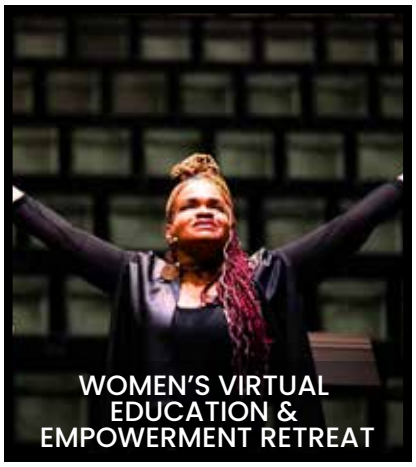
THE COALITION FOR HEMOPHILIA B

WINTER 2023

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

MEET CHRISTIAN



WOMEN'S VIRTUAL EDUCATION & EMPOWERMENT RETREAT

MEN'S EDUCATION & EMPOWERMENT RETREAT

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GEN IX

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THE COALITION FOR HEMOPHILIA

MISSION

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

SEW CONNECTED!

BY RENAE BAKER AND SHELLY FISHER

Some people turn to music, meditation or other ways to manage their pain. Christian Harris creates garments. "Going through the process of making garments allows me to focus. It's a form of meditation for me." Christian's living room has no couch, but it does have six sewing machines! This might seem odd to some people, but it's a natural fit for Harris.

"One of the coolest people in my family, who is gifted, charismatic, and charming, is my Uncle," says Harris. Growing up, Christian admired his Uncle John's creativity and ability to draw. "He would draw (clothing) designs, have them made, and then have fashion shows. It was always a big event." Christian smiles as he remembers watching the VHS tapes of his mother and other relatives modeling Uncle John's designs.

Today, Christian Harris is a fashion-industry pro, who has worked with cutting-edge names and brands, internationally, and whose work has been featured by WWD, Elle and Esquire. But Christian's aim is not to shine a light on himself and his work as much as to help others realize their creative goals, reconnecting them to their love for this glamorous, often disappointing industry.

Christian grew up in the Baltimore, Maryland area. His most-keen interests were history, geometry and art. For someone with hemophilia B, those interests served him well. His diagnosis came after a routine procedure when he was born. There had been no family history of the bleeding disorder. As a child, he frequently missed school and spent

a lot of time in the ER at John Hopkins Hospital in Baltimore. Maryland didn't have its own camp for kids with bleeding disorders, but Harris credits Emma Miller, the Executive Director of the Hemophilia Foundation of Maryland, who flew Christian and several other kids to Camp Freedom in Tennessee. It was at Camp Freedom that Christian began learning about leadership and where he would take on the responsibility of running an art program for years.

An active member of The Coalition for Hemophilia B, Christian has participated in every leadership module of Generation IX and has served as a role model and mentor at CHB's Symposium. COO, Kim Phelan, considers Harris a Coalition treasure. "Christian exudes a lovable warmth," she bubbles. "He's approachable and so talented. He's a wonderful role model to teens and an inspiration to all of us!"

When asked what his friends would say is a strength for him, he said he thought that they might say he is "reliable and easy to talk to," and the one they might call first to get things done. Christian smiled broadly when I asked if anyone had been especially supportive and meaningful to him. "Mom is definitely number one, but beyond her, a whole world and universe of people."

When asked what advice he would give to someone who had just been diagnosed, he offered, "Step one, breathe; it will be okay. Hemophilia is not an easy thing to live with. At the same time, it is not something that needs to hold you back. It does come with the added burden of responsibility, but if you are able to get to the point where you can live up to that responsibility, or you're able to help your child live up to that responsibility, it begins to open things for you that you wouldn't have otherwise been aware of." He went on to give examples such as understanding the importance of healthcare and being a resource for others going through a medical issue. Christian added, "If you're willing to walk on that path, it can help you create a life that you, one, didn't plan, but two, still enjoy. I'm thankful for how my life has evolved, and hemophilia is such a big part of that."

After earning an undergraduate degree from American Intercontinental University, Christian went on to receive an MFA in Fashion Design. Although the Savannah College of Art and Design (SCAD) set Christian up beautifully to succeed, his first months there made him question if whether he'd ever find his bearings. "It was rough," he discloses. "When I first started at SCAD, I was uninsured, because I was just stupid and wanted to do my education instead of prioritizing insurance. So, I ended up doing a clinical trial. That's how I got my medication through grad school. The problem was, I was also without a car at the time, so I was on the bus hauling ninety days' worth of factor, while I was working and trying to get my footing at SCAD."

Christian could see that his professors, not knowing his situation, thought he was uncommitted. "They didn't realize that I was having thirteen vials of blood drawn while I'm also trying to pull an all-nighter and get things done. "But halfway through this program, the clouds lifted, and "Christian L. Harris, Fashion Designer," began to emerge! Although his hemophilia impeded his progress in the beginning, it provided the inspiration to his crowning achievement at SCAD, and his "God Save the Prince" MFA thesis collection hit the runway.

That prince is Prince Alexei of the House of Romanov. Christian's love of history, art and geometry, underpinned by his firsthand knowledge and experience with the needs and discomforts hemophilia imposes on the body, wove together to create this artistic, dramatic playground of apparel. To view it and read the accompanying history behind Harris's inspiration, go to christianlharris.com/godsavetheprince.

On the importance of fashion, Christian ponders, "A photographer from the New York Times, Bill Cunningham, says that fashion is the armor we put on to survive everyday life. For me, personally, being a large, black man in fashion, I'm interested in exploring and using how I dress as a tool to disarm people; to change their perception of who I might be."

"I look at myself as a large canvas that I get to paint however I choose." He enjoys robbing people of the opportunity to assign their ideas onto him. "It's difficult for others to project their ideas onto someone who's wearing something that looks like it's half-inspired by Japanese garments, but then it's an African print, and the guy wearing it kind of looks like John Coffee from The Green Mile," he's smiling like the Cheshire Cat, and adds, "No one looks at me and automatically thinks I can create beautiful evening gowns!"

"(Fashion) does arm and protect me, because sometimes what I have to protect myself from is what people expect of me."

Currently, Christian is a 3-D leader of global development standards at Nike, which means he is a principle technical designer. He helps the company realize market-driven designs by working with designers, creating the garment patterns, determining the specifications the factory will need to sew them and managing the sometimes laboriously repetitive approval process before putting them in the hands of the factory. It's his job to make sure the garment meets the designers' and product managers' intentions, that the factory will be able to sew it, and that it'll come out on the other end as something solid.

One of the ways they "shorthand" that process is to use 3-D technology. "We'll make a pattern, put it in 3-D on a computer and first approve it there. "That way, the factory doesn't have to cut and sew it and send it back and forth. We can do that digitally."

Christian relates that this technology has been around for ten to fifteen years, but that computers are not yet able to capture the fundamentally amazing concept of cloth.

"We've been weaving cloth for thousands upon thousands of years. There's a reason a technology like cloth is still in our lives today. It's dynamic and amazing and just beyond what computers are able to capture. Even the best computer systems today still can't one-hundred percent-accurately replicate a fabric. So the technology is continuing to expand and try to catch up with the complexity that is garment-making and textile science."

Christian equates textiles with the science of cooking. "It's something we continue to use and evolve, but it's not going anywhere."

"Right now, I'm delving deeper and deeper into the apparel industry, and my aim is not necessarily to be a creative force, myself, but I want to help others who are in this industry stay connected to what they love. It's really easy, when you want to do something creative, for the corporate side to rip away so much of why you loved it in the first place.

"Using digital tools excites me, because it gives me new opportunities to help people stay connected to what they fell in love within the fashion industry in the first

place. To do that, I participate in conferences, panels, workshops and discussions to help streamline 3-D development and take it further.

"I like to work with the software engineers who code the software to help them understand, because they usually assume there is no need for them to know much about fashion. Once you break it down for them and explain how garments are engineered, it lights a different kind of fire with them in figuring it out!"

Christian tells a story that tickles him, "I had my engineers playing with different skirts that I'd made on a half-scale doll-like forms so they could understand how the fabric would drape and how changing the construction of something changes how it hangs. That was an effective tool to help them understand garment complexity in engineering. I love being able to connect people with information that's going to be able to help them do their job better, enjoy their work and make the apparel industry better."

