciated. Thank you so much."

"Thank you for the fantastic 2023 Symposium. It was a wonderfully busy weekend filled with education, meditation, art, and connecting with other hemophilia B participants. We greatly appreciate you having the Symposium remotely so that we and others who could not be there could participate. Thank you for the meal vouchers, entertainment, and all that you do for the hemophilia B Community!"

"My family and I can't thank you enough for the opportunity to come to this event. If we weren't awarded the grant, we would not have been able to financially make the trip from NY to be here. I really hope we can come again next year. The things my 7-year-old got to do, and my husband who has no family history or experience, took so much away from the sessions he attended. Again, thank you so much. Words cannot express our appreciation."

"This was the best thing for my 7-year-old. He has a port but is very eager to start learning how to access veins. He tried and accessed the vein for the very first time with the help of some very amazing nurses. This Symposium has been the best experience in meeting families and connecting with parents. My family and I can't thank the coalition enough for this opportunity!!!"

"On Friday while we were at The Coalition for Hemophilia B conference, our son self-infused for the first time. This is a HUGE accomplishment for him. As we watched him, we both were so proud of him knowing this will allow him to have so much more freedom as he gets older. To be 11 and brave enough to try after watching a couple other gentlemen with hemophilia infuse, gave him the confidence to try. We cannot thank you enough."

"My family and I enjoyed the event so much! You gave me the opportunity to do some volunteering and I'm so grateful for that, I met more families being there and the Latino families did a Whatsapp chat during the Symposium. My kids enjoyed the activities so much, including the awesome ones on Saturday. All your hard work was reflected in a wonderful event that families enjoyed so much."


Connected to milestones.
Life with hemophilia shouldn't be defined by limits. Through personalized education and empowering resources, we're focused on making more possible for you and the people you love.
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CHB LET'S PLAY NINE GOLF OUTING: CONNECTING PASSION AND GENEROSITY

BY ROCKY WILLIAMS

It was a chilly yet invigorating Wednesday morning March 15th when more than 40 community members gathered at Falcon's Fire golf course in Kissimmee for our annual Let's Play Nine Golf Outing. Held in conjunction with our Annual Symposium, the event brings together individuals who are both passionate about golf and dedicated to supporting the hemophilia B community. What a great crew to hit the green with!

Perry Parker of CSL, delivered an exceptional clinic, sharing his expertise and providing valuable tips to enhance participants' driving skills. We extend our sincere appreciation to Perry for his commitment and dedication to making this program extra special year after year. Shout-out to Hope Woodcock-Ross as well, whose unwavering generosity of time and effort has significantly contributed to the success of this event.

Huge congratulations to the first and second place teams. Your exceptional performance showcased your mighty golfing prowess!

1st Place:	*2nd Place*:
Myles G.	Wayne C.
Kevin H.	Brian S.
Dwayne H.	Craig V.
Brian R.	Kevin V.

Also, congratulations to Myles G. who achieved the "Nearest to Pin" distinction, and Kevin A., who won the award for "Longest Drive." Well done, and may your accomplishments inspire others in the community!

The success of this event not only exemplifies the power of coming together for a shared cause, but also emphasizes the profound impact we can make when we unite our passion with generosity. As we reflect on this memorable day, it's humbling to remember that every swing of the club and every generous contribution helps us continue our vital work in supporting those with hemophilia B. The funds raised from this event directly contribute to the B Cares Patient Assistance Program, providing much-needed support to individuals and families in our community.

We extend our heartfelt gratitude to all those who participated and made this day a tremendous success! A giant thanks to our sponsors, without whose support our Let's Play Nine Golf Outing would not have been possible!

THE COALITION FOR HEMOPHILIA

FOUR QUESTIONS AND EIGHT THINGS TO CONSIDER BEFORE COMMITTING TO A GENE THERAPY TRIAL

Bobby Wiseman Offers Tips from Personal Experience

BY RENAE BAKER

This interview was conducted before the approval of gene therapy and shares Bobby's experience while undergoing the trial. We will catch up with Bobby in a future issue. Stay tuned to see how he's doing!

"I think there is a strong belief in the bleeding disorder's community that if you get gene therapy, you are 'cured' and you won't have to treat anymore. That is not the case."

"Treatments of yesteryear used to be 'James T. Kirk.' Treatment today is 'Piccard,'" says Bobby Wiseman.

Yes, Bobby is a Star Trek fan.

"It's the same ship, (The USS Enterprise, i.e.: the bleeding disorder), but there are more choices on the control panel now." Some of the choices to which he is referring are the multiple product options for on-demand and prophylaxis treatments.

"It's not just a one-and-done. Back in the day, it was more restricted. You were told 'You have to do this or that.'"

Peeking out over the horizon is another option on the hemophilia B control panel: gene therapy.

At 50 years old, Bobby is an active member of The Coalition for Hemophilia B. As an African American gay man with severe hemophilia B, Bobby has found his voice advocating for more than a couple of underrepresented groups.

He is an avid researcher who tenaciously works to shine a light on important issues with which he is intimately familiar. Gene therapy is one of those issues, and he wants to help you have a well-informed experience when deciding whether a gene therapy trial is right for you.

Bobby has been taking part in a phase 2 clinical gene therapy trial since September of 2018. Deciding to participate was a big decision which he took seriously. He dove deeply into as much research on the subject as he was able. Indeed, his research began about 10 years ago when he first started hearing "rumblings" about gene therapy at national and international bleeding disorder meetings.

He opened a dialogue with his doctor about it, and they began sharing new information they learned with each other. Gene therapy was such new field that Bobby felt the need to reach a comfort level with what he was learning before volunteering his time and body to the scientific study of it.

Bobby also felt it was important to have serious conversations with family and close friends about the possibility. "It was so new," he explains, "that I wanted to tell them, 'Hey, I may need some support along the way.'" Bobby's spirituality has gotten him through a lifetime of challenges, and prayer was a big piece of his process of navigating through his fears.

On the other side of that coin was his desire to give back to the hemophilia B community and to see what gene therapy could do for hemophilia. A conversation he had with his mother helped him make his decision. That conversation helped clarify that by taking part in the trial, he would be helping other people.

He was also reminded that there were very few people of color in many of these trials, and he wanted to help add some of those data points to the research. After a few weeks of weighing the pros and cons, he decided he would like to be part of the trial. He thought he was as prepared as he could be to make this decision but would soon find himself in unforeseen situations.

He doesn't regret participating in this trial, but there are questions he wishes he had known to ask in advance. He shares his hard-won wisdom with us, so that you may make a well-informed decision, should you consider being part of a gene therapy trial.

Time commitment.

The first point Bobby wants to stress is that being in a gene therapy trial is a big commitment! "You can't schedule these visits around your calendar and location. The samples need to be drawn at the study site, because there are certain protocols around the storage of the tubes," Bobby emphasizes.

In his case, the trial is a five-year trial. Although the treatment (the vector) may only be administered one time, his commitment to be a monitored study subject will continue until the five-year mark, and maybe even beyond.

Consider the travel time involved.

When Bobby started the trial, he was living in Chico, California, and traveling the hour and half to UC Davis. The devastating "Campfire" in 2018 prompted Bobby to move his family to Phoenix, AZ. After the move, he had to fly back and forth from Phoenix to San Francisco for the study. Bobby is now able to have his gene therapy visits in Phoenix.

The study visits may be more intimate than you expect.

Bobby was taken by surprise when, in the beginning phase of the trial, he was asked to give a semen sample on site. "Yes, you may feel comfortable signing up for the study," Bobby understands, "but if you're a male in a study with traditionally female research coordinators, that may be an issue."

The frequency of visits may surprise you.

At the onset of the trial, Bobby had to be seen by the trial team weekly. That transitioned to once every other week, then every three weeks, once a month, once every other month, every four months, and then every six months. Bobby is now being seen every six months.

When does it end? "That's the unknown," Bobby cautions. He remembers back to a hep C trial he did. "It was supposed to be a five-year study, but they kept extending the deadline."

There is homework!

"I got in trouble," he admits with lingering aggravation, "because I would not fill out the diary. You had to do it daily! And by a specific time! You have to report every little condition and medication, even if it's over the counter."

Bobby was working a full-time job as a project coordinator for people with IDD and co-parenting four foster kids with his husband. The daily journaling was just too much for him, and he let them know. "It got to the point that they would email the research coordinator to ask me to do it."

Misconceptions.

There are misconceptions about gene therapy due to varying degrees of education among the general public and even within the local chapters of the various health-focused societies. "I think there is a strong belief in the bleeding disorders community that if you get gene therapy, you are cured and you won't have to treat anymore," Bobby says. "That is not the case."

When Bobby looks at his gene therapy trial experience, a key element that has been fueling it is the very interpersonal relationship he has been maintaining with his doctor and the research coordinator.

You may have to educate your health providers.

For instance, Bobby is typically prone to sinus infections. At one point, during the trial, he had to go to the ER for acute sinusitis. The doctor looked at his chart and saw that Bobby had been diagnosed with severe hemophilia b, making him unsure of what to do. It was hard for the doctor to understand that they only needed to treat the sinusitis.

You may be confused.

Being part of a gene therapy trial can be amazing and confounding at the same time. "My definition of gene therapy used to be that I wouldn't have to treat anymore," Bobby admits.

"In the trial, my levels were going up from my less than 1% and steadily increasing. My whole concept of being a "severe hemophilia B" was changing weekly. My levels were going up so much that the doctor called me to ask me if I was treating in between study visits."

As his levels were increasing, Bobby was going about living his life. "Things happened," Bobby divulges. "I was doing stuff around the house, and I got a bleed in my calf. My brain said, 'Treat!' But then I thought, 'Wait a minute. I can't do that. I'm on a trial, and I have to talk to the doctor about what to do.'"

Bobby knew if he treated it, he might invalidate the data of the trial. "So, I called the doc, and he said, 'Well we want to make sure it's a bleed.' That was one part of the conversation. And then he also said, 'We still want you to be proactive and treat it if it's a bleed.'" So, Bobby felt "stuck in the middle."

You may find yourself in unexpected emotional states.

Everything he'd been taught for so many years was shifting. Traveling was much easier. He found himself able take extended trips without worrying about factor conditions. But there is also a no man's land aspect for people who have participated or who are currently participating in gene therapy trials. There aren't established support systems for them, at this early stage, and yet, there are unanswered questions and insecurities.

What happens at the end of the trial? How do we proceed? What happens if our levels start to decline? Nobody can answer these questions. The product on trial may or may not come to market. How will that affect us?

Bobby reiterates that he doesn't regret being in this study. He is happy to be a guinea pig for the community, and he is hopeful that these trials will yield improvements for his hemophilia B community. For those who decide to take this on, he offers this advice:

"Be patient with the process and with yourself. The nurses and social workers don't yet have the tools and resources to offer their patients, which can lead to frustration."

Questions to Ask:

1. What is the actual time commitment? "
2. Will the study team be able to provide transportation to and from the study visits?
3. Who do I talk to besides the doctor, the nurse and research coordinators?
4. How will this affect my intimate relationship(s)? Bobby encourages people looking into gene therapy trials to invite your primary support person to the initial discussion sessions with the treatment center. Not only may they ask questions you may not have thought of, but it may be helpful to have their set of ears taking in the discussion about how the trial will impact your life.

Conclusion

"My fear for gene therapy," he begins, "is that when it does become a readily available product that is successful for those in the bleeding disorders community, it may be cost-prohibitive.

I'm also concerned that not all the community will have access to the information and be able to have someone advocating for them, because of cost."

Bobby's philosophy:

"Just because it's new does not make it right or wrong. Do your own research. If you don't understand it, Google it, ask your doctor, or call up your nurse hotline.

Don't limit yourself to the hemophilia B community. Reach out to other communities who are doing gene therapy and learn from their experience.

And if you do decide to participate in a gene therapy trial, know that your pioneering efforts to go 'where no man has gone before' will be a tremendous help to the bleeding disorder community and would be worthy of Captain Kirk, Piccard and even Janeway!

FIRST AND ONLY FDA-APPROVED GENE THERAPY FOR HEMOPHILIA B

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ELEVATED FACTOR IX LEVELS THAT LAST FOR YEARS

A one-time infusion delivers greater bleed protection*

Step into a new world today
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Patient portrayal; HEMGENIX not intended for women.