As for his own creative expression, he tries to inject it into all of his projects. He is a patient ambassador for Medexus and Xinity. "Right now, we're doing this project with the (bleeding disorders) community where we've asked people what it means to be themselves. They decorate a "B" to reflect that. Then we scan the pages of colored "B's" and make a print,"

"I'm always excited about what I might see and who I might be on the other side of creating something new. Being myself is making new discoveries every day."

Christian's travel destinations always revolve around fashion, art, and history for him. While visiting Shanghai, he made sure to shop for silks and included art and history museums in his itineraries. He also shared that one of his favorite ways to remember a place he's visited is to purchase fabric from the destination and create clothing for himself as a souvenir. The one place he would like to visit the most is the Balenciaga Museum in Spain. When asked why, he shared, "Balenciaga is the greatest courtierian who ever lived, and his work is so next level that I get emotional."

Christian shared "If you had told me I would be where I am now five years ago, I wouldn't have believed it, so I hope I feel that same way five years from now." Less concerned with a defined path, Christian was more interested in the "next discovery" and continuing to grow as a person. I can almost hear his chosen theme song, "I Will Get There," by Boyz to Men playing in the background, and I believe him with all my heart.

SHARE YOUR STORY

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

READY TO DISCOVER WHAT COULD BE NEXT IN HEMOPHILIA TREATMENT?

Discover what may be on the horizon!

To learn more, scan the code or visit hemhorizon.com

Pfizer

FROM THE EAST TO THE WEST, THE B FAMILY IS THE BEST: 2023 MEETINGS ON THE ROAD RECAP

BY KATIE COLÓN

Between June and November 2023, the CHB team embarked on an exciting journey, visiting 12 cities to host our cherished Family Meetings on the Road. These one-day events, made possible by the incredible support of sponsors CSL Behring and Novo Nordisk, aimed to offer education, support, and empowerment to our B Family members. The gatherings featured engaging sessions with distinguished speakers from the hemophilia B community, focusing on health and wellness, future planning, navigating healthcare systems, and exploring new therapy treatments. With more than 200 attendees, our events created a space for intimate, family-oriented, and localized information sharing.

Our heartfelt gratitude goes to CSL Behring for sponsoring meetings in Indianapolis, IN, Philadelphia, PA, Boston, MA, Miami, FL, Concord, CA, New Orleans, LA, and Phoenix, AZ, and to Novo Nordisk for supporting meetings in Milwaukee, WI, Albuquerque, NM, Salt Lake City, UT, Austin, TX, and Cleveland, OH.

At the heart of these events were the diverse voices of our distinguished speakers, who, with personal connections to hemophilia B, shared their expertise and insights. From healthcare professionals and researchers to patient advocates and artists, each speaker brought a unique perspective, enriching the community with their valuable contributions.

This distinguished lineup of speakers highlights the diverse expertise and resilience within the hemophilia B community.

AUSTIN, TEXAS

CLEVELAND, OHIO

CONCORD, CALIFORNIA

INDIANAPOLIS, INDIANA

MIAMI, FLORIDA

Corbett Reinbold, RN, BSN, CCRC, a nurse instrumental in establishing Wisconsin's first hemophilia camp, and Dr. David Clark, Chairman of The Coalition for Hemophilia B, with expertise in plasma and tissue product development, led the lineup.

Dezarae N. Morales, RN, a Hemophilia Nurse Coordinator, collaborated closely on treatment guidelines, while Donnie Akers, Jr., ESQ., a seasoned attorney, volunteered extensively. Dr. Doris Quon, the medical director emphasizing interdisciplinary care, and Ellen Kachalsky, LMSW, ACSW, CCM, focusing on empowerment, added unique perspectives.

The roster also featured Fernando Reyes, M.ED.PSY, a holistic college counselor, and Gha'il Rhodes Benjamin, an award-winning spoken-word artist. Judy Doyle, a Novo Nordisk Liaison, Dr. Lisa Hensley, a virologist, and Lisa Littner, MPH, MSW, CHES, contributed diverse experiences.

Lucy Ramirez, MSW, LCSW, a clinical social worker, and Martha Boria Negron, a former teacher, highlighted community engagement. Matthew D. Barkdull, MS, MBA, LMFT, advocated for national healthcare, and Michelle Leona Cecil, a Novo Nordisk Liaison, supported the community. Dr. Mina Nguyen-Driver, a mental health specialist, and Mosi Williams, Psy.D, a Clinical Social Worker, offered valuable insights. Patricia Amerson, MSN, RN, CNP, FNP-C, a pediatric nurse practitioner, Dr. Richard Lemons, a pediatric oncologist, and Rick Starks, a tai chi enthusiast, enriched the diverse panel. Stacy Hilburn, a Novo Nordisk Liaison, Timothy W., a Patient Ambassador, and Wendy Wollner, CEO, brought unique perspectives, along with Dr. William Patsakos, Pharm.D, a CVS/Specialty executive and retired Army captain.

The diverse backgrounds and expertise of these speakers contribute to the holistic support, education, and advocacy within the bleeding disorders community. Each speaker delivered sessions based on their expertise and tailored to The Coalition for Hemophilia B's Family Meetings on the Road mission to provide education, advocacy, empowerment, and community. Their commitment and passion play a crucial role in enhancing the lives of individuals and families affected by hemophilia B.

Each event was unique and memorable because of our amazing CHB members who attended and brought themselves, their experiences, and their love. At The B Family Rap Session, community members openly discussed various aspects of their lives and shared insights on matters affecting the community. The forum was a vibrant space

ALBUQUERQUE, NEW MEXICO

SALT LAKE CITY, UTAH

filled with ideas, questions, and the warmth of friendship, fostering a sense of unity and connection among participants.

The events also featured the opportunity to engage in game show style play. Participants were divided into two teams, adding a fresh and enjoyable twist to childhood games that elicited laughter, friendly competition, and hemophilia B facts bringing a delightful end to the festivities.

Icebreakers were also an integral part of the gathering, serving as engaging activities to create a welcoming atmosphere. These interactive sessions successfully allowed participants to bond and strengthen the sense of community within the B Family.

Overall, the past event was a resounding success, bringing together community members in a spirit of unity, fun, and mutual support. The B Family remains a close-knit community, and such events play a crucial role in enhancing the bonds between its members.

BOSTON, MASSACHUSETTS

CSL Behring

novo nordisk®

COMMENTS:

"The Meetings on the Road are one of my favorite CHB events because they give you the opportunity to have more of an intimate interaction with not just local community members, but folks in your region."

"There's nothing better than knowing that "Someone's Got Your Back." During the hardest times in our lives and dealing with a child/children/family who has an

NEW ORLEANS, LOUISIANA

PHILADELPHIA, PENNSYLVANIA

MILWAUKEE, WISCONSIN

incredible amount of pain, anxiety, mobility issues, depression, emotional, physical, and mental stress, it is just a blessing knowing you're not doing all this alone. We have the Coalition to back us up. The community of family support is what we are. There are so many of us that are dealing with the same issues and understand us. We get it. We all get it. The education we get from the CHB, the support from our community members who are now family, and it's definitely because the CHB has changed the quality of our lives for the better through all the programming and outreach."

"The Meetings on the Road that the Coalition offers to the community are packed with invaluable information and family connections. If you and your family have never attended a MOR, you are missing out on an amazing opportunity. I hope you'll take advantage of these programs this year!"

"Meetings on the Road provided me and my loved ones an opportunity to meet other community members in my state. I was able to connect with one family that is going through similar hurdles that my sister and her family are going through. It was invaluable to learn from them on how to navigate the challenges of raising a healthy and confident son with hemophilia B."

"Attending a Coalition for Hemophilia B meeting is always special, family reunion special. The Meetings on the Road are even more so because they come to us, letting us know that no matter where we are in the country, the Coalition is always there for us."

"The Meetings on the Road symbolize an opportunity to connect with other hemophilia B families to share important experiences and lessons."