*In the clinical trial, annualized bleed rate (ABR) for all bleeds decreased from an average of 4.1 for patients on prophylaxis (prophy) during the lead-in period to 1.9 (54% reduction) in months 7–18 after treatment.

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.

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.

CSL Behring

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

HEMOPHILIA RESEARCH NEWS

BY DR. DAVID CLARK

Results from 11,341 Subjects Enrolled in My Life, Our Future

6/29/22 Many of you signed up to have your factor IX gene sequenced under the My Life, Our Future (MLOF) program. In fact, out of the 11,341 subjects, 2361 have hemophilia B, including three with both A and B. This is about one-fifth (20.8%) of the total number of subjects, which is consistent with the overall prevalence of hemophilia A and B. For hemophilia B, there were 1616 males (68.4%), 742 females (31.4%) and two transgender people who had transitioned from male to female. Ages ranged from less than two years (5.0%) to 65 and over (3.9%), with the largest segment in the 20 - 44 age range (35.5%).

In 2012, only about 20% of hemophilia patients had been genotyped, that is, had the sequences of their factor VIII or IX genes determined. MLOF, which ran from 2013 to 2017, significantly increased that proportion. In 2016, the program was opened to carriers, so a fairly large group of women was also included. Subjects enrolled through their HTCs, giving permission to MLOF to access their medical records and providing a sample for genotyping.

The subjects were also given the opportunity to give a blood sample to the Research Repository for future studies, which about 80% of subjects did. The medical data were connected to the patient's gene sequence, but all of the data were de-identified to protect patient privacy. All participants had both their factor VIII and factor IX genes sequenced.

The study found 431 unique factor IX gene mutations, 134 of which were novel, that is, they hadn't been seen before. They found that about 1.9% of Bs did not appear to have any mutation. All of those with no mutation were milds and moderates. Mutations were found in all subjects with severe disease.

The lack of an identifiable mutation suggests that these people's hemophilia is not caused by an issue with their factor IX. It could be caused by problems in the vitamin K-dependent processing of the factor IX protein, which is essential for clotting activity, or it could be a result of problems with other proteins with which factor IX interacts. For instance, see the article in this issue on Ehlers-Danlos syndrome, where factor IX can't bind to collagen because of a problem with the collagen, not the factor IX.

More than one mutation was identified in 95 patients, including 36 female patients. Because they sequenced both the factor VIII and IX genes of each subject, they were able to identify a number of benign mutations, mutations that don't cause disease. For instance, all the Bs had their factor VIII gene sequenced and a number of variants were found in those genes that did not appear to be harmful. Vice versa for the As who had their factor IX genes sequenced.

The study focused a lot on inhibitors, which are a big problem in hemophilia A. Fewer hemophilia B patients develop inhibitors, but when they do, the issues can be more severe. In hemophilia B, about 12% of severes had a history of inhibitors but only 1.9% of milds and moderates. Overall for all severities about 5.7% of Bs developed inhibitors. This is much higher than the UDC study from 1998 to 2011, which found an inhibitor rate of only 1.3% for Bs. The MLOF result is probably more accurate since it came from a much larger sample size.

They also looked at inhibitor development according to ethnicity and race. For hemophilia B, they found that Blacks have the highest incidence with 13.1% developing an inhibitor, while whites have an incidence of 4.9%. They also looked at Asians, American or Alaskan natives, Hawaiians and other Pacific Islanders and mixed races, but the numbers of patients were too small to show significant differences. For ethnicity, they looked at Hispanic (9.9% incidence) versus non-Hispanic (5.1%). The reasons for these differences are unknown.

The complete study article can be downloaded for free by Googling "DOI:10.1111/jth.15805", which will take you to the article referenced below. If you want to play around with the results yourself, scroll down to the bottom of the web page where you can download a supplementary Excel file containing all the data. [Johnsen JM, et al., J. Thromb. Haemost., 20:2022-2034 (2022)]

Study on the Use of Pain, Depression and Anxiety Drugs in Hemophilia

1/31/23 A recent study looked at pain, depression and anxiety in hemophilia in Nordic countries by analyzing their use of medication. Pain has been widely studied in hemophilia, but anxiety and depression much less so. The government healthcare systems in those countries make this much easier because all of the patient data is centralized in registries, unlike the U.S. They divided

3246 hemophilia patients (596 Bs, with or without inhibitors) into groups by factor consumption. For hemophilia B, the three groups were moderate-to-high (MTH) factor consumption (≥10 IU/kg/week), low factor consumption (LFC) and women

The study found that overall, hemophilia patients used more pain, depression and anxiety medications than the controls (the general population without hemophilia). This was most accentuated in the MTH group, but also in males in the LFC group and in women. Opioid use in the MTH group was 4 - 6 times greater than in the controls, and 2 - 4 times greater in the LFC group, across all age groups. The researchers conclude that this "suggests a need for improved bleed protection and hemophilia care for all severities, including mild hemophilia." [Carlsson KS, et al., Res. Pract. Thromb. Haemost., 7(2):100061 (2023)]

Thrombin Generation as an Indication of Hemophilia Severity

2/1/23 We often divide hemophilia patients into categories of severe, moderate and mild based on their level of clotting factor. However, in about 15% of patients, their bleeding behavior doesn't fit their category. We need a better system.

A group of researchers in The Netherlands have proposed that a thrombin generation assay (TGA), rather than a factor assay would be a better way to characterize patient's disorders. Thrombin (factor IIa) is the final enzyme produced by the clotting system. It converts fibrinogen (factor I) to fibrin, a protein that sticks to itself to form the clot. Everything that happens in the clotting system upstream of thrombin formation just determines how much thrombin will be made, and thus, how much of a clot will be formed.

The study looked at 446 patients with hemophilia (35 Bs), measuring their levels of thrombin generation compared to their factor levels and bleeding characteristics. They found that thrombin generation did indeed appear to be a better predictor of bleeding than did factor level.

Measuring thrombin generation is not a new idea. The problem is that TGAs are very difficult assays to run. The results vary from lab to lab and are not very reproducible. However, one of the researchers in the study is also the Chief Science Officer of Enzyre, a Dutch company that, in collaboration with Takeda, is developing a small device that could allow people with hemophilia to test their clotting status at home from a drop of blood. They have developed proprietary compounds that react with thrombin and emit light in proportion to the thrombin level. The device could help patients more precisely determine the amount of factor they need at any given time. [Verhagen MJA, et al., Res. Pract. Thromb. Haemost., 7(2):100062 (2023)]

No Difference in Quality of Life between Hemophilia A and B

2/15/23 Periodically, we see reports comparing hemophilia A and B. For scientists, that could be important for figuring out the basics of the two diseases. For the community, however, these differences sometimes seem to lead to "bragging rights." I've overheard As saying to Bs things like "we have it much worse than you Bs…". That always makes me laugh. Does having a more severe disease make you the winner?

Anyway, the results of these studies are often mixed. As have it worse in some areas and Bs have it worse in others. In other cases, there does not seem to be a difference. That seems to be the case with quality of life, based on a study from The Netherlands. Using several different measures, they found that there is no statistically significant difference between the two groups. In both cases they found that joint health was a major factor. The worse your joints, the worse your quality of life.

The issue then became, not a difference between As and Bs, but the fact that both groups, even with prophylaxis, had diminished quality of life. We've seen this kind of finding as a byproduct from a number of recent studies. We still have a way to go before all people with hemophilia can lead normal lives. [Kihlberg K, et al., Haemophilia, online ahead of print 2/15/23]

FIX, EDS and Clotting Research History

BY DR. DAVID CLARK

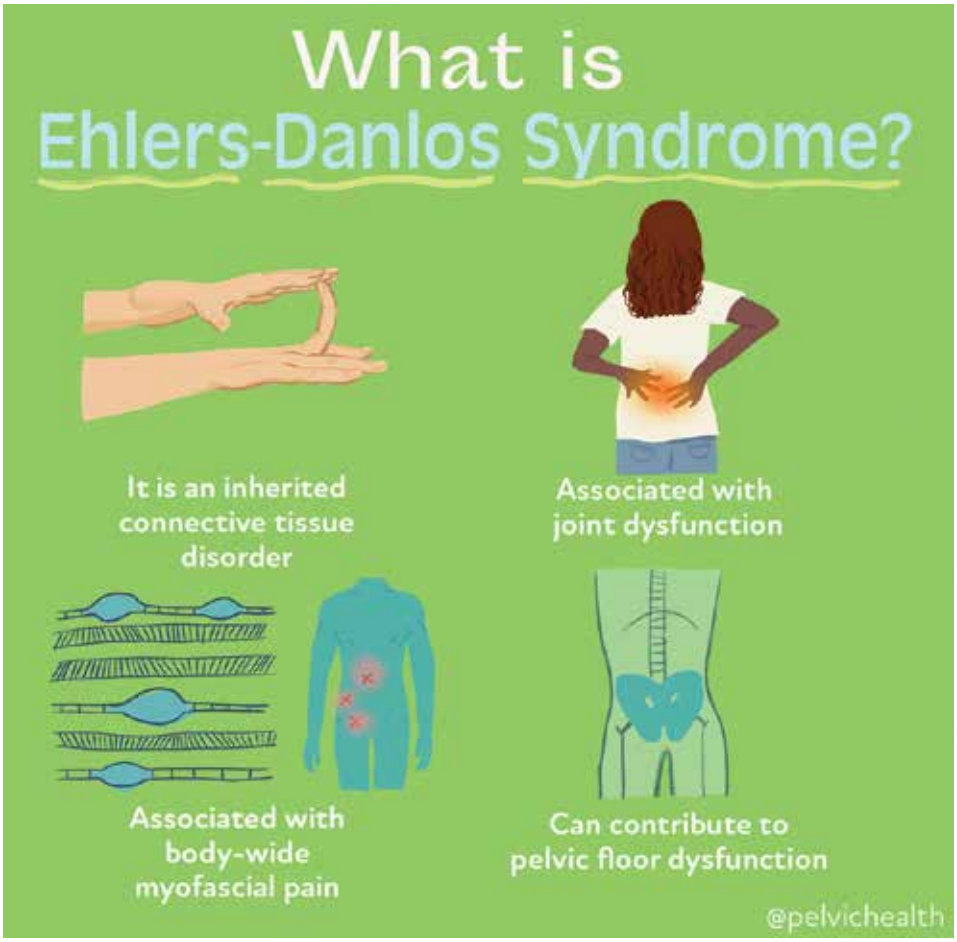
Years ago, Kim Phelan asked me whether there was a connection between Ehlers-Danlos syndrome (EDS) and hemophilia B. She asked because we seem to have an unusually large number of members who have both. I looked into the medical literature to see what I could find and found little. There might be a connection, but no one could explain it. However, new ideas about how factor IX actually works have shown that there might, in fact, be a direct connection.

EDS is a connective tissue disease. Connective tissue is "tissue that supports, protects, and gives structure to other tissues and organs in the body," according to the National Cancer Institute, part of NIH. Connective tissue is the material that makes up a lot of the physical structure of the body. Much of it is made of collagen, the main structural protein in our body. Collagen is in everything from our skin and bones to the walls of our blood vessels. It's what holds our bodies together.

Patients with EDS have mutations in their collagen genes. In the same way that hemophilia B patients have mutations in their gene for factor IX that keep the factor from working properly, EDS patients can have mutations that keep their collagen from performing correctly. Since there are several different types of collagens and it has so many uses, the mutations can cause a number of different disorders.

One possible symptom is being "double-jointed." Back in the dark ages when I was a kid, we had a girl in our class who could bend her thumb completely backward against the back of her hand. We all thought that was pretty cool, but in fact she might have had EDS. Many collagen mutations make the protein stretchier. Our classmate might have been double-jointed because the collagen in the ligaments and tendons in her hand stretched too much.

That might have seemed cool to us kids, but EDS can cause a number of not-so-cool symptoms. Easy bruising is a common result. This was thought to be a result of collagen mutations causing weak blood vessels that break easily. However, as we'll see below, some of this



bruising might arise from poor clotting. More serious outcomes of EDS include delayed wound healing, rupture of arteries causing severe internal bleeding or death, and rupture of hollow organs like the intestines or uterus.