"I very much enjoyed the Hemophilia B Meeting on the Road in Albuquerque, NM in September of 2023. I often feel like I don't have much left to learn about managing bleeding disorders, and then I attend a Coalition event like this, and it proves me wrong. I learned a lot that day! In particular, Dr. Nguyen's session was very eye opening and engaging for me. (But they were all wonderful!) Also, the opportunity to be around my "B" family was something I really needed right then in my life. I loved meeting new family members and getting to spend time with my loved ones. Thank you so much for offering these events and the excellent educational opportunities you always offer. You have changed my and my family's life for the better on more levels than I can describe!"

***For more photos, please visit us on our website, Facebook, and in the CHB Educational Hub.**

PHOENIX, ARIZONA

RIXUBIS® [Coagulation Factor IX (Recombinant)] Important Information

What is RIXUBIS?

RIXUBIS is an injectable medicine used to replace clotting factor IX that is missing in adults and children with hemophilia B (also called congenital factor IX deficiency or Christmas disease).

RIXUBIS is used to control and prevent bleeding in people with hemophilia B. Your healthcare provider may give you RIXUBIS when you have surgery. RIXUBIS can reduce the number of bleeding episodes when used regularly (prophylaxis).

Detailed Important Risk Information for RIXUBIS® [Coagulation Factor IX (Recombinant)]

Who should not use RIXUBIS?

You should not use RIXUBIS if you

- are allergic to hamsters
- are allergic to any ingredients in RIXUBIS.

Tell your healthcare provider if you are pregnant or breastfeeding because RIXUBIS may not be right for you

What should I tell my healthcare provider before using RIXUBIS?

You should tell your healthcare provider if you

- have or have had any medical problems
- take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies
- have any allergies, including allergies to hamsters

What should I tell my healthcare provider before using RIXUBIS? (cont'd)

- are breastfeeding. It is not known if RIXUBIS passes into your milk and if it can harm your baby
- are pregnant or planning to become pregnant. It is not known if RIXUBIS may harm your unborn baby
- have been told that you have inhibitors to factor IX (because RIXUBIS may not work for you).

What are the possible side effects of RIXUBIS?

Allergic reactions may occur with RIXUBIS. Call your healthcare provider or get emergency treatment right away if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.
Some common side effects of RIXUBIS were unusual taste in the mouth and limb pain.

Tell your healthcare provider about any side effects that bother you or do not go away.

Your body may form inhibitors to factor IX. An inhibitor is part of the body's defense system. If you form inhibitors, it may stop RIXUBIS from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for development of inhibitors to factor IX.

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 RIXUBIS Important Facts on the following page and discuss with your healthcare provider.

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

Takeda

Important facts about RIXUBIS®:

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

This leaflet summarizes important information about RIXUBIS. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider.

What is RIXUBIS used for?

RIXUBIS is a medicine used to replace clotting factor (Factor IX) that is missing in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Hemophilia B is an inherited bleeding disorder that prevents blood from clotting normally. RIXUBIS is used to prevent and control bleeding in people with hemophilia B. Your healthcare provider may give you RIXUBIS when you have surgery. RIXUBIS can reduce the number of bleeding episodes when used regularly (prophylaxis).

Who should not use RIXUBIS?

You should not use RIXUBIS if you
- are allergic to hamsters
- are allergic to any ingredients in RIXUBIS

Tell your healthcare provider if you are pregnant or breastfeeding because RIXUBIS may not be right for you.

What should I tell my healthcare provider before using RIXUBIS?

You should tell your healthcare provider if you
- have or have had any medical problems
- take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies
- have any allergies, including allergies to hamsters
- are breastfeeding. It is not known if RIXUBIS passes into your milk and if it can harm your baby
- are pregnant or planning to become pregnant. It is not known if RIXUBIS may harm your unborn baby
- have been told that you have inhibitors to factor IX (because RIXUBIS may not work for you).

What is the most important information I should know about RIXUBIS?

Allergic reactions have been reported with RIXUBIS. Stop using the product and call your healthcare provider or get emergency treatment right away if you get a rash or hives; rapid swelling of the skin or mucous membranes; itching; tightness of the throat; chest pain or tightness; wheezing; difficulty breathing; low blood pressure; lightheadedness; dizziness; nausea; vomiting; tingling, prickling, burning, or numbness of the skin; restlessness; or fainting.

Your body may form inhibitors to factor IX. An inhibitor is part of the body's defense system. If you form inhibitors, it may stop RIXUBIS from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor IX.

The use of factor IX containing products has been associated with the development of blood clots. Talk to your doctor about your risk for potential complications and whether RIXUBIS is right for you.

What are the possible side effects of RIXUBIS?

Some common side effects of RIXUBIS were unusual taste in the mouth, limb pain, and atypical blood test results. Tell your healthcare provider about any side effects that bother you or do not go away. These are not all the side effects possible with RIXUBIS. You can ask your healthcare provider for information that is written for healthcare professionals.

Consult with your healthcare provider to make sure your factor IX activity blood levels are monitored so they are right for you.

You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia B learn to infuse their RIXUBIS by themselves or with the help of a family member.

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

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

The risk information provided here is not comprehensive. To learn more, talk about RIXUBIS with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at https://www.shirecontent.com/PI/PDFs/RIXUBIS_USA_ENG.pdf or by calling 1-877-825-3327.

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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Takeda

FALL MEN'S RETREAT MEN CRY TOO

BY SHELBY SMOAK

Let me be honest and say that had you told me 20 years ago that a weekend in Nashville without girls but with 30 other guys would be fun and exactly what I needed, I would not have believed you. And yet that is exactly the case. I have returned from the Coalition for Hemophilia B's Men's Retreat in Nashville, held Thursday, October 19 – Sunday, October 22, 2023, and I am better for it. If Nashville is indeed the city of music, then our place nearby might have just been the hotel of harmony.

Mornings began with the calming zen of Rick Stark's Tai Chi. I thought it might come with a lovely Tai-Chai latte, the kind that Starbucks does that are so milky smooth and sweet, but Rick had other plans. He was about balance and poise and was all Mr. Miyagi with the group! After a while, I no longer missed the latte. And this set the tone for the weekend event: being thoughtful, finding balance, improving our well-being, sharing, and kind of just being silly boys trying to forget about the hardships of hemophilia.

Many sessions addressed our emotional well-being. Cool-man bro Mark Zatyrka in, "Mental Well-Being Together," addressed the many ways dads of, partners of, and persons with hemophilia B could find support for the mental impacts of hemophilia B while big-hearted, easy going Mosi Williams followed in a later session with, "Dealing with Change." I thought it might be about all those pennies I have laying about, but it wasn't. Mosi got the conversation started about the infinite disruptions and unplanned accommodations hemophilia B forces. One thing people with hemophilia B have learned is that nothing is static, and 'change is gonna come. We learned the tools needed to navigate the journey.

I added to this conversation with "Singing to Heal" where I imparted my love of music and basically explained to the group that my brilliance is a result of music and the numerous ways it rewrites the brain. It helps with pain, too. Participants then rewrote and performed karaoke to hit songs, giving us great laughs from "I Wanna Clot," "Bleed It," and "Achy Breaky Arm." It was a shame Weird Al or Sony Records were not on hand to scoop up this talent.

President of the Coalition and all-around loveable guy Wayne Cook rolled out his best recipe salad demonstration. That's right! Us guys were throwing down in Nashville, making salad! A few learned exactly what a radish looks like, while a few more learned that not all peppers (like green and red ones) are hot.

Sponsor Pfizer contributed with two independent sessions: one that facilitated a group discussion regarding the tug a chronic illness has on our emotional wellness, and another that relayed new and novel therapies for hemophilia B. Things got a little serious when friendly Wall Street ace Matt Barkdull started talking about money.

No Men's Retreat would be complete without 2 things: Wayne's rap session, and The Bleeder Olympics! I think Wayne may have hit a record this year. No, not at The Bleeder Olympics. At the rap session because he had us crying in under 5 mins! This life is hard. We got 99 problems (and more), ya know. We had also just lost an amazing and dear friend Fonzy (Alfonso Jaramillo). We set a place for him at our table, we mourned his loss, we remembered him. As for the Bleeder Olympic, I was going for best limp, or least flexible, but those weren't categories. We hung together, played some corn hole, and just let it be.