The association of EDS with hemophilia is not new. A publication from 1960 is titled "Plasma Thromboplastin Component Deficiency in the Ehlers-Danlos Syndrome." Plasma thromboplastin component is now called factor IX, and its deficiency is now called hemophilia B. What is new is that we can now see what the connection might be between EDS and hemophilia.

Let's go back to the hemophilia side before we connect the two. Research over the past couple of decades has shown that factor IX binds inside the walls of blood vessels. More specifically, it binds to the type IV collagen that gives the vessel wall its strength. So what? We've thought all along that the factor IX in the blood vessel walls was only there as an extra supply in case we used up too much in the bloodstream. We were apparently wrong.

I got into clotting research in the early 1980s, right at the time of a revolution in how we thought clotting happened. Before that, everyone assumed that clotting happened in solution, since the clotting factors are dissolved in the blood. However, people had noticed that clotting required phospholipids.

Phospholipids are the molecules that make up the walls of cells. Researchers figured that the phospholipids came from the broken cell walls at the injury site. They floated out into the bloodstream where they could interact with the clotting factors to cause the clotting reactions.

Not so fast! Some very smart people did some extensive experiments that showed that the clotting reactions were actually taking place on the phospholipid surfaces, not out in the bloodstream. They were actually happening on the broken cell walls at the injury site, as well as on the walls of platelets and other cells. That makes sense since you need to keep the clotting reactions at the site of injury. If the reactions were happening out in the bloodstream, the clots could just float away.

We've got another revolution going on today. We're realizing that the factor IX that is bound inside the walls of the bloodstream is not just extra; it's essential. (See the article *"Where Is the Factor IX and What Does It Do There?"* in the Winter 2021 issue.)

A recent study along these lines shows that if you give hemophilia B mice a recombinant factor IX that is less able to bind to collagen, the mice don't clot as well as if you gave them normal factor IX. However, if you give them a factor IX that binds more strongly to collagen, they clot even better than with normal factor IX.

This is further evidence that the factor IX that binds to collagen in the walls of the blood vessels is not just extra material, it is important for clotting. [Machado SF et al., Thromb. Haemost., online ahead of print May 10, 2023]

Now let's tie these together. Note that this hasn't been completely proven yet, so some of it is just speculation. We're joining the scientists working on this at the edge of our current knowledge.

If you have EDS and make a mutated form of collagen, type IV that factor IX doesn't bind to, you won't have much factor IX inside your blood vessel walls. Because of the lack of factor IX in the vessel walls, you won't clot as well. You'll look as though you have hemophilia B, even if your factor IX is completely normal.

Your factor IX won't be able to do its job, not because it is faulty but because the collagen that it's supposed to bind to, won't accept it. Note that von Willebrand factor and platelets also bind to collagen, so collagen mutations could also affect them, causing other bleeding disorders.

What we're apparently seeing with EDS is that you could have perfectly fine factor IX, but have hemophilia B because your collagen isn't working right – it doesn't bind to your factor IX. You've also seen some of the scientific process. We often only learn things by baby steps. First, we had to discover factor IX. Then we had to figure out how it works. Now we're figuring out where it works and why.

Along the way, we have to make assumptions and then do experiments to show whether or not those assumptions are correct. It's a long slow process, and that's frustrating for many people. However, if we're patient, it can have a big payoff.

When I first started, hemophilia B patients were treated with Factor IX Complex, a mixture of clotting factors; we didn't even have a method for purifying factor IX. However, factor IX complex had lots of limitations. It couldn't be used for prophylaxis or surgery. Now, four decades later, we have gene therapy, a possible cure.

Where has the time gone?

EXPERIENCE 25 YEARS* MATTERS

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*BeneFix was approved February 11, 1997.

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BeneFix has been supporting individuals with hemophilia B for 25 years—and our support continues

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1997

BeneFix becomes **the first** recombinant factor IX (rFIX) treatment for hemophilia B approved by the US Food and Drug Administration (FDA).

2020

The FDA approves **BeneFix once-weekly** prophylactic use in addition to its on-demand indication.

2022

On February 11, BeneFix proudly became the only rFIX **supporting individuals with hemophilia B for 25 years**.

What Is BeneFix?

BeneFix, Coagulation Factor IX (Recombinant), is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Your doctor might also give you BeneFix before surgical procedures.

BeneFix is **NOT** used to treat hemophilia A.

Important Safety Information

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash, or hives.
- Your body can make antibodies, called "inhibitors," which may stop BeneFix from working properly.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness, and rash.

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.

Please see Brief Summary of full Prescribing Information on next page.

To learn more about once-weekly dosing with BeneFix,
visit benefix.com/about-benefix/once-weekly-prophylaxis.

PP-BEN-USA-0794

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Pfizer

BeneFix®

Coagulation Factor IX (Recombinant)

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Brief Summary

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-438-1985.

Please read this Patient Information carefully before using BeneFix and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

What is BeneFix?

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Your doctor might also give you BeneFix before surgical procedures.

BeneFix is **NOT** used to treat hemophilia A.

What should I tell my doctor before using BeneFix?

Tell your doctor and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal medicines.

Tell your doctor about all of your medical conditions, including if you:

- have any allergies, including allergies to hamsters.
- are pregnant or planning to become pregnant. It is not known if BeneFix may harm your unborn baby.
- are breastfeeding. It is not known if BeneFix passes into the milk and if it can harm your baby.

How should I infuse BeneFix?

The initial administrations of BeneFix should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

See the step-by-step instructions for infusing in the complete patient labeling.

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

Call your doctor right away if bleeding is not controlled after using BeneFix.

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

Allergic reactions may occur with BeneFix. Call your doctor or get emergency treatment right away if you have any of the following symptoms:

wheezing	fast heartbeat
difficulty breathing	swelling of the face
chest tightness	faintness
turning blue (look at lips and gums)	rash
	hives

Your body can also make antibodies, called "inhibitors," against BeneFix, which may stop BeneFix from working properly.

Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFix may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFix is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

Tell your doctor about any side effect that bothers you or that does not go away.

How should I store BeneFix?

DO NOT FREEZE the BeneFix kit. The BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Throw away any unused BeneFix and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

BeneFix does not contain a preservative. After reconstituting BeneFix, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFix if the reconstituted solution is not clear and colorless.

What else should I know about BeneFix?

Medicines are sometimes prescribed for purposes other than those listed here. Do not use BeneFix for a condition for which it was not prescribed. Do not share BeneFix with other people, even if they have the same symptoms that you have.

If you would like more information, talk with your doctor. You can ask your doctor or pharmacist for information about BeneFix that was written for healthcare professionals.

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-14.0, revised September 2021.

Pfizer

EMERGING THERAPIES

BY DR. DAVID CLARK

Spring 2023 - There is a huge amount of new product development going on in hemophilia B. The potential new products can currently 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 product.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using. The improvements include longer half-lives and delivery by subcutaneous injection. This section also contains news about some newer products that are already on the market.

FDA Accepts Medexus' Application for a Pediatric Indication for Ixinity

6/15/23 Medexus Pharmaceuticals markets Ixinity, a recombinant factor IX product for treatment of hemophilia B. It is currently only licensed for patients 12 years and older. They recently completed a Phase IV study (a clinical study that takes place after a product has already been licensed) to extend that indication to children under twelve. They announced that the US. FDA has accepted their license application for the additional application. [Medexus press release, 6/15/23]

TiumBio Developing Extended Half-Life Product for Inhibitor Treatment

4/4/23 TiumBio, a South Korean company, is developing TU7710, an extended half-life recombinant factor VIIa product for treatment of hemophilia A or B patients with inhibitors. The two factor VIIa products currently on the market, Novo's NovoSeven and HEMA Biologics SEVENFACT, both have relatively short half-lives and may need to be infused more than once-a-day for treatment of a serious bleed. TU7710 is a factor VIIa molecule fused to a transferrin molecule. Transferrin is a normal human protein that binds iron from the gut and transports it to other tissues in the body. It has a longer lifetime in circulation that is expected to give TU7710 a half-life up to seven times longer than the current products. TiumBio is currently beginning a Phase Ia clinical study of TU7710.

TiumBio is also working on extended half-life versions of factor VIII for hemophilia A and factor IX for hemophilia B, also using transferrin. The company is relatively new, but the staff have extensive experience working for SK Pharma on the hemophilia A product Afstyla. [TiumBio press release, 4/4/23]

Zea Biosciences to Make Lettuce for FIX Inhibitors

3/28/23 Back in the Winter 2016 issue, we told you about groups from the Universities of Florida and Pennsylvania (now also including a group from the University of Indiana) who were developing a factor IX product produced in lettuce. The product showed promise in eliminating inhibitors from hemophilia B patients. Since then, they have done extensive development on the project and are working toward a clinical study. They recently signed a deal with Zea Biosciences to produce the product. Zea is a specialist in producing plant-based biological medicines that was founded by Jim Wilson, one of the pioneers of gene therapy.

The idea is that tolerance, preventing or eliminating an inhibitor, can be achieved by orally exposing the intestines (gut) to the antigen, in this case factor IX. (An antigen is anything that triggers the immune system to make antibodies.) The immune system in the gut tends to produce tolerance by suppressing the regular immune system's response against the antigen. This helps us keep from developing allergies to all the foods we eat, although some people do still develop food allergies.

People have worked for decades trying to develop an oral factor IX, one that can be taken as a pill or capsule. The problem is getting factor IX into the intestines since it tends to get broken down in the stomach. So, instead of purifying the factor IX from the lettuce, they leave it in the plant's cells. Then, the lettuce is freeze-dried and crushed into a powder that is filled into capsules. The strong cell walls of the lettuce tend to protect the factor IX as it's going through the stomach, so it can make its way to the intestines intact. The researchers have shown in mice and dogs that feeding them the product can eliminate an inhibitor or prevent its formation. [Zea press release, 3/28/23 and Srinivasan A, et al., Plant Biotechnol. J., 19:1952 (2021)]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so the blood clots when it should and doesn't clot when it shouldn't. The clotting system is a complex system of clotting factors that promote clotting and 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 much anticoagulant activity keeping the blood from clotting. Rebalancing agents mainly reduce or inhibit the activity of the anticoagulants in the system. Most of these agents work to help restore clotting in people with hemophilia A or B, with or without inhibitors.

Centessa Announces Phase II Studies of SerpinPC, Plus Fast Track Designation

3/31/23 Centessa Pharmaceuticals is developing SerpinPC, an inhibitor of the anticoagulant activated protein C (APC) to control bleeding in patients with hemophilia A and B, with or without inhibitors. SerpinPC is a subcutaneous injection, once every two weeks. After good results in their Phase I/IIa study, they are now planning a full Phase II study to begin later this year. [Hemophilia News Today article, 3/31/23]

5/22/23 Centessa announced that they have received Fast Track Designation for SerpinPC from FDA. Fast Track is designed to facilitate development and expedite review of products that treat serious conditions with unmet needs. It brings more frequent meetings with FDA and possible eligibility for faster license reviews. [Centessa press release, 5/22/23]

Novo's Concizumab Approved in Canada; Delayed in U.S.