One night we went to the Grand Ole Opry, but it looked pretty new to me, so I put a note in their suggestion box that they should rename it. It was deceptive marketing, I said, what with the new building and all. I was pretty excited because one of my ultimate favorite heavy metal acts was supposed to









play. I was a huge Motley Crue fan, and absolutely loved Vince Neil! I couldn't believe my ears when they told me he was playing the Grand Ole Opry. Well, he wasn't. Turns out it was Vince Gill. So, yeah. That was a let down for me. But everybody else loved it, and I could tell he had some real heart in those twangy tunes of his. And, of course, I had a good time, as we all did.

The evenings were really where it was at: we had 2 golf clubs and some wiffle balls. Top Golf was on! Some who were not reliving their atavistic heritage and swinging clubs shared stories or songs for the few who traveled with guitars. It was a true Hootenanny! We were dancing – the half step, of course – and singing – not well, naturally – but it didn't matter. We were together.

Throughout the weekend we were reminded of the closeness of the group, the need we each have to be supported by the other. Even for first timers, the welcoming spirit and generous heart is palpable. Would I do this again? Heck yeah! Should you go? Well, if you're a guy, YES! And maybe next Nashville trip, I will see Vince Neil. I'd also accept Bret Michaels if anybody is listening.

Thank you to Pfizer for making this incredible event happen. It is such a powerful experience for us to come together. The time we spend connecting with each other and learning together is priceless, and means so much to each of us!

COMMENTS:

"To me, the men's retreats mean family and community."

"It was great getting to meet so many Brothers that are dealing with hemophilia B, whether they have hemophilia or someone in their family has hemophilia. It is nice knowing that we are all there to support our new family members."

"It's hard to explain when there's an inner peace and very emotional connection that helps us mentally."

"It means a lot to me. It feels like one of the few places I can come and really be myself and be open with the other guys about what's going on in my life and how I'm doing."

"This event means disappearing into the arms of friends and leaving with a giant hug of love and support."

"Through this, I have a newfound support system."

"Very rewarding program! It always amazes me every time, since it helps us a lot to understand each other and helps us mentally. So empowering!".

"This program is essential to provide emotional support for those with hemophilia B and for those fathers who want to understand their sons and daughters."

*** For more photos, please visit us on our website, Facebook, and in the CHB Educational Hub.**

WOMEN'S VIRTUAL WINTER RETREAT: A WEEKEND OF CONNECTION, LEARNING, AND EMPOWERMENT

BY KATIE COLÓN

We are thrilled to celebrate a significant milestone in The B Hub's evolution as we hosted our first-ever virtual event in this space: The 2023 Virtual Winter Women's Education and Empowerment Retreat. The event, which took place virtually over the weekend of December 1-3, 2023, was a profound journey of self-discovery, connection, and empowerment that transcended the conventional event experience. Its unique tapestry of informative sessions, engaging workshops, and moments filled with joy and laughter, left participants feeling empowered, rejuvenated, and connected with their B Family. This remarkable event brought together women from different parts of the country to connect, learn, and inspire one another.

This event marked a historic moment for The Coalition for Hemophilia B, showcasing its capability to seamlessly host virtual events in a secure, private, and accessible space just for our community. Whether in a bustling city or a tranquil rural area, members were able to connect in new and exciting ways through this unprecedented online resource center and community platform.

Friday night kicked off the retreat with insightful sessions and engaging activities. Author Susan Salenger led a thought-provoking session titled "Sidelined: How Women Navigate a Broken Healthcare System," sharing her research findings about the unique challenges women face when managing their healthcare.

The night concluded with the much-anticipated "Opening Night Pajama Party," a virtual gathering that included Bingo, heartwarming conversations, music, and laughter. The journey commenced with this joyous event, where laughter and stories flowed freely, creating a virtual haven that bridged distances and united the community in a spirit of togetherness. This warm kickoff set the stage for a weekend dedicated to connection and empowerment.

Saturday morning began bright and early with a rejuvenating session led by Catherine Canadeo, a Holistic Health Coach, Board Certified by the American Association of Drugless Practitioners and an Integrative Nutritionist. Participants engaged in gentle mindfulness exercises, starting their day with a focus on self-discovery and positive energy. Catherine included tips and tricks to help participants to not sweat the small stuff.

Next up, CHB's very own Erica Garber led the group in an "Arty Tea Party" where participants were able to take a moment from their busy lives to intentionally slow down. Participants were invited to discover the joy of self-expression through easy and enjoyable drawing techniques. Amidst sipping tea, engaging in lively conversations, and embellishing wooden boxes with unique touches, attendees not only crafted beautiful keepsakes but also forged connections destined to last beyond the retreat. The event encapsulated the essence of camaraderie, creativity, and lasting memories.

In the afternoon, two breakout sessions offered valuable insights. "From Clinic to Kitchen Table: Infusing Kids at Home" with Carrie Koenig celebrated the diversity of home infusion experiences and provided strategies for families who are just starting this process. Simultaneously, "The Joint Movement: Keep It Together" with Alexis Akins focused on the importance of joint health for individuals living with hemophilia.

These breakout sessions allowed participants to choose what they were most interested in and what would provide the most value to their lives and the lives of their families.

In a captivating keynote, Dr. Jill Johnson, MD, an expert in classical hematology, delivered an educational and impactful overview on the Hemophilia B diagnosis. As a researcher at the Bloodworks Research Institute and an Associate Professor of Medicine at the University of Washington, Dr. Johnson shared cutting-edge insights into the genetics of clotting factors, with a focus on factors VIII, IX, and von Willebrand. Her expertise in blood group genetics, particularly ABO and Rh systems, contributed to a comprehensive understanding. Dr. Johnson's commitment to unraveling the unique impact of bleeding disorders on females, especially in hemophilia and von Willebrand disease, made the presentation an enlightening journey, fostering collective commitment to advancements in research and patient care.

Sunday morning commenced with another session of "Morning Rejuvenation," led by Catherine Canadeo. The emphasis on holistic well-being and self-care set a positive tone for the day. Following the same wellness journey, a session on "Ayurveda for a Balanced Healthy Life" followed, featuring Aura H. Bermúdez, a licensed psychologist and Ayurvedic Health Counselor. Participants took away a variety of tools to prioritize self-care and wellness while navigating life with hemophilia B.

The day continued with a unique session titled "Rough Patch Roadmap," led by Gha'il Rhodes Benjamin. Participants co-created a roadmap for finding solace through life's challenges, indulging their senses, and nurturing their souls.

The closing session, "Chit Chat & Chocolate," featured personal stories from community members, Aamina and Debbie. This heartwarming session provided a space for bonding, laughter, and shared experiences, further strengthening the connections forged over the weekend. The retreat ended on a celebratory note with a raffle, offering surprise prizes to participants.

Overall, the Virtual Winter Women's Education and Empowerment Retreat was a journey of self-discovery, connection, and empowerment. It was a unique and enriching tapestry of informative sessions, engaging workshops, and moments filled with joy and laughter. We express our sincere appreciation to Sanofi, the gracious sponsor of the Virtual Winter Women's Education and Empowerment Retreat, for their unwavering support and commitment to the well-being of women affected by Hemophilia.

sanofi

COMMENTS:

"It was so great to get together with everyone. I always look forward to connecting with everyone in this group. I love to hear all of their amazing stories."

"You all are such an amazing group of warriors. I am so glad to have found all of you!"

"It meant a lot to me because now I feel like I have the support that I always wanted from someone that's going through the same thing that I'm going through and dealing with on an everyday basis."

"I always enjoy my time with my blood sisters. They are such an amazing source of love and support. They inspire me to keep shouting out about women's struggles with getting adequate treatment. I enjoyed our tea party with our awesome teacups and our gorgeous hair accessories!"

"I love coming together with our Coalition for Hemo B moms because I always leave the retreat feeling uplifted, appreciated, and understood. We are an amazing group that supports one another. The Coalition always finds outstanding speakers to energize us, revive us, and motivate us to be an even greater version of ourselves. I count this retreat as self-care time!"