3/10/23 Novo Nordisk is developing concizumab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) to control bleeding in patients with hemophilia A and B, with or without inhibitors. Concizumab is a once-daily subcutaneous injection. On 3/10/23 concizumab, brand name Alhemo, was approved in Canada for hemophilia B patients with inhibitors over 12 years of age. Note that Canada only gave the product a limited indication. Ultimately, the product is intended for all people with hemophilia, A or B, with or without inhibitors. Canada only gave it a limited indication for treatment of B inhibitor patients, a huge unmet need. [Novo Nordisk Canada press release, 4/17/23]

4/24/23 The U.S. FDA sent Novo a Complete Response Letter (CRL) requesting more information on the product. FDA wants additional details on the manufacturing process and on Novo's plans for "monitoring and dosing of patients to ensure that concizumab is administered as intended." The FDA is presumably concerned about the risk of thrombosis, which occurred in three patients during the clinical studies, resulting in a temporary clinical hold and a revision of the dosing instructions. Since they restarted the studies, there have been no additional thrombotic events. [Fierce Biotech article, 5/4/23 and Biospace article, 5/5/23]

Pfizer Reiterates Commitment to Hemophilia

4/17/23 – World Hemophilia Day In January, Pfizer announced that it is stepping back from early-stage R&D for rare diseases. We didn't know what that would mean for hemophilia, but during a media event for World Hemophilia Day, Pfizer "reiterated its goal to make sure patients living with hemophilia are seen, heard, and never forgotten as it continues to work tirelessly to find break-through solutions and therapeutic options to change their lives." [Pfizer press release 4/20/23]

Pfizer Announces Positive Results from Phase III Studies of Marstacimab

5/30/23 Pfizer is developing marstacimab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) to control bleeding in patients with hemophilia A and B, with or without inhibitors. Marstacimab is a once-weekly subcutaneous injection. On 5/30, they announced that their pivotal Phase III study has met its primary endpoints. In 116 patients, both As and Bs, but without inhibitors, they saw a 92% reduction in the annualized bleeding rate (ABR) compared to on-demand treatment and a 35% reduction compared to prophylaxis with factor concentrates. They have not released the actual ABR numbers yet. They also saw no new safety concerns and there were no incidents of thrombotic complications.

Pfizer is currently analyzing the complete Phase III results, which will be presented at upcoming conferences, and plans to meet with FDA in the near future. They also have ongoing studies of marstacimab in inhibitor patients and in children. [Pfizer press release 5/30/23]

Sanofi Publishes Results of Phase III Fitusiran Studies

3/29/23 Sanofi is developing fitusiran, an inhibitor of the anticoagulant antithrombin, to control bleeding in patients with hemophilia A and B, with or without inhibitors. Fitusiran is a once-monthly subcutaneous injection. Fitusiran is a silent interfering RNA (siRNA) that inhibits the production of antithrombin. In the last issue, we summarized the results from their Phase III study that were presented at the American Society of Hematology (ASH) and the European Association for Haemophilia and Allied

Disorders (EAHAD) recent annual meetings. Now those results have been published in The Lancet for the Phase III study on inhibitor patients and in Lancet Haematology for the non-inhibitor patient study. [Young G et al., Lancet, online ahead of print, 3/29/23 and Srivastava A et al., Lancet Haematol., online ahead of print, 3/29/23]

GENE 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.

Belief BioMed Completes Dosing in Phase III Study

4/23/23 Belief BioMed, a Chinese company, is developing BBM-H901, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. In their earlier studies of dose exploration/escalation, ten patients with pre-treatment factor IX levels below 2% were able to stop treatment with factor IX and had annualized bleeding rates (ABRs) of zero. The product appeared safe with no serious adverse events. Now they have begun a Phase III study. [Belief BioMed press release, 4/23/23]

CSL Announces First Patient Treated with Hemgenix

6/20/23 CSL Behring announced that the first commercial patient has been treated with their Hemgenix gene therapy for hemophilia B. Also, discussions between CSL and the payer community have been generally positive with payers insuring about 60% of the U.S. population establishing clear medical policies covering Hemgenix. CSL is providing ongoing training to clinical centers, primarily HTCs, who will administer the product and encouraging long-term data collection through ATHN.

For patients who have decided, along with their physicians, to move forward with Hemgenix treatment, CSL has established the HEMGENIX ConnectSM program. They will be assigned a dedicated support team including a Patient Resource Navigator and a CSL case manager to help with questions, monitor their treatment process and help with obtaining insurance coverage. [CSL press release 6/20/23]

CSL's Hemgenix Licensed in Additional Countries

3/27/23 CSL's Hemgenix gene therapy for hemophilia B has now been approved in several other countries, including the EU, UK, Liechtenstein, Iceland and Norway. [Pharma Times article, 3/27/23]

Freeline Makes More Cuts

4/4/23 Freeline Therapeutics has been developing FLT180a, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. The treatment appeared promising, being able to increase factor IX levels into the normal range. Unfortunately, they have run into money problems and in mid-2022 decided to stop their work on FLT180a until they can find a development partner. Now, they have cut another gene therapy project for Fabry Disease and made more layoffs.

Their one remaining project is a gene therapy for Gaucher Disease. They say they have enough cash to fund operations into the second quarter of 2024. Biotech funding has become very tight, so unless something happens, this might be the end of FLT180a. [Biopharma Dive article, 4/4/23]

Genascence Developing Gene Therapy for Osteoarthritis

3/6/23 Genascence is developing GNSC-001, a gene therapy for treatment of osteoarthritis in the knee. It is delivered via an AAV vector and carries the gene for interleukin-1 receptor antagonist (IL-1Ra), a potent inhibitor of interleukin-1 (IL-1). IL-1 is considered one of the key molecules involved in osteoarthritis, causing inflammation and cartilage destruction.

The treatment is injected directly into the knee joint where it transforms the cells inside the joint capsule. Production of IL-1Ra by those cells is expected to inhibit the destruction caused by IL-1. IL-1Ra production appears to be localized to the joint.

Many hemophilia patients develop a similar condition called hemarthrosis due to bleeding into the joints. It is suspected that IL-1 is also an actor in the long-term damage in hemarthrosis, so GNSC-001 could also be effective in dealing with the pain and degradation of hemarthrosis.

In a Phase I study, nine patients were treated with increasing doses of GNSC-001. The researchers found elevated levels of IL-1Ra that persisted for at least the twelve months of the study, with no serious adverse events. Although the Phase I study only looked at safety, not efficacy (effectiveness), pain and function scores did improve. [C&ENews article, p, 13, 3/6/23]

Pfizer's Gene Therapy License Application Accepted by FDA and EMA

6/27/23 Pfizer is developing fidanacogene elaparvovec, a gene therapy for hemophilia B. It is an AAV vector containing the Padua high-activity factor IX gene. The product was originally licensed from Spark Therapeutics in 2014. Pfizer submitted both a Biologics License Application (BLA) to the U.S. FDA and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) based on 15 months of data from their Phase III clinical study. They announced that both applications have been accepted by the agencies. FDA has set a goal for a decision on the application in the second quarter of 2024. The Phase III study is ongoing, and the 45 participants will be monitored for a total of 15 years. [Pfizer press release, 6/27/23]

Takeda Trims Early-Stage Efforts in AAV Gene Therapy

4/6/23 Takeda has been involved in hemophilia gene therapy through mergers and spin-offs going back through Shire to Baxalta and Baxter. Their gene therapy plans have never been too clear, but since March 2020, they have invested in gene therapy deals with ten small biotech companies, including one with Poseida Therapeutics for hemophilia A. Most of those deals have been for projects that use other vectors than AAV.

Now, they have announced that they will no longer perform early-stage R&D on AAV gene therapies or on rare hematology projects. Early-stage R&D is work that is done in the beginning stages of development, before clinical studies. They say that the changes won't affect any projects currently in development.

Reading between the lines, this suggests that Takeda still has a strong commitment toward gene therapy, just not with AAV. Although AAV has gotten us to a licensed product for hemophilia B gene therapy, as well as numerous other gene therapies, it is not the ideal vector. It interacts strongly with the immune system, which causes problems. It also preferentially infects liver cells, which are probably not the best cell type for all applications. For instance, hepatocytes (liver cells) may not be the best place to make factor VIII for hemophilia A gene therapy.

Factor VIII is normally made in sinusoidal endothelial cells, which line the blood vessels in the liver, but are not hepatocytes. Targeting the wrong cell may be the reason that we've seen factor VIII levels decline over time in several A gene therapies. In contrast, factor IX is made in hepatocytes, its normal source. Thus, Takeda is not abandoning gene therapy but instead taking a leap into the future for non-AAV gene therapy. [Fierce Biotech article, 4/6/23]

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.

women & girls with hemophilia

articles to support, educate, and empower

Navigating Life:
A Journey of Diagnosis, Care, and Personal Growth

BY CHRISTINE BOND

Introduction: As a child, my life seemed relatively normal, with a typical family, good friends, and attending public school. However, there were a few things that made my childhood different from others. Frequent nosebleeds, unexplained injuries requiring surgery, and recurrent ankle sprains were just some of the signs that would eventually lead to a life-changing discovery. It was not until the birth of my second child, Tony, that hemophilia B entered our lives.

Little did I know that this diagnosis would uncover not only Tony's condition but also my own. This article chronicles my journey with hemophilia B, Hashimoto's thyroiditis, and the challenges I faced along the way.

A Quest for Diagnosis:
After Tony's diagnosis, I began researching hemophilia B and realized that I might also have the condition. Frustratingly, his treatment center offered no assistance in diagnosing me, so I turned to my primary care doctor for help. Testing my factor IX activity level revealed shocking results of 18, confirming my suspicion of having hemophilia B. However, the treatment center's response was underwhelming, dismissing the importance of my condition, and failing to provide the necessary support for emergencies.

Advocacy for Myself and My Health:
Undeterred, I persisted in seeking the care I needed. I pushed for an appointment with an adult hematologist at my son's treatment center and shared my concerns about joint issues, previous injuries, and problematic menstrual bleeding. Unfortunately, the hematologist's response was limited to suggesting birth control pills as a solution. Unsatisfied with this option, I continued my quest for proper care.

Finding the Right Treatment:
Through interaction with others in the hemophilia community, I learned about a hematologist nearby who treated women with hemophilia. Desperate to receive the care I required, I promptly scheduled an appointment. This hematologist recommended short-acting factor infusions during my period and injuries, which significantly improved my condition.

Later, I discovered another doctor in Western Pennsylvania who treated women with longer-acting factor prophylactically. This physician advised me to switch to a longer-acting factor once per week,

minimizing vein damage and ensuring sufficient coverage throughout the week.

Building a Healthier Body:
Empowered by my improved treatment regimen, I gradually realized the importance of prioritizing my overall health. Having experienced fewer injuries and bleeds, I became more comfortable exercising and subsequently felt motivated to lose weight. However, my journey towards weight loss was complicated by another condition I had called Hashimoto's thyroiditis. This autoimmune disorder, coupled with insulin resistance, posed additional challenges.

The Importance of Finding the Right Doctor:
Understanding the need for specialized care, I sought out the best doctor in my area to address my thyroid condition. This doctor diagnosed me with insulin resistance and prescribed Metformin, a medication commonly used for type 2 diabetes. Metformin helped control my blood sugar and kick-started my weight loss,



but my doctor informed me that a GLP-1 medication would be added to my treatment plan for further weight loss.

Overcoming Obstacles and Achieving Progress:
Despite initial concerns about potential stomach issues, I trusted the process and began taking both Metformin and the GLP-1 medication concurrently. Additionally, I engaged in physical therapy to improve joint health and build muscle, working towards my goal of losing weight. With the assistance of a continuous glucose monitor, I gradually shed pounds and built muscle, experiencing improved overall well-being.

Conclusion:
My journey with hemophilia B and Hashimoto's thyroiditis has been one of self-discovery, resilience, and personal growth. Rather than viewing these conditions as burdens, I see them as opportunities to learn and overcome challenges.

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MEDEXUS PHARMA

Mining for Gold in the "What Ifs?"

An Interview with Kristin Shelton

BY RENAE BAKER

Some people ask the best questions! Have you met Kristin and Dan Shelton? They are both engineers by trade, and the intersection where that education meets with their hemophilia B experience is helping the community. When they joined The Coalition for Hemophilia B, they jumped in with both feet!

Kristin, at Kim Phelan's urging, started serving on the B2B Pfizer Advisory Group almost immediately and helped create their B2B books. I sat down to a Zoom interview with her to find out more about her story.

The story of the Shelton's journey from Alabama to Missouri to South Carolina, and the multiple doctors with whom they tried to reason with before finding their "wonderful" current situation, reveals their no-nonsense intelligence and dogged persistence. Five doctors, four children (three with hemophilia B,) and three states later, they still have questions, and they also have some very useful solutions!

Bleeding disorders were nowhere on their radars before their first child, Danny, was born. (See Factor IX newsletter article, Danny's Dose, Winter 2022.) By the time he was diagnosed, at ten months old, with severe hemophilia B, Kristin was pregnant with their second.

Danny's case was taken seriously and he was prescribed prophy. Dan's work in mine engineering made it difficult to live near HTCs, but he began keeping a spreadsheet record of Danny's measurements.