*** For more photos, please visit us on our website, Facebook, and in the CHB Educational Hub.**

GEN IX FALL EXPERIENCE:

BUILDING CONNECTIONS THROUGH ADVENTURE & EDUCATION

BY ERICA GARBER

The Generation IX Fall Experience, held in the scenic Imperial Beach, CA, from October 26 to 29, 2023, unfolded as a transformative journey immersed in the captivating themes of community, advocacy, and social capital. Our hemophilia B community members engaged in activities that explored the nuanced dynamics of healthy ecosystems, highlighting the connection between individual well-being and the overall health of the community. By venturing into the outdoors and enjoying our spacious Surf Camp venue, the program provided a healing experience while soaking in the San Diego sunlight and coastal breeze.

Adding a splash of adventure, participants had the opportunity to take on the waves at Mission Beach. We donned our wetsuits and helmets and hit the surf! Throughout this journey, the GutMonkey staff guided participants through experiences that pushed boundaries and facilitated personal growth. The appreciation extended to the GutMonkey team was a

testament to their role in creating an environment that blended adventure, learning, and community-building seamlessly. Thanks to their encouragement, we conquered the waves—many of us for the first time—solidifying bonds formed during this experience.

A big highlight was our day trip to the San Diego Zoo, offering a unique opportunity to apply newfound insights about social structures and community systems by observing the fascinating dynamics of the animal kingdom. At the zoo, participants marveled at the diverse array of wildlife. We met giraffes, rhinos, and many more animal friends, up close and personal on a safari ride! We marveled at the beauty of the California Condor and giggled along with the Laughing Kookaburra in the immersive aviary systems in the park. The intricate social interactions and unique behaviors of each species provided not only joyful memories but also valuable perspectives on the interconnectedness of communities, reinforcing the program's themes.

Special gratitude was expressed to Medexus; without their generous support over many years, this program would not be possible. Our friends at Medexus are a wonderful example of what partnership with purpose can do by bringing meaningful community initiatives to fruition.

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The Generation IX Fall Experience was a harmonious blend of adventure and education, set against the backdrop of Imperial Beach's natural beauty. From the unpredictable weather to the oceanic escapade and the captivating visit to the San Diego Zoo, every detail contributed to an unforgettable journey, fostering connections, promoting advocacy, and showcasing the vital role of social capital in community well-being.

COMMENTS:

"I get so much value out of this type of program. The connections I make are incredible. I learn better by doing and experiencing."

"The Generation IX experience was one of strong emotions and lots of love and empowerment. I had a great time, and it was fun to see all of the family again."

"I am so grateful to Medexus for sponsoring, CHB for their partnership, and GutMonkey for building an engaging and fast paced program where we learned so much about how we can help each other and help the community."

*** For more photos, please visit us on our website, Facebook, and in the CHB Educational Hub.**

GINGERBREAD HOUSE DECORATING: A NIGHT OF FUN, TRIVIA, AND SWEET CREATIONS!

BY KATIE COLÓN

We're excited to revisit a memorable event that took place on Saturday, December 16th, 2023 —our Gingerbread House Decorating and Trivia Night. This festive gathering was a delightful celebration of creativity, camaraderie, and seasonal joy.

One of the highlights of the event was the opportunity for participants to come together in the spirit of the season. With the first 50 registrants receiving a Gingerbread Kit, attendees were equipped with all the essentials to create their sugary masterpieces. The event aimed not only to entertain but also to foster connections within our community, bringing smiles and laughter to everyone involved.

As the evening progressed, attendees were treated to a Festive Version of "This or That," adding an extra layer of merriment to the festivities. Laughter echoed as participants shared their preferences and discovered commonalities, creating an atmosphere of joy and togetherness.

While the event provided plenty of fun, it also embraced the educational side of things. Our Trivia Night featured an education twist— questions centered around informative tidbits related to hemophilia B. Participants engaged in a lively exchange of knowledge, learning more about hemophilia while enjoying the thrill of competition.

For those feeling a bit competitive, an optional Gingerbread House Decorating Contest was held that turned the event into a festive competition with rewards for participants who demonstrated exceptional knowledge and creativity. Participants showcased their culinary skills by submitting photos of their delectable creations. Congratulations to our winners: 1st Place: The Gardner Family; 2nd Place: The Duray Family; and 3rd Place: The Gregory Family.

The heart of our community beats stronger when we come together. Throughout the evening, the sense of community was palpable, with participants sharing their progress, exchanging tips, and celebrating each other's achievements. The virtual space transformed into a hub of positive energy and support.

This enchanting experience was made possible by the support of our wonderful sponsors— Medexus Pharma and Paragon Hemophilia. Their commitment to our

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community's well-being and happiness was evident throughout the event.

COMMENTS:

"This festive event was a joy to do with the whole family and brought us closer to our community."

"I am so thankful that the Coalition for Hemophilia B brings families together for sessions like the Gingerbread House Decorating. It's something that we look forward to every year!"

"We had so much fun! This was our second year participating. We are already brainstorming gingerbread house ideas for next year."

"Building a gingerbread house together with the Coalition on zoom, has become a fun family tradition that we look forward to every year! Thank you for bringing us together before the holidays!"

"Our family LOVES the gingerbread event! The kids are always so excited when I let them know it's happening and can't wait to get their creative minds to work. Being able to see our friends on the computer makes my son so happy to know he is not the only person with hemophilia."

*** For more photos, please visit us on our website, Facebook, and in the CHB Educational Hub.**

THE COALITION FOR HEMOPHILIA B

THE B EDUCATION HUB

The Community Hub & Resource Library

Through a comprehensive range of resources and support, CHB is committed to enhancing health literacy, promoting mental and physical well-being, and ensuring a holistic approach to care for the Hemophilia B community. Education is our best tool and together we can make a tremendous difference in the lives of our community members.

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HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

Hemophilia Carrier Screening

12/9/23 A group of physicians and researchers from Belgium pointed out that "Despite multiple awareness-raising initiatives, and in contrast with male persons with hemophilia (PwH), a considerable number of carriers and female PwH still go undiagnosed." At the American Society of Hematology (ASH) meeting they presented the early findings of a carrier screening project.

They began by updating the family trees for all of the patients at their center and offering to genetically screen all of the females who hadn't already been screened. In 228 families (180 A; 48 B), they found 900 females of which 454 were obligate carriers. An obligate carrier is a female whose father has/had hemophilia. She will be a carrier of her father's mutated gene but may or may not have hemophilia herself. They also found 118 females who were not carriers, about 13%. This suggests that if you are a female in a hemophilia family, you only have about a 13% chance of not being a carrier. They also found eight women who should have been carriers based on the family trees but did not carry their "father's" mutated gene and were actually not carriers. With so many subjects, genetic testing is still ongoing, so this is an interim report, but it still shows some concerning results.

Among the carriers of hemophilia B, 29.5% had factor IX levels below 40% of normal (considered the lower limit of normal internationally) and 41.0% had a factor level below 50% (the lower limit of normal in the U.S.). Note that women have been reported to bleed even with levels in the 60% range. In the hemophilia B-group, 1.3% of carriers had factor IX levels of 6-15%, 26.9% had levels of 16-39%, 24.4% had levels in the 40-60% range and the rest (about 47%) had levels above 60%. The proportions for hemophilia A were similar, plus there did not appear to be a significant difference between families with mild, moderate or severe hemophilia. The only significant difference is that the Bs had more instances of reproductive tract bleeding at 38.9% than the As at 5.7%.

The important finding here is that the average time to diagnosis for the females was seven years later than for the men included in the study as control subjects (members of the same families). Even for the women with factor levels below 40 or 50%, who actually do have hemophilia, the average age at diagnosis was 31.8 years for the Bs (25.8 for the As). (Note that Belgium has universal health care. The numbers in the U.S. may be worse.) The report concludes with the statement, "The carrier screening efforts that have been initiated at our center, and which should ideally be replicated across the hemophilia community globally, appear as a critical step towards providing equal access to hemophilia diagnosis and care to all potentially affected individuals, regardless of their gender." [Krumb E et al., ASH abstract 288]

Is Inhibitor Development Affected by the Product Used?

11/20/23 Inhibitor development in hemophilia is still a mystery. There is a lot that we don't know. One question that has come up over the years is whether the product used to treat the hemophilia patient affects inhibitor development. The answer has never been clear. Recently a large group of European and Canadian researchers decided to re-address that question. They looked at previously-untreated patients (PUPs – usually kids) with either hemophilia A or B at 56 European treatment centers and 23 Canadian centers. They found 312 subjects out of 1219 total hemophilia A severe PUPs who developed inhibitors, about 26%. For the As, inhibitor development was lower on plasma-derived factor VIII products; highest on standard half-life (SHL) products and intermediate on extended half-life (EHL) products. Inhibitor development rates also varied among the various SHL and EHL products.

For hemophilia B, the situation appears to be different. First, overall Bs had much lower inhibitor development rates, an average of 8% (14 of 173 study subjects). And, because there were many fewer subjects, the statistics were not able to show any significant difference among products. The results were that 11% (CI: 3-25%) developed an inhibitor on plasma-derived products, 8% (CI: 3-15%) on SHL products and 7% (CI: 1-22%) on EHL products.

Now, you might be thinking that's the answer: plasma-derived is worse than SHL, which is worse than EHL. You would be wrong, and that's why I included the confidence intervals (CI) with the numbers. In fact, this study doesn't show any difference among the products.

The confidence intervals (CI) show the statistical ranges

in which we believe that the result lies. For instance, with plasma-derived products the result is 11%, but we can only say with confidence that the actual number, if we included all possible subjects, is between 3% and 25%. To reduce the size of the confidence interval would require more subjects, but subjects with hemophilia B and an inhibitor are harder to find. Since the confidence intervals all overlap, we can't say that the results are different.

Statistics are extremely important in science and medicine. Although the media hardly ever publish the statistics for the public to see, scientific publications usually do. Otherwise, we could be easily misled. Using the above example, for instance, I could say that 11% of hemophilia B PUPs develop inhibitors on plasma-derived products. My friendly-competitor in the next lab, however, could completely accurately tell me I was wrong – I only showed that the inhibitor rate is somewhere between 3% and 25%. That's quite a difference, and I should be careful about staking my argument on the fact that I think the result is exactly 11%. Statistics show that 3%, 11%, 25% or anything in between are equally valid results from my data. (There goes my Nobel prize!)

Normally, we don't show you the statistical results, but I'm showing them to you here, so you can get a better idea of how science actually works. We always look at the statistics before reporting findings in this newsletter, even if we don't show them.

You don't just do an experiment and get a result and that's that. Usually, you do a number of experiments so you can get a statistical idea of how good your answer is. In medicine especially, we have to have good confidence in our results. We don't just test one subject and assume that applies to all other patients. We have to test a number of subjects and average the results to get a good idea of what's actually going on. The more subjects we test; the better our answer is.

So, the next time that your neighbor boasts that he's getting 50 mpg from his new car, ask him what the confidence limits are on that number – maybe he had a tail wind? [Fischer K et al., Res Pract Thromb Haemost, online ahead of print 11/20/23]

Intradermal Injection Can Cause Inhibitor Development

11/4/23 Inhibitor development in hemophilia A can often be reversed by a method called immune tolerance induction (ITI) in which high factor VIII doses are given repeatedly over a period of time. However, ITI doesn't work very well for hemophilia B, for unknown reasons. A group of US researchers wondered whether giving hemophilia B inhibitor patients factor IX injections into the skin (intradermal injections, ID) might work better. It didn't, but their method may have given them an important insight into hemophilia B inhibitor development.

They found out that factor IX injection into the skin is actually a great way to give someone an inhibitor. Your skin is not just a simple covering for your body – it is a much more complex tissue that really protects you from a lot of outside dangers. It contains enzymes that can break down any foreign proteins that try to enter your body through the skin, plus a number of immune system components, including antibodies, that can fight off bacteria and viruses.

In hemophilia B mice, the researchers found that injection into the skin triggers inhibitor formation at about a 100 times lower dose than is typically needed to produce inhibitor formation by intravenous (IV) injection. Interestingly, however, they also found that ID factor injections seemed to keep the mice from developing the anaphylactic reactions that are often seen in B inhibitor patients. An anaphylactic reaction is a major allergic reaction that can be life-threatening.

So, what does this mean for inhibitor patients and also for products being developed for subcutaneous (SC) injection? Most B inhibitor patients probably did not acquire their inhibitors by ID contact, but it's possible and needs more study. For the SC products being developed, it could be a concern? SC products are injected under the skin, not into it, but still in close proximity.

As the authors point out, we have already seen cases in which manufacturers of currently-licensed EHL products have tried to develop an SC version. Development of two potential EHL-SC products was discontinued because of increased inhibitor development. This has been the case even with products that do not produce inhibitor formation when injected IV. This could be a can of worms that needs to be further explored. [Sherman A et al., Res Pract Thromb Haemost, online ahead of print 11/4/23]

Iron Overload in Hemophilia Joint Damage

12/6/23 We don't know exactly what, on a molecular level, causes hemophilic joint damage. We know it's caused by bleeding into the joints and there is evidence that iron from the hemoglobin in the blood is involved. In the last issue, we saw that a protein called YKL-40 is probably involved. Now another piece of the puzzle is becoming clear.

A group from China has now shown that the excess iron from the blood can trigger macrophages to transform into an inflammatory form. Macrophages are a type of white blood cell, part of the immune system.

When you bleed into a joint, macrophages are one of

the types of immune cells that arrive at the bleeding site to clean up the mess. Chronic inflammation from repeated bleeds leads to damage to the synovial tissue around the joint, which is called synovitis. [Pang N et al., Haemophilia, online ahead of print 12/6/23]

Scramblases ?

8/25/23 Many of you have seen diagrams of the coagulation cascade (blood clotting system) and assumed that we therefore know everything about how blood clotting works. Not so fast! The diagrams that you see are only an approximation. There are many other molecules involved, probably many that haven't even been identified yet, and many other processes. The diagrams give us a framework to think about clotting but aren't the final answer, by far. We are still trying to understand the complex system that is blood clotting and still finding new components. Another relatively new group of compounds involved is the TMEM16 family of scramblases.

One of the major findings about clotting happened in the 1980s when it was discovered that the clotting reactions actually take place on surfaces, not in solution out in the bloodstream. The surfaces are the broken cell walls at the injury site and the cell walls (membranes) of activated platelets. This makes sense because you want the clotting reactions to be at the site of injury, not floating away in the bloodstream. Now we're finding that those surfaces are not just passive but also participate in the clotting process. To explain this, we need to learn a little about cell membranes.

The cell wall or cell membrane is the covering of the cell. It is made of molecules called phospholipids. These are fatty molecules (lipids) that also contain phosphorus groups. They are long molecules that line up next to each other in a double layer to completely enclose the contents of the cell, as shown in the following diagram.

In this diagram[i], the image on the left shows a cutaway view of a cell surrounded by the cell membrane. Intracellular refers to inside the cell and extracellular refers to outside the cell. The blown-up image at the top shows the structure of the cell membrane, which is made of a phospholipid bilayer

The bilayer consists of two layers of phospholipid molecules lined up with their heads (round circles) facing the outside of the membrane and the tails (long sections) on the inside of the membrane. Because of the way the different parts of the phospholipid molecules attract each other, this forms a strong but flexible covering for the cell. The cell membrane also regulates which materials can pass in or out of the cell.

There are a number of different types of phospholipid molecules that make up the cell membrane. One of the most prevalent (about 15% of the total) is called phosphatidylserine (PS). It is procoagulant (promotes clotting) because it is the molecule to which many of the clotting factors bind during clotting.

In the endothelial cells (ECs) that line the inside of the blood vessels, the PS molecules are all on the inside wall of the cell membrane where the blood can't see them. The wall of the ECs that are in contact with the blood has no PS and is therefore anticoagulant – it impedes clotting.

When you have an injury the EC walls are broken open, which exposes the blood to the PS molecules on the inside of the cell wall. That provides a surface containing PS on which the clotting reactions can proceed. Tissue factor, which is a protein that is also on the inside wall of ECs, is also exposed and starts the clotting process by activating factor VII, which binds to the PS-containing surface.