"Every time we get new data points," Kristin explains, "we'll look at Danny's logbook and say, 'OK, they did a blood draw on this day. This is what the factor level came back at. This is how many days it's been since he had factor. We'll double check the equation and make sure the number is falling in line with it."

"Equation?" I ask. "Is it rare for families to go about it in this way?"

"What I've seen," Kristin starts, "Is that there are a lot of families who follow the doctors' wishes blindly. I also

think doctors are given more credibility on the math end of things, when many of them are not very good with data sets like this.

"When Danny was first diagnosed, we got upset, because according to MASAC guidelines you should do your infusion and then check after so many days to see what your levels are. This would help ensure your dosage is correct for your levels and metabolism, but the doctors wouldn't do that."

We said, "Okay - if Danny's going to start this regimen, then where are the data points? Are you just grabbing this arbitrary number of factor and increasing it if he has a bleed? Surely, there must be some data that we can go off of!"

The doctors in Alabama started Danny on a very low dosage. When Kristin and Dan calculated the dosage based on his factor level of 1% and his weight, they discovered it was half of what Danny should have been taking according to the product insert. Dan presented the doctors with his graphs and calculations.

"We became the family of 'Just give the Sheltons whatever they want,' because we took data to them!" Kristin laughs. "But they never answered our questions about where they were getting their numbers from. If you're giving these prescriptions out, shouldn't you have a reason you're choosing these points?"

Even their current hematologist, whom they respect, erred prescribing Danny's new long-lasting product by calculating it for one week instead of two. When the Sheltons noticed that Danny wasn't making it to the two-week mark, they pulled out the insert, did the math and figured out the mistake.

"There is a lot more content on that insert than just warnings." Kristin reflects. "Many people don't realize that the equation is on there, and the equation is different for every product." Kristin and Dan realize they have experienced in following the math, but math is not in everyone's wheelhouse. Might they consider leading a webinar? Stay tuned!

During her second pregnancy, Kristin was tested and diagnosed with hemophilia B. Despite an early life riddled with missed symptoms of a bleeding disorder (e.g., unexplained bruises, fatigue, bleeding gums), Kristin wasn't diagnosed with hemophilia B until then.

"I thought a little bit of blood was a normal part of brushing your teeth," she recalls, "and no one really ever told me what normal vs abnormal bleeding was when I started my period." Her diagnosis put everything into perspective though, and she knew she wouldn't allow the same dismissal of her daughter's symptoms.

"We knew to watch our little girl carefully," Kristin starts.

"From the get-go, Lilyan had many red flags. She would cry and pop blood vessels in her eyes. She would bruise easily and the bruises would take weeks to heal."

Kristin remembers. "We spoke to Danny's hematologist to have Lilyan tested using similar evidence of symptoms as her brother, but they refused, stating 'ethical concerns.'" Kristin and Dan protested saying, "Well, it wasn't unethical to have our son tested. You said he possibly had a bleeding disorder and went right to genetic testing."

Kristin and Dan pleaded their case, reminding the doctor how far away from the hospital they lived and how necessary a medical alert bracelet was for their baby girl. The doctor wouldn't budge.

"So, we went to a second hematologist in Alabama, and were given the same excuse: "There's no reason to test her because even if she (tests positive,) she'll only be a carrier." The Sheltons were angry.

When Lilyan was a year and a half, the Sheltons moved to Missouri. Kristin set about advocating for Lilyan to be tested at a St. Louis HTC. She was such a squeaky wheel that, Kristin says, "I think they finally got annoyed with me and said, 'Just test this kid!'"

Lilyan had the genetic testing done, and her factor level was 30%. Kristin felt vindicated, but any feeling of satisfaction immediately dissipated when the hematologist refused to write a factor prescription for her daughter. So, they headed to a second St. Louis hematologist.

"That HTC hematologist told us they wouldn't give her a prescription for an unacceptable reason." In brilliant Mama Bear mode, Kristin looked the doctor in the eye and said, "That's a crock. If that's what you want, you put that in writing." The doctor said "Okay, we'll give you a prescription."

Dan and Kristin still marvel at the fact the doctors did not want to test a girl for hemophilia B, calling it "unethical. "We got the prescription for one, tiny dose of factor for her."

After a playtime accident where Lilyan banged her forehead, resulting in a unicorn-horn-like bump, their doctor advised them to go to the hospital emergency room in St. Louis, an hour and a half from where they lived. In the ER, an MRI was ordered, but the hematologist never came down to look at her.

The ER doctor didn't seem to understand bleeding disorders and sent them home. Eight years later, they can still feel the scar tissue in her forehead from that bleeding event.

Meanwhile, Kristin and Dan welcomed two more daughters. They suspect one does not have hemophilia and one has a case very much like Kristin's.

Two years ago, the Shelton's moved to South Carolina. Kristin walked into their new HTC with data and pictures and said, "This is what we are up against and nobody else has listened."

The young, female hematologist was receptive to treating Lilyan. At ten years old, Lilyan received her first dose of factor for a leg bleed and was amazed at how much better her wrists and other parts of her body felt. "It was heartbreaking." Kristin remembers. "Lilyan is now on prophy," Kristin is thrilled to report!

One of Kristin and Dan Shelton's favorite things to do is sit on their South Carolina back patio after the kids have gone to bed and talk about the "what ifs."

"I think it's fascinating to look at the evidence of things and to ponder 'What if things were different?'" Kristin muses.

"What If the health industry treated women and girls with the same consideration they give the men and boys? In my opinion, in the hemophilia realm, women get thrown by the wayside, because we don't have the typical symptoms as men." Kristin states, "I believe the biggest mistake is that women are not being treated with factor. Heavy periods lead to fatigue, lost time at work, and chronic anemia which causes gum disease and early tooth decay. Chronic joint bleeds lead to surgeries for joint replacements much earlier. All of this leads to a diminished quality of life for women with bleeding disorders."

"What if women were included in more medical research trials?"

"What if we could get better tracking of factor levels based off of hormone fluctuation?"

Kristin is looking forward to putting her education in environmental engineering to use in the next few years. Meanwhile, she has found that her environment within the hemophilia community could use some engineering. Her call to action is that we keep pushing for research for girls and women. Ask the insurance companies and medical establishments to follow the data without bias.

What if...?

ADVOCACY NEWS

BY DR. APRIL WILLIS

WORLD HEMOPHILIA DAY

World Hemophilia Day was on April 17, 2023, and the theme this year was *"Access for All: Prevention of Bleeds as the Global Standard of Care."* Building on last year's theme, the call to action for the community in 2023 was to come together and advocate with local policymakers and governments for improved access to treatment and care with an emphasis on better control and prevention of bleeds for all people with bleeding disorders (PWBDs).

The Coalition for Hemophilia B has supported a variety of international efforts to improve life for people affected by bleeding disorders around the world. We sponsor two patients with Hemophilia in India through Save One Life and provide annual contributions to the humanitarian programs of the WFH. We also supported the production of the film Bombardier Blood which raised awareness of bleeding disorders worldwide. Additionally, we donate to the WFH Susan Skinner Scholarship Fund which provides a unique opportunity for recipients to network and develop skills that can be used to empower themselves and others to advocate for the improved care of women with bleeding disorders in their communities and worldwide.

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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.

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Email: contact@hemob.org
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BE READY FOR THE UNEXPECTED

ONCE-WEEKLY REBINYN® HELPS YOU KEEP FACTOR 9 LEVELS HIGHER FOR LONGER[a]

Expect higher factor levels and bleed protection with once-weekly Rebinyn®.[b]

~80% With once-weekly Rebinyn®, adults and adolescents with hemophilia B can spend approximately 80% of their week with Factor 9 levels in the non-hemophilia range (greater than 40%).[b]

115 hour half-life in adults treated with Rebinyn® 40 IU/kg[c]

103 hour half-life in adolescents treated with Rebinyn® 40 IU/kg[c]

Visit www.rebinyn.com to learn more about once-weekly Rebinyn®

[a]Rebinyn® achieved and maintained higher factor levels than recombinant Factor 9 based upon a phase 1 study comparing 25, 50, and 100 IU/kg doses of Rebinyn® to a 50 IU/kg dose of standard half-life recombinant Factor 9 in 7 adults and a 50 IU/kg dose of plasma-derived Factor 9 in 8 adults. For Rebinyn®, estimated average Factor 9 activity is adjusted to a dose of 50 IU/kg. Incremental recovery at 30 minutes (IR_{30}) and half-life were higher and longer with Rebinyn® than recombinant Factor 9 (IR_{30} 0.0131 vs 0.0068 (IU/mL)/(IU/kg) and half-life 93 vs 19 hours). The clinical relevance of these pharmacokinetic differences is unknown.
Incremental Recovery: The increase in plasma concentration per IU/kg of factor administered.
Half-life: The time it takes for the level of factor in the blood to fall by half (50%).

[b]Data represent mean steady-state pharmacokinetic (PK) profiles from previously treated adolescent/adult patients with moderate-to-severe hemophilia B (N=9) taking repeated doses of Rebinyn® 40 IU/kg once weekly. Factor 9 levels were within the non-hemophilia range (greater than 40%) for 5.4 days (about 80% of the week).

[c]Based on analysis using a 1-stage assay in patients (N=6) aged 18 and older, the half-life at steady state was 115 hours following once-weekly (40 IU/kg) dosing; in patients (N=3) aged 13 to 17, the half-life at steady state was 103 hours. Following single-dose administration (40 IU/kg) in the same patient population, the half-life was 83 hours (adults) and 89 hours (adolescents).

Indications and Usage

What is Rebinyn® Coagulation Factor IX (Recombinant), GlycoPEGylated?

Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat, prevent, or reduce the frequency (number) of bleeding episodes in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for immune tolerance therapy.

Important Safety Information

What is the most important information I need to know about Rebinyn®?

- **Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.** Carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing Rebinyn®.

Who should not use Rebinyn®?

Do not use Rebinyn® if you:
- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my healthcare provider before using Rebinyn®?

Tell your healthcare provider if you:
- have or have had any medical conditions.
- take any medicines, including non-prescription medicines and dietary supplements.
- are nursing, pregnant, or plan to become pregnant.
- have been told you have inhibitors to Factor IX.

How should I use Rebinyn®?

- Rebinyn® is given as an infusion into the vein.
- **Call your healthcare provider right away if your bleeding does not stop after taking Rebinyn®.**
- Do not stop using Rebinyn® without consulting your healthcare provider.

What are the possible side effects of Rebinyn®?

- **Common side effects include** infusion site reaction (bruising, bleeding, swelling, pain, or redness), itching, and rash.
- Your body can also make antibodies called "inhibitors" against Factor IX, including Rebinyn®, which may stop Rebinyn® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.
- **Call your healthcare provider right away or get emergency treatment right away if you get, for example, any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- You may be at an increased risk of forming blood clots in your body, especially if you have risk factors for developing blood clots. Call your healthcare provider if you have chest pain, difficulty breathing, leg tenderness, or swelling.
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) in certain cells in the brain. The potential human implications of these animal tests are unknown.

Please see Brief Summary of Prescribing Information on the following page.
Rebinyn® is a prescription medication.
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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Brief Summary Information about:
REBINYN® Coagulation Factor IX (Recombinant), GlycoPEGylated

Rx Only

This information is not comprehensive.

- Talk to your healthcare provider or pharmacist
- Visit www.novo-pi.com/REBINYN.pdf to obtain FDA-approved product labeling
- Call 1-844-REB-INYN

Read the Patient Product Information and the Instructions For Use that come with REBINYN® before you start taking this medicine and each time you get a refill, as there may be new information.

This Patient Product Information does not take the place of talking with your healthcare provider about your medical condition or treatment. If you have questions about REBINYN® after reading this information, ask your healthcare provider.

What is the most important information I need to know about REBINYN®?

Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.
You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing REBINYN® so that your treatment will work best for you.

What is REBINYN®?

REBINYN® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Hemophilia B is an inherited bleeding disorder in all age groups that prevents blood from clotting normally.

REBINYN® is used to treat, prevent, or reduce the frequency (number) of bleeding episodes in people with hemophilia B.