Tissue factor and factor VII start the clotting process, but to really get enough clotting activity going requires activating the other clotting pathway, the pathway that includes factors VIII and IX. That pathway amplifies the clotting signal to produce enough fibrin to actually seal the blood vessel closed. That requires more PS-containing surface for the clotting reactions. That's where the scramblases come in.

The recently discovered scramblases scramble the arrangement of the phospholipid molecules in cell walls, bringing the PS to the outer surface of the injured cell and its neighboring intact cells. That provides more sites for the clotting factors to bind to while forming the clot. It also continues to localize the clotting reactions in the area of the injury.
Is the scramblase rearrangement really important? Apparently so, because when they looked at mice in which the scramblases had been inhibited, they saw that 50% of the clotting activity had also been inhibited. They also saw that fibrin (the protein that makes up the clot) did not stick to the EC surface. That means that the clot doesn't stick to the injury site. Thus, this rearrangement that brings PS to the surface of the endothelial cells appears to be very important.

What does this mean for the average hemophilia patient? Probably not much, at least not right now, but as we continue to learn more about the clotting system, we potentially will be able to find better treatments for clotting and bleeding disorders. [Prouse T and Majumder R, J Thromb Haemost, online ahead of print 8/25/23]

Reference

i. From Oregon State University at https://open.oregonstate.education/aandp/chapter/3-1-the-cell-membrane/, accessed 1/22/24.

HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Winter 2023

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

IMPROVED FACTOR PRODUCTS

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

Expert Opinion on HEMA Biologics' Sevenfact for Inhibitor Treatment

8/21/23 A group of U.S. researchers recently published an expert opinion on the use of HEMA Biologics' Sevenfact product for treatment of hemophilia A and B patients with inhibitors. Sevenfact, licensed in 2020, is a recombinant activated factor VII (FVIIa) product that joins two other bypassing agents, NovoSeven and FEIBA, currently available for inhibitor treatment. Bypassing agents induce clotting in inhibitor patients by bypassing the factor VIII/factor IX step in the clotting cascade. FEIBA is a plasma-derived mixture of activated clotting factors (including FVIIa), while Sevenfact and NovoSeven are recombinant versions of FVIIa.

Sevenfact is produced in the milk of transgenic rabbits which gives it a different glycosylation pattern than NovoSeven, which is produced in cell culture from baby hamster kidney (BHK) cells. Glycosylation refers to chains of carbohydrate (sugar) molecules that are attached to many mammalian proteins. Sevenfact and NovoSeven have identical protein backbone structures but with different carbohydrate chains attached. That small difference seems to make a difference in how the products perform. For instance, Sevenfact appears to exhibit enhanced binding to activated platelets, which may help improve clotting. It also appears to inhibit protein C activation, which reduces the amount of anticoagulant activity at the site of the clot.

So, does this mean that Sevenfact is better than NovoSeven? Not at all! Remember that every patient is different. Some inhibitor patients will do better on NovoSeven and some will do better on Sevenfact. Some will see no real difference. Note that FEIBA is not used as often for hemophilia B inhibitor patients because it contains factor IX, to which many B inhibitor patients develop an allergy.

One of the important issues that this article points out is that research on inhibitor treatment for hemophilia patients is decreasing. The majority of inhibitor patients have hemophilia A and are now being treated with Hemlibra. There are so few inhibitor patients with hemophilia B that it is becoming difficult to recruit enough subjects for a meaningful clinical study. Fortunately, most of the rebalancing agents (see below) being developed also work for inhibitor patients, so inhibitor patient's standard method of treatment may be changing (and improving). [Pipe SW et al., Expert Review of Hematology, 16(10), 715-729, 2023]

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 anticoagulants in the system. Most of these agents work to help restore clotting in people with hemophilia A or B, with or without inhibitors.

Pfizer's Marstacimab Licensure Expected in Late 2024 in U.S.

12/11/23 Pfizer is developing marstacimab, a rebalancing agent that inhibits tissue factor pathway inhibitor to restore clotting. Marstacimab is a once-weekly subcutaneous treatment delivered via an auto-injector pen for treatment of hemophilia A and B patients, with or without inhibitors. They have completed their clinical studies (described in the previous issue) and submitted their license applications to the FDA in the U.S. and to the EMA in Europe. The FDA has set an action date for their response in the last quarter of 2024. The EMA response is expected in the first quarter of 2025. [Docwirenews article 1/11/24]

GENE AND CELL THERAPY

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

FDA Developing Pilot Program for Collaborative Review of Gene Therapy Products

1/12/24 The U.S. FDA is working on a pilot project to explore the possibility of concurrent collaborative review for gene therapy products with other global regulators. The goal will be for various worldwide regulatory bodies to share in the review of new gene therapy products, instead of each country reviewing the applications separately. One field that should be positively affected by the project is rare diseases. Where there are only a few patients with a rare disease in a country, it may be difficult for a company to pursue licensure. Pooling the data and regulatory reviews is expected to make it easier for countries to approve products for rare diseases. [RAPS article 1/12/24]

Pfizer's Beqvez Gene Therapy Approved in Canada

1/3/24 Pfizer is developing Beqvez (fidanacogene elaparvovec), 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. They announced that Canada has approved the product. Licensure is also pending in the U.S. and Europe. The FDA has set a decision date in the second quarter of 2024. [Biospace article 1/4/24]

WHAT'S A CoRe MANAGER?

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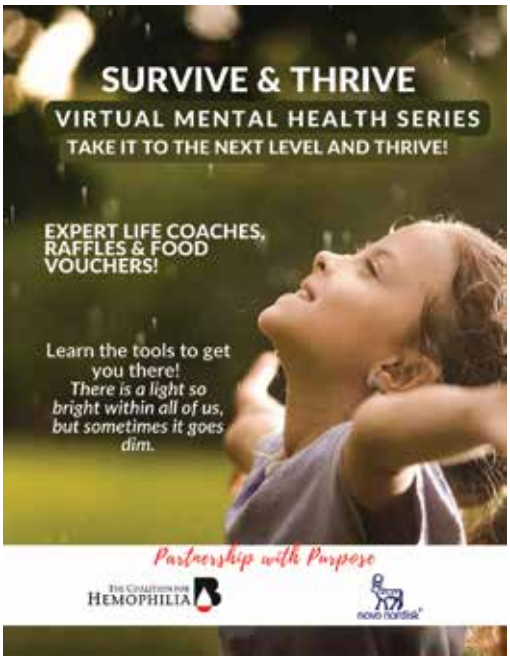
SURVIVE AND THRIVE: A RECAP OF EMPOWERING MEETINGS

BY KATIE COLÓN

As we reflect on the past year, we are filled with gratitude for all of the incredible journeys we've embarked on together including the "Survive and Thrive" series of virtual meetings. These gatherings not only held moments of connection and fun but also invaluable opportunities for self-discovery, holistic well-being, and the exploration of ancient practices. Every session was unique, each contributing to our collective journey toward not just surviving but thriving in our daily lives.



Our first meeting in the series on September 20th, focused on Ikigai. Led by the esteemed Catherine Canadeo, CEO of Catherine Canadeo Health and Wellness Corp, this session introduced us to the profound Japanese concept of Ikigai. Catherine expertly delved into the components of Ikigai, emphasizing the delicate balance between passion, mission, vocation, and profession. Through her insights and executive wellness coaching expertise, we gained valuable tools to redefine our personal Ikigai—setting the foundation for not just survival but true thriving.

The next time we met for this series on October 18th, Vanessa Harris, a Holistic Nutritionist, guided us on a transformative journey to revitalize our energy levels. With a keen focus on daily habits, Vanessa encouraged self-reflection to distinguish between practices that uplift and those that deplete. Her expertise in hormone imbalance and digestive health empowered us to embrace a life filled with renewed vigor and vitality. This session was a testament to the power of holistic approaches to well-being.

On November 29th, we focused on prioritizing self-care and navigating the holiday season with ease. In preparation for the festive season, Debbie de la Riva, LPC, shared practical tips on prioritizing self-care with calm and resilience. As a licensed mental health therapist with extensive experience in the bleeding disorders community, Debbie's tailored approaches ensured that the holiday season was something members felt prepared to navigate. Her insights were a beacon of support during a time often filled with both joy and stress.