Your healthcare provider may give you REBINYN® when you have surgery.

Who should not use REBINYN®?

You should not use REBINYN® if you

- are allergic to Factor IX or any of the other ingredients of REBINYN®
- if you are allergic to hamster proteins

If you are not sure, talk to your healthcare provider before using this medicine.

Tell your healthcare provider if you are pregnant or nursing because REBINYN® might not be right for you.

What should I tell my healthcare provider before I use REBINYN®?

You should tell your healthcare provider if you

- Have or have had any medical conditions.
- Take any medicines, including non-prescription medicines and dietary supplements.
- Are nursing. It is not known if REBINYN® passes into breast milk or if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if REBINYN® may harm your unborn baby.
- Have been told that you have inhibitors to Factor IX (because REBINYN® may not work for you).

How should I use REBINYN®?

Treatment with REBINYN® should be started by a healthcare provider who is experienced in the care of patients with hemophilia B.

REBINYN® is given as an infusion into the vein.

You may infuse REBINYN® at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your hemophilia treatment center or healthcare provider. Many people with hemophilia B learn to infuse the medicine by themselves or with the help of a family member.

Your healthcare provider will tell you how much REBINYN® to use based on your weight, the severity of your hemophilia B, and where you are bleeding. Your dose will be calculated in international units, IU.

Call your healthcare provider right away if your bleeding does not stop after taking REBINYN®.

If your bleeding is not adequately controlled, it could be due to the development of Factor IX inhibitors. This should be checked by your healthcare provider. You might need a higher dose of REBINYN® or even a different product to control bleeding. Do not increase the total dose of REBINYN® to control your bleeding without consulting your healthcare provider.

Use in children

REBINYN® can be used in children. Your healthcare provider will decide the dose of REBINYN® you will receive.

If you forget to use REBINYN®

If you forget a dose, infuse the missed dose when you discover the mistake. Do not infuse a double dose to make up for a forgotten dose. Proceed with the next infusions as scheduled and speak to your healthcare provider if you have any questions or concerns.

If you stop using REBINYN®

Do not stop using REBINYN® without consulting your healthcare provider.

If you have any further questions on the use of this product, ask your healthcare provider.

What if I take too much REBINYN®?

Always take REBINYN® exactly as your healthcare provider has told you. You should check with your healthcare provider if you are not sure. If you infuse more REBINYN® than recommended, tell your healthcare provider as soon as possible.

What are the possible side effects of REBINYN®?

Common Side Effects Include:

- infusion site reaction (bruising, bleeding, swelling, pain, or redness)
- itching
- rash

Your body can also make antibodies called "inhibitors" against Factor IX, including REBINYN®, which may stop REBINYN® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

You could have an allergic reaction to coagulation Factor IX products. **Call your healthcare provider right away or get emergency treatment right away if you get, for example, any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.

You may be at an increased risk of forming blood clots in your body, especially if you have risk factors for developing blood clots. Call your healthcare provider if you have chest pain, difficulty breathing, leg tenderness or swelling.

These are not all of the possible side effects from REBINYN®. Ask your healthcare provider for more information. You are encouraged to report side effects to FDA at 1-800-FDA-1088.

Tell your healthcare provider about any side effect that bothers you or that does not go away.

What are the REBINYN® dosage strengths?

REBINYN® comes in four different dosage strengths. The actual number of international units (IU) of Factor IX in the vial will be imprinted on the label and on the box. The four different strengths are as follows:

Cap Color Indicator	Nominal Strength
Red	500 IU per vial
Green	1000 IU per vial
Yellow	2000 IU per vial
Dark Gray	3000 IU per vial

Always check the actual dosage strength printed on the label to make sure you are using the strength prescribed by your healthcare provider.

How should I store REBINYN®?

Prior to Reconstitution (mixing the dry powder in the vial with the diluent):

Store in original package in order to protect from light. Do not freeze REBINYN®.

REBINYN® vials can be stored in the refrigerator (36-46°F [2°C-8°C]) for up to 24 months until the expiration date, or at room temperature (up to 86°F [30°C]) for a single period not more than 6 months.

If you choose to store REBINYN® at room temperature:

- Note the date that the product is removed from refrigeration on the box.
- The total time of storage at room temperature should not be more than 6 months. Do not return the product to the refrigerator.
- Do not use after 6 months from this date or the expiration date listed on the vial, whichever is earlier.

Do not use this medicine after the expiration date which is on the outer carton and the vial. The expiration date refers to the last day of that month.

After Reconstitution:

The reconstituted (the final product once the powder is mixed with the diluent) REBINYN® should appear clear without visible particles.

The reconstituted REBINYN® should be used immediately.

If you cannot use the reconstituted REBINYN® immediately, it should be used within 4 hours when stored at or below 86°F (30°C). Store the reconstituted product in the vial.

Keep this medicine out of the sight and out of reach of children.

What else should I know about REBINYN® and hemophilia B?

Medicines are sometimes prescribed for purposes other than those listed here. Do not use REBINYN® for a condition for which it is not prescribed. Do not share REBINYN® with other people, even if they have the same symptoms that you have.

For more information about REBINYN®, please call Novo Nordisk at 1-844-REB-INYN.

Revised: 08/2022

REBINYN® is a trademark of Novo Nordisk A/S.

For Patent Information, refer to: http://novonordisk-us.com/products/product-patents.html

Manufactured by:
Novo Nordisk A/S
Novo Allé, DK-2880 Bagsværd, Denmark

More detailed information is available upon request.

Available by prescription only.

For information about REBINYN® contact:
Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA

US22REB00153 August 2022

novo nordisk®

Patrick Collins

We are delighted by the news that Patrick Collins, a long-time friend and colleague of the Coalition for Hemophilia B and the entire hemophilia community, has been named the Vice President for Corporate Relations at the National Organization for Rare Diseases (NORD). We are sharing this recent article by Patrick about Medicaid reimbursement for gene therapy as an example of Patrick's deep understanding and insight into this and many other important subjects.

WILL THE MEDICAID PROGRAM CATCH UP WITH INNOVATION?

BY PATRICK COLLINS

The last few decades have seen incredible progress in treatment for people with hemophilia. People have many more options today as we have seen the evolution from the original plasma-derived products to recombinant therapies (which were the innovators of the 1990s and 2000s) and are now available as shorter half life and extended half life, and now the advent of monoclonal antibody therapies. These products have become the standard of care for the treatment of both Factor VIII deficiency (hemophilia A) and Factor IX deficiency (hemophilia B). With increased options for inhibitor patients as well, we are in the middle of a treatment renaissance for bleeding disorders.

Now the next innovative advancement in treating people with hemophilia has arrived. Gene therapy, after decades of promise has finally become a reality. The first gene therapy for the treatment of hemophilia B has been approved by the Food and Drug Administration (FDA) in late 2022. A second gene therapy product for hemophilia B is currently in late-stage clinical development and will hopefully prove effective. Specific to hemophilia A, the FDA this year has also approved the first gene therapy. The potential for this evolution in treatment is significant, but this also comes with significant policy questions, most notably surrounding the price and reimbursement of these new gene therapies.

The initial approved gene therapy for hemophilia B comes with a price tag of $3.5 million, which at present would make this product the highest priced FDA approved pharmaceutical ever. However, unlike blood clotting factors, where traditional use requires an infusion every few days, weekly or every two weeks (depending on each patient and therapy being used) via a prophylaxis regimen, the promise of gene therapy is that it would be a one-time infusion, allowing the individual to produce their own blood clotting factor for a period of years (studies illustrate efficacy exceeding twenty years). Therefore, looking at costs over years, gene therapies, despite their high upfront costs, should provide long-term cost savings for the healthcare system.

This brings us to the policy question at hand – will state Medicaid programs catch up with innovation and cover the costs of these therapies?

State Medicaid programs are not presently designed to absorb therapies with high upfront costs even with the promise of little to no costs in future years. Medicaid programs are designed to examine cost on a yearly basis – in fact, that is how most states fund their Medicaid programs, annually, through the passage of a yearly budget (a small minority of states do use two-year budget cycles) - and not consider the long-term cost savings for the program. Such a situation is ironic in that state Medicaid programs may see the potential for long term savings but may struggle with making the decision to cover gene therapy because of that first-year cost exceeding their budget parameters.

There is a bright side to this situation in that hemophilia B is such a small population and the target audience for gene therapy with Medicaid as their insurer is even smaller. Therefore, state Medicaid programs will likely have very few covered individuals using this therapy (likely in the single digits for many states or low double digits for larger states). Gene therapy for hemophilia B will not be a budget buster for state Medicaid programs.

On the public policy front, those in the hemophilia B community can join with other communities seeing the promise of gene therapy and advocate for policies that will increase the potential for coverage of such therapies. These include:

- **Empowering state Medicaid programs with more flexibility in how to pay for expensive gene therapy programs.**

  Instead of the typical fee for service type of reimbursement, providing Medicaid with the flexibility to pay for gene therapies through value-based arrangements could allow for greater coverage of these new therapies moving forward. Such value-based arrangements can include a pay for performance type model, payment over time through amortized payments, or any other number of innovative models that can blunt the high upfront costs. Through federal rulemaking, Medicaid's do have increased ability for such negotiation with pharmaceutical companies, but there is federal legislation proposed that would codify in law such Medicaid flexibility.

  Congressman Brett Guthrie (R-KY) who is the Chair of the House Energy and Commerce Committee Subcommittee on Health, led the reintroduction of the Medicaid VBPs for Patients (MVP) Act with Reps. Anna G. Eshoo (D-CA), John Joyce, M.D. (R-PA)), Jake Auchincloss (D-MA), and Mariannette Miller-Meeks (R-IA).The MVP Act would provide vulnerable populations with rare diseases access to innovative treatments and cures by enabling states to voluntarily enter into value-based purchasing (VBP) agreements, which tie the cost of treatments to patient outcomes

- **Allowing Medicaid coverage to cross state lines**

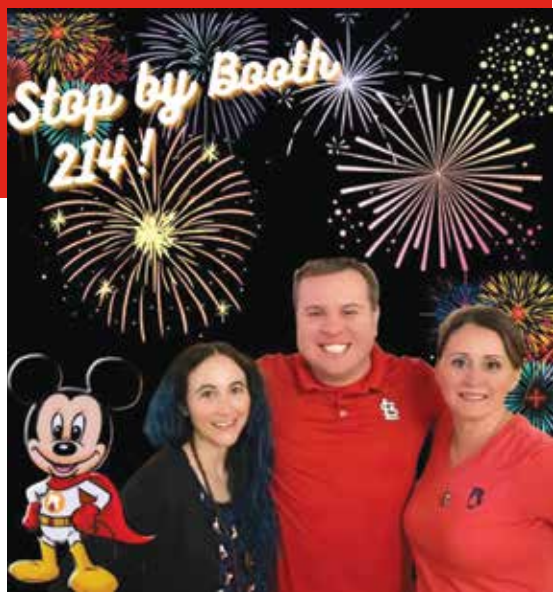
  Another potential policy measure that can impact access to gene therapy is allowing Medicaid coverage to cross state lines. Because each state has its own Medicaid eligibility requirements, an individual cannot transfer coverage from one state to another, nor can an individual use their coverage when temporarily visiting another state, unless the individual needs emergency health care. Hemophilia treatment centers will likely be the site for gene therapy treatment, but they are not in every state. This may create difficulty for those on Medicaid living in one state from seeking gene therapy treatment in another state.

  There have been legislative efforts in the past to address this issue (Senator Charles Grassley (R-IA) has long been such an advocate) and the bleeding disorders community can partner up with others to continue to pursue such efforts.

Overall, the future is bright for those with hemophilia B and having the availability of additional treatment options can only further brighten the prospects. The leaps in innovation for treating hemophilia over the last 30 years have been incredible, but we need to ensure that the healthcare payment models keep up with such innovation.

HFA SYMPOSIUM

BY ROCKY WILLIAMS



HFA Symposium was held April 13–15, 2023 in Orlando! Erica, Farrah, and I had an incredible time representing CHB, connecting with fellow attendees, participating in insightful educational sessions, and celebrating the outstanding achievements of some remarkable individuals in our community. One of the highlights of the event was reconnecting with familiar faces. Seeing so many passionate individuals dedicated to the bleeding disorders community was truly heartwarming.

We would like to extend our heartfelt congratulations to Bill and Debbie for their well-deserved awards. Debbie de la Riva, LPC received the prestigious *Terry Lamb Health and Wellness Award* for her outstanding work in mental health education. Her unwavering commitment to promoting health and wellness in the bleeding disorders community is truly remarkable. We applaud Debbie for her dedication and the positive impact she continues to make in our community.

Another distinguished honor was bestowed upon Bill Patsakos, who received the *Charles Stanley Lifetime Achievement Award*. This award recognizes his extraordinary lifetime service, encompassing national volunteerism, professionalism, and leadership. Bill's contributions have been instrumental in shaping the bleeding disorders community, and we are grateful for his tireless efforts and dedication.

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TRAINING WHEELS

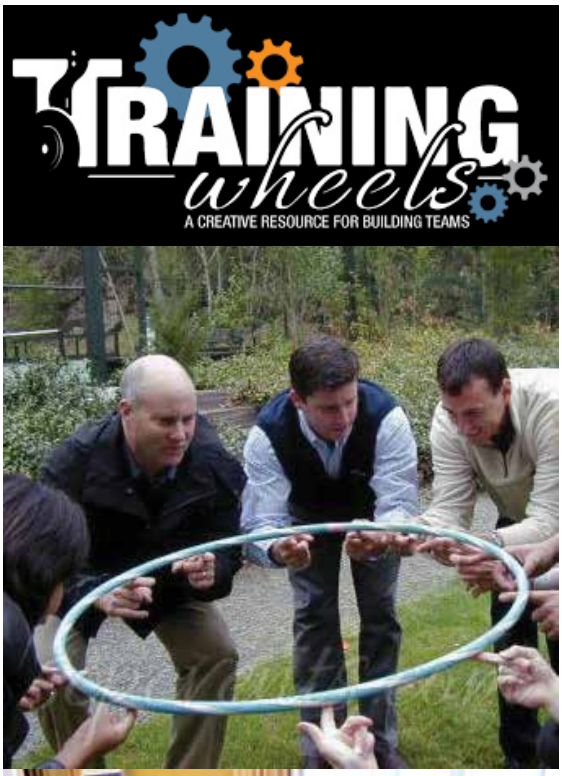
BY ROCKY WILLIAMS

During March 28 to 30, Erica Garber and I had the awesome experience of attending a Training Wheels workshop held in Littleton, Colorado. Training Wheels is the ultimate resource for leadership workshops, icebreakers, group games, and team building activities. It has an extensive collection of resources that ignited in us a spark of excitement for engagement and collaboration!

For three days, we immersed ourselves in an experiential learning experience that left an indelible mark on our approach towards education and team building. Gone are the days of passive learning through lectures and textbooks—and CHB wants to make sure we're on the cutting edge of experiential learning, with hands-on activities that aid learning and retention. We're so excited to share the transformative lessons we learned with the hemophilia B community!

During our time at the workshop, we were introduced to a wide range of captivating activities and games that really show the essence of experiential learning. Some of our favorite activities included fun ways to share about each other. We learned interactive ways to do pair sharing, where we exchanged questions and answers in a group, but with random new participants every couple of minutes. This kept the meeting and greeting going while being able to quickly change up the questions. We did this in a game that I like to call People to People.

We also used some fun games like Spot It, Dude, Finger Fencing, Opera Man, 7 Up, and Screaming Toes. These are sure to have people laughing, working together, and engaged!



The workshop not only expanded our repertoire of interactive resources, but also provided us with the tools to implement experiential learning techniques in our own programs. We left Colorado feeling invigorated and inspired, armed with new tools to shape the future of our events. Can't wait to try out the new handshakes that we learned. Have you ever done the salmon handshake! How about the Australian handshake? These are just too funny to pass up.

My favorite team activity was balancing tennis balls on strings as a team. It really takes everyone working well together to achieve the game's goal. This is very much true as a community. The better we work together, the more we can achieve.

We are eagerly looking forward to taking what we have learned at Training Wheels and infusing it into our programs. The power of experiential learning will enable us to create dynamic, engaging, and impactful experiences for our participants.

Want to see it in action? You are just going to have to come to our upcoming in-person events! We plan on mixing things up this year and making things even more interactive, fun, and interesting! Stay tuned!

NORD'S 40TH RARE IMPACT AWARD GALA

BY KIMBERLY PHELAN

Oh, what a night! Wayne and I were truly honored to present on behalf of NORD, the 2023 Industry Innovation Award to Honorees, CSL Behring and uniQure for HEMGENIX, the first approved Gene Therapy for people with hemophilia B at the NORD (National Organization for Rare Disorders) Gala. Accepting on their behalf were Robert Lojewski, Senior Vice President & General Manager, North America at CSL Behring and Matt Kapusta, Chief Executive Officer at uniQure.

This was also NORD's 40th Anniversary Celebration! We were delighted to see Abbey Meyers (the "Mother of Movement") receive a *Lifetime Achievement Award*. A movement, that in 1983, became the first national nonprofit organization (NORD) for all rare disorders!

Shout out to Bernie Williams (Yankee fans) for his *Rare Impact Award*, helping families with idiopathic pulmonary fibrosis (IPF), which Bernie's father was diagnosed with and passed away, and other forms of interstitial lung disease (ILD).

Opening remarks were made by Peter Saltonstall, President and CEO of NORD, followed by a welcome by emcee for the evening, Peter Alexander, an Emmy award-winning journalist, co-anchor of Saturday Today, and Chief White House Correspondent for NBC news and many other platforms. Special musical performances by CeeCee and Christian Guardino.

There were so many wonderful honorees and heartfelt stories this evening. We also had the distinguished pleasure of having Mark Skinner at our table. It was so wonderful to see him and catch up! We applaud all for their good work and perseverance! This was truly a wonderfully memorable evening.

SPRING INTO WELLNESS:

A HEARTWARMING SERIES FILLED WITH LAUGHTER, HEALING, AND DELICIOUS MOMENTS

BY ERICA GARBER

In our recent two-part event series, we brought together a community seeking healing and connection, and let me tell you, it was an absolute blast!

On May 20th, we had the pleasure of hosting the one and only Dr. Robert Lawrence Friedman, a true rock star in the world of psychology and comedy. This guy knows how to manage stress like a pro, and he did it with a twist of humor that had us rolling on the floor laughing. From practicing mindfulness with a comedic spin, to finding the silver lining in tough situations, Dr. Friedman gave us practical strategies that left us feeling like stress-busting superheroes.

Fast forward to June 3rd, when we had the honor of having Wayne Cook, a culinary aficionado and a mentor in the hemophilia B community, lead us for an unforgettable session from his kitchen. Wayne put on his chef's apron and took us on a mouthwatering journey inspired by his own garden. He showed us how to create two salads that were not only nutritious but also bursting with flavor. Let me tell you, our taste buds were doing a happy dance! And the best part? Wayne shared not just one, but four salad dressing recipes that turned our salad game from ordinary to extraordinary. It was a salad adventure like no other.

But wait, there's more! We couldn't have a wellness series without a little bit of Zen, right? So, we brought in Rick Starks, our very own tai chi guru to guide us through a restorative practice that left us feeling as calm as a Zen master. Rick's story of overcoming personal challenges and finding solace in martial arts truly touched our hearts. With his gentle guidance, we tapped into mindfulness and body connectivity, finding peace and balance within ourselves.

Laughter filled the virtual spaces, questions flew left and right, and hearts were filled with inspiration. Dr. Friedman, Wayne Cook, and Rick Starks shared their personal stories, reminding us that no matter what challenges we face, we can always find strength, growth, and a little bit of laughter along the way.

This heartwarming series was a beautiful reminder that even in the face of difficulties, our community can come together, support each other, and create moments of joy and healing. We left the series feeling empowered, armed with stress-busting strategies, delicious salad recipes, and a sense of connection that will stay with us for a long time.

So, here's to laughter, growth, and the power of coming together. Cheers to heartwarming moments that remind us that we're all in this journey of wellness and connection together.

Here's what some of our attendees had to say about it:

"I need more laughter in my day since I started working from home. I don't have coworkers that are present, so no one except patients to talk to... and they don't laugh much! I miss the personal contact and being able to laugh during my workday. This session let me get some laughter out of my system!"

"I thought this event was very helpful and creative with expression and laughter being together with my B family."

"I always enjoy attending your events online, but Mr. Freidman's program exercise was different from what I was expecting in such a good way! Involving laughter and intentional silliness was really enjoyable. Thanks for all you do!"

"I enjoyed connecting with the hemophilia B family at the Virtual Wellness event. We have already used some of the Tai Chi techniques to prepare to infuse. The salads and vinaigrettes are on the list for go to meals. Thanks for hosting these events!"

In Memoriam

MARK ANTELL

It's with a heavy heart that we share the news that Mark Antell, longtime community advocate and friend, passed away on Wednesday, March 29th. Mark volunteered in so many capacities including the Committee of Ten Thousand (COTT) and tirelessly advocated for patient access to treatment. Mark will be greatly missed.

"Words cannot describe the loss this means to me and the bleeding disorders world. He was committed to ensuring future generations of persons with bleeding disorders didn't have the worries of the past generations." – *Christopher M. Templin*

"It is with great sadness that we lost a wonderful community member, advocate and great friend. Mark's advocacy for the hemophilia community will never go unnoticed. I will never forget our great conversations and friendship that we shared. I also will never forget his wisdom and love for this community. Mark, you were a true leader and friend to all. I will miss you, my friend." – *Wayne Cook*

"A life well lived. Peace out my friend… (Mark always ended our calls with that line.)" – *Ray Dattoli*

"Mark was a great advocate, Blood Brother and friend! He will be dearly missed." – Carl Weixler



MIGUEL BROWN

It's with a heavy heart that we honor the passing of Miguel Brown. Our thoughts and prayers are with the Brown/Dominguez family.

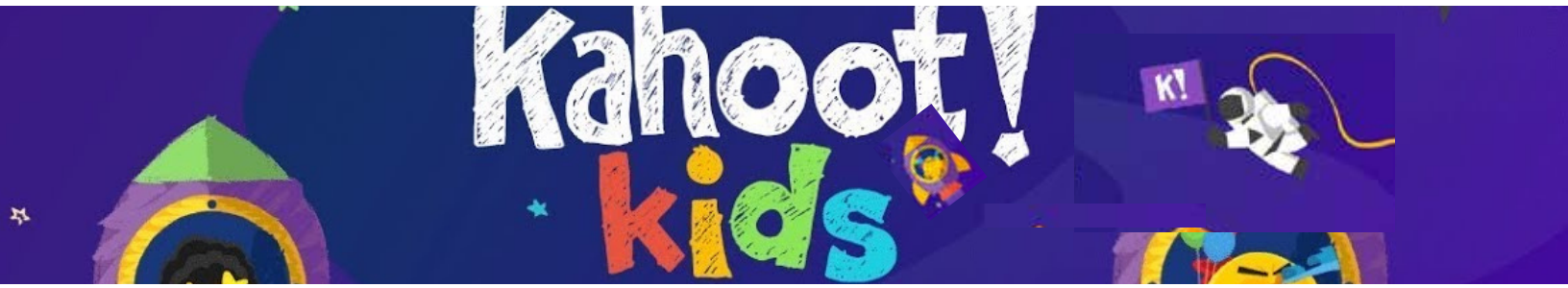
From a young age, Miguel exhibited a vibrant and charismatic personality. He was outgoing, charming, determined, and intelligent. He lived life with enthusiasm, and for those that knew him well, you could not help but be impressed by his easy going and loving nature. Miguel's smile, personality, and presence will be greatly missed.

"I've known Miguel since he was a young child. I still chuckle remembering the texts we exchanged when he attended the Partners' Retreat. He was having such a good time. I'm going to hold the sweet memories of you deep in my heart. " – Kim Phelan

"Mike was my camper, junior counselor, counselor, and friend. He was also one of the first to show me how truly important community is. It meant the world to him to be involved at camp and in the hemophilia community. He showed me how being involved means making a positive impact in the lives of others. He carried himself in the same way throughout life - always being kind and wanting to help. Mike, thank you for being such a good person and friend. You are missed." – Rocky Williams



FOR TWEENS WITH HEMOPHILIA B AND THEIR SIBLINGS AGES 6-12



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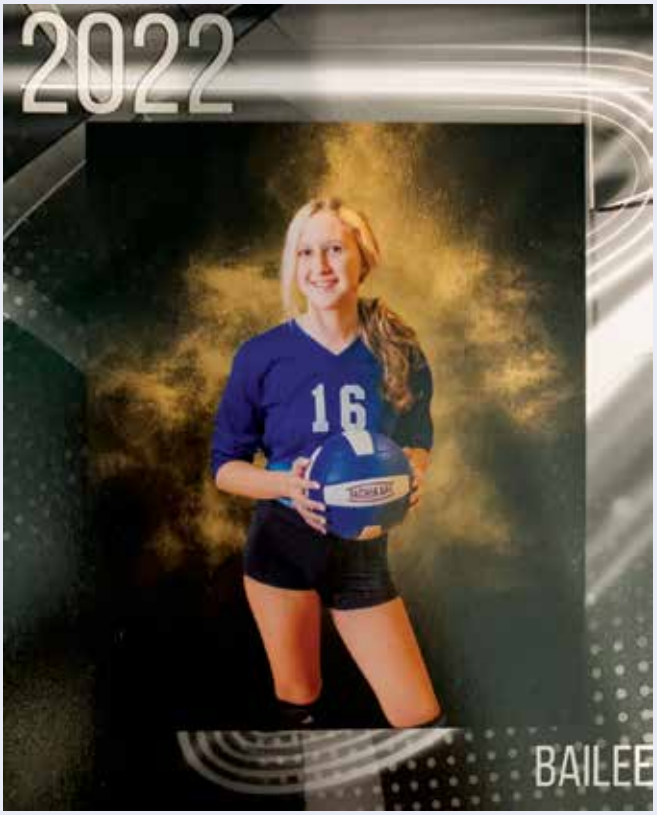
BAILEE: GET INFORMED, BE CAUTIOUS, AND JUST LIVE



BY SHELLY FISHER

Bailee is one of the coolest freshmen around and she has some chill advice for hemophilia B carriers! "Get informed, be cautious and "just live."

Bailee is one of the coolest soon-to-be freshmen I know and not just because she was two days into her summer job shaving ice at a snow cone stand when we visited. With a disposition as sweet as the syrup she pours, she took some time out during her busy work schedule to talk about her jam-packed summer, thoughts on high school, the importance of family and friends, her love of art and volleyball, and living as a carrier of hemophilia B.

With a ready smile and shy laugh, Bailey confided the true purpose of her summer work schedule early on in our conversation. In addition to having fun and making a little spending money, her hard-earned wages from working at the snow cone stand were earmarked for something special, and some might even say a little unusual. It seemed that Bailee had her heart set on a five-foot, animatronic clown! Where might one find such a decoration? "It's actually at the Spirit of Halloween." When asked where something like that might be kept, she said quickly, "I think it's gonna go in my room." When I laughed and mentioned that some people are actually afraid of clowns, her smile brightened, and she giggled. "I think they're unique and funny in a way."

Aside from her busy days of dishing out flavored ice, Bailee had a few other things on her mind and it's no wonder. With an impending, full course load including pre-advanced placement courses in algebra, English, biology, history, and art, playing on the freshman volleyball team, and continuing to work at the snow cone stand planned for the fall, she was already thinking ahead and wondering what it will be like as a freshman. Although her friends from the surrounding neighborhood will all go to the same high school, Bailee wasn't sure she would see them in the cafeteria. "Lunch is randomized, so you don't know who you have lunch with each year." Unfortunately, she's got a while to wait, and she won't find out until orientation, so, like every other freshman, she will be anxiously waiting for lunch assignments all summer.

Volleyball has been a part of Bailee's life for the past four years. "I think it's pretty fun. I hope to play on the high school team, but if it interferes with the snow cone place, I will run track." Interested in both short and long-distance events, Bailee seemed determined to incorporate some type of sport in her schedule in addition to her studies as long as it didn't interfere with her projected paycheck and eventual purchase.

When asked what others might say she does well, she responded immediately. "My art. I've been drawing for a very long time." It was no surprise when she clarified further. "I like to draw pictures of clowns. We put them on the refrigerator." When I asked if she had considered a career in art, she said, "I don't know. I think I need to work more on it to be really good." It would seem that a humble nature, in addition to her artistic abilities, may be one of Bailee's many talents.

For the first time during our visit, Bailee's smile faded somewhat when I asked about her diagnosis. She shared that she tested for Factor IX deficiency a long time ago, but Bailee has certainly never been a stranger to life with hemophilia. "My grandpa and all my male cousins have hemophilia B." At the age of ten, she

actually started helping her grandfather by giving him his shot on multiple occasions. When asked if she had any advice for anyone who was just diagnosed, she said, "I would tell them to just live, don't really let it bother you. You're gonna have to live with it somehow. I would also tell them to be cautious about what they are doing, like my grandpa tells me." Bailee also had some advice regarding understanding hemophilia for those who have been recently diagnosed. "I feel like it's important to know how it works." Bailee's words of wisdom were sound advice from someone with experience and firsthand knowledge of hemophilia B.

When given the opportunity to add anything she would like to her article, the freshman smiled broadly. "I'd like to mention one person, my closest friend Zea, because she's always told me that there is nothing wrong with having hemophilia and she's always just been there for me anytime I need help." Her best friend for 2 and ½ years, Zea is on Bailee's volleyball team, and she was the first person she told about her diagnosis. "We help each other with things going on and tell each other it's okay."

Lima the cat made an appearance at the end of our visit and the long-haired tabby was more than happy to hang with Bailee. Having spent some time with her, I completely understood why. Whether it's a randomized lunch schedule, full academic load, family member in need, or a hemophilia B diagnosis, Bailee's got this and what's more important, she knows it.

IT WAS A KAHOOT!

BY ROCKY WILLIAMS

On March 2, 2023, an exciting virtual event took place, specially made for our community's tweens. We gathered online and dived in the interactive game of Kahoot, which enthralled participants with its captivating and often funny kids' trivia. From testing their knowledge about beloved cartoon characters to embarking on a Disney Challenge, exploring the Marvel universe, and discovering fascinating facts with "Weird But True - Animal Trivia," the tweens had a blast throughout the event. Engaging, educational, and full of laughter, the virtual gathering provided an excellent platform for tweens to get together, see each other, and showcase their knowledge. Stay tuned for more exciting events designed to inspire and entertain our dynamic tween community!

MUSIC TO MY EARS

BY ROCKY WILLIAMS

On February 23rd, we held a virtual event designed for teenagers in the hemophilia B community about a topic that often brings people together: MUSIC! It was an opportunity for teens to connect with each other, have fun, and learn from one another. Bailee, an exceptional teen in our community, played a pivotal role in planning, promoting and hosting this remarkable event. She did a fantastic job of building a captivating event the teens loved!

The evening began with warm welcomes from Bailee and our event sponsor, CSL, after which Bailee led a jazzy icebreaker session where participants shared their favorite music and songs. The responses were eclectic and awesome! Some popular responses included "Heart of Glass" by Blondie, "Take Me Home, Country Roads" by John Denver, "The Way You Are" by Bruno Mars, and "Heart Like a Truck" by Lainey Wilson. It was such a cool way to start the night out by talking about and listening to music shared by the teens.

After playing music, therapist Matt Barkdull joined us and led a rap session that sparked lively and engaging conversations around our dream travel destinations. We talked about where we would like to take in our music, and the responses were literally all over the map! They included Charleston, SC; Holden Beach, NC; Alaska; Greece; Austin; Italy; London; and even Tuvalu. (I had to look this one up, so for those of you wondering, it is in the South Pacific, and is an independent island nation within the British Commonwealth). Wow, so cool! We had a ton of fun talking about all the places we would go!

Following the rap session, the group took part in a trivia game that educated them about hemophilia and advocacy. Just to name a few items, we talked about the history of hemophilia relating to the royal family, we learned that inhibitors are antibodies, and we discussed the many ways teens can and do advocate for what they need.

Then, we played our signature game of the night called "Earwax" from Jackbox Games, where participants created funny or fitting responses by combining sound effects using given prompts. Imagine combining sounds like a slide whistle into the sounds of a very large explosion or imagine hearing monster growl coupled with off-key bagpipes. The combinations were delightfully random and absurdly amusing! The game definitely hit all the right notes, leaving everyone in sync and thoroughly entertained!

The teens seemed to groove with the whole evening. "Creating an event with CHB was scary at first, but it turned out to be an amazing experience after meeting everyone." Bailee said. "The thing that surprised me the most was how much you can learn in a short amount of time. I would definitely recommend this with teens who also have Hemophilia B. It is a very fun program. Running a game with teens was like a big group of friends just playing a game, you laugh and have a great time."

We are immensely grateful to CSL Behring for their generous sponsorship, which made this memorable teen event possible.

CSL Behring

NATHAN: HEAVY METAL DOING HEAVY LIFTING

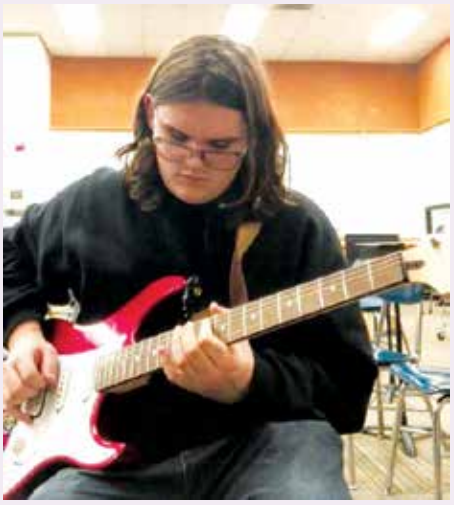
BY ROCKY WILLIAMS

How does music make your life better? Meet Nathan, a 14-year-old from Tennessee, who finds comfort and stability in playing and listening to guitar-pulsing heavy metal music. Nathan came with his family to see our Beats music grand performance and from that is when he picked up the guitar and is a natural! He's been a devoted attendee of CHB's Beats program since learning more about hemophilia, music, and the opportunities available through CHB.

With a mind that races a million miles an hour, Nathan finds that playing music is his refuge, and he sees music as a way to slow down and find clarity. "There's a lot going on in my head during the day, and playing music helps me slow it down and clear my mind" he explains. Nathan attended the Beats Music program, where he discovered his passion for music. This experience was truly transformative. Music became a therapeutic outlet, allowing him to escape from the anxiety of everyday life and redirect his focus towards something he loves.

Now, as an aspiring musician, Nathan's dedication to his craft shines through. He plays the guitar and sings, albeit modestly claiming, "I'm not great." Yet, his commitment is unwavering, practicing for a few hours every day. Among his favorite songs to play and sing are iconic tracks like "Ride the Lightning" by Metallica, "Snuff" by Slipknot, "Holy Wars" by Megadeth, and "Ace of Spades" by Motörhead.

When asked about his practice space, Nathan reveals, "We play in the bass player's garage." It's there that they come together, pouring their hearts into the music that unites them. While heavy metal remains his primary genre of choice, Nathan also dabbles in blues and even a little country, showcasing his versatility and musical ability.



As we witness Nathan's growth and determination, we are inspired by his dedication to music. We stand alongside him, supporting his dreams of playing shows in the coming years, building a career in music, and ultimately making a living doing what he loves most. Nathan embodies the essence of finding strength, joy, and purpose, reminding us all of the transformative power of music and the resilience of the human spirit.

Nathan's story shows how music can have a significant impact on someone's life. For Nathan, it has been a source of comfort, stability, and joy, helping him with life's challenges. We wish Nathan all the best in his musical journey and hope that he achieves his dreams. Be sure to check out this year's Beats video to see Nathan in full action! What talent!

B inspired!

Stories and artwork from teens in the Hemophilia B Community

SPRING 2023

IN THIS ISSUE:

BAILEE: GET INFORMED, BE CAUTIOUS, AND JUST LIVE

NATHAN: HEAVY METAL DOING HEAVY LIFTING

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!