Lastly, we were happy to share an intro to Ayurveda on December 7th. Aura H. Bermúdez, LP, enlightened us on the ancient Ayurvedic practice of seasonal routines. Exploring the connection between our bodies, senses, and changing seasons, Aura's session provided profound insights into aligning our lives with the wisdom of Ayurveda. Complementary dietary tips enhanced our understanding of sustaining health seasonally, creating a holistic approach to well-being.

Each meeting was not just an opportunity to acquire valuable knowledge but also a chance to strengthen connections within our community. The diverse expertise of our speakers—from holistic health coaching to Ayurvedic practices—contributed to a rich and holistic understanding of well-being.

A warm and heartfelt thank you to Novo Nordisk for their support behind this virtual event series. Their sponsorship has been instrumental in creating a space for our community to come together, learn, and grow.

novo nordisk®

COMMENTS:

"I liked having these helpful sessions during the fall, going into the cold winter months with some positive ideas to thrive."

"This was a wonderful way to connect and de-stress. Remembering that we are a connected community means everything."

THE HEMOPHILIA B PRODUCT LANDSCAPE COMMUNITY MEETINGS

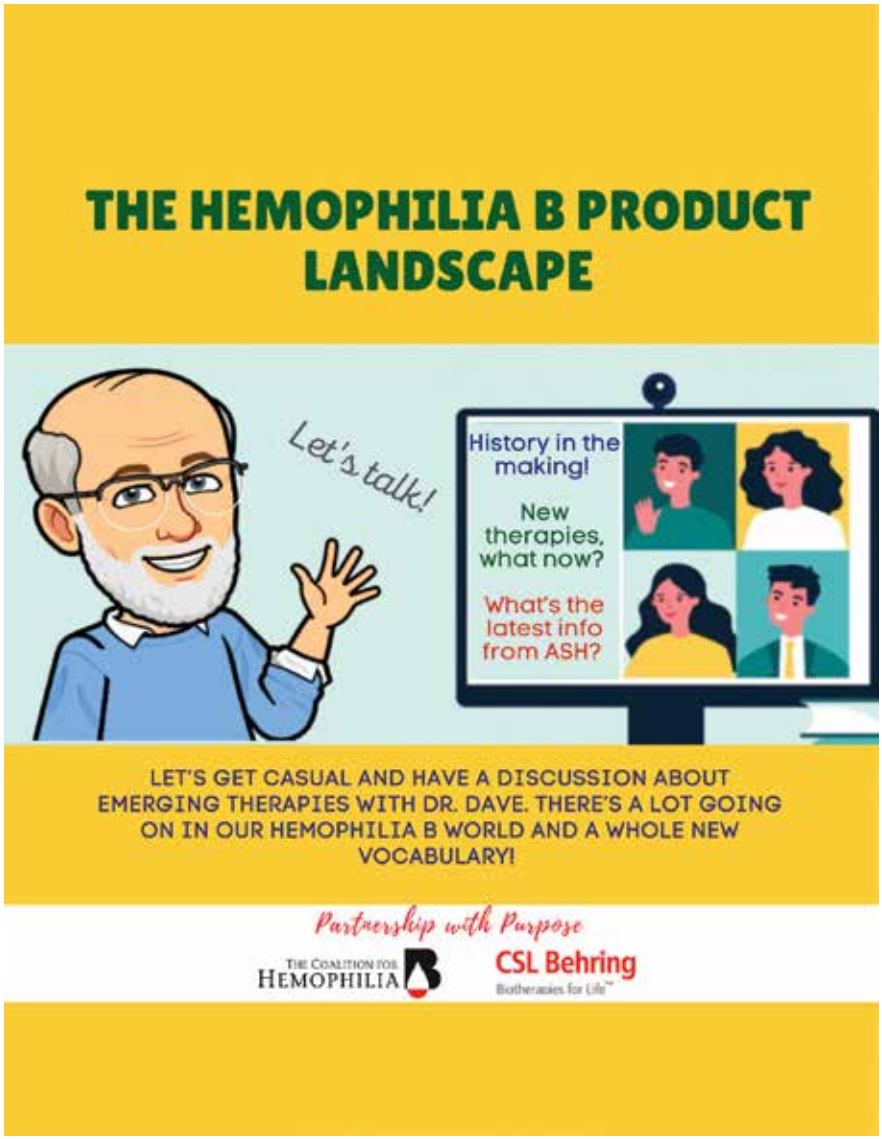
DR. DAVID CLARK

On November 8, 2023, and January 25, 2024, the Coalition held virtual community meetings about "The Hemophilia B Product Landscape" and I had the opportunity to host them!

I'm Dr. David Clark, Chairman of the Coalition, and I gave talks about current treatments for hemophilia B as well as new treatments under development. I always start my presentations with a slide that says, "Every Patient is Different." Every patient, even closely related ones, responds differently to different products.

We kicked off the meetings with some background. Around 1990, there were really only two plasma-derived factor IX products for hemophilia B, Alphanine and Mononine. There were also Factor IX Complex products, but those tend to cause thrombosis when used for prophylaxis or surgery. Thus, choices were limited.

If these products worked for you, great, but if they didn't, you just had to get by as best as you could. In the early 1990s, we got the first recombinant product, BeneFIX, but still choices were limited. Then, nothing for a long time. But now, we seem to be in a golden era for hemophilia B product development with many products available and many more under development.



Today, there are seven factor IX products available. Alphanine, the only plasma-derived product, is still available, and there are patients who actually do better on it than on recombinant products. We don't know why, but remember every patient is different.

There are three recombinant products, which are genetically engineered versions of the factor IX in plasma. They have slight differences, and some might work better in some patients than in others. These four are standard half-life (SHL) products, that is, they all have approximately the half-life of the factor IX in plasma, about 21 – 25 hours.

We then explored the current landscape. More recently, we've seen the approval of extended half-life (EHL) products with half-lives in the range of 83 – 104 hours. These products stay in the circulation longer, so they require fewer infusions. They also have the added benefit that they keep a patient's factor IX levels higher and thus provide more protection from bleeds. The

three current EHL products are quite different from each other and also may work differently for different patients.

Inhibitor patients also have three choices of bypassing agents, one plasma-derived and two containing recombinant activated factor VII (FVIIa). These give inhibitor patients some choice, but inhibitor treatment is still a difficult proposition. That will hopefully be about to change.

There are three general categories of new products under development, improved factor products, rebalancing agents and gene therapy. Improved factor products under development include one EHL factor IX and several new FVIIa products for inhibitor treatment.

The improvements include subcutaneous injection and longer half-lives. Subcutaneous (SC) injection is injection under the skin, which is a much easier process than intravenous infusion. It uses a short needle, which can just be poked into the skin, no need to find a vein, very similar to the way a diabetic injects insulin.

The rebalancing agents are a unique new method to tweak the clotting system to prevent bleeds. Rather than replacing the defective factor IX, they work around the need for factor IX by making it easier for the system to produce clots.

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 anticoagulants in the system. Most of these agents work to help restore clotting in people with hemophilia A or B, with or without inhibitors, and may also work for some other bleeding disorders. These products should be very beneficial for inhibitor patients. Many of them are also designed for SC injection.

Last, but certainly not least, is 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. There is one gene therapy for hemophilia B already approved by FDA and several more on the horizon.

Gene therapy or similar treatments might one day provide an actual cure for hemophilia B. Right now, it generally brings people from severe (<1% of normal factor IX level) or moderately severe (<2%) up into the mild hemophilia range (5 – 50%). Some patients even make it up into the normal range (50 – 150%).

Mild hemophilia has its own problems, but it is generally much easier to deal with than moderate or severe hemophilia.

After our talk and a question & answer session, the group enjoyed a fun quiz and a raffle with prizes. It was nice to get together over Zoom to see people we don't get to see that often. A good time was had by all! We are so grateful for our sponsor CSL Behring for making these meetings possible!

CSL Behring

COMMENTS:

"It was great to connect with others in the community and hear directly from Dr. Clark."

"This CHB session was just what our family needed to update ourselves on what treatment options exist for our child. Dr. Clark makes the confusing into the understandable."

"Dr. Clark always teaches me something new! These meetings are so important to me and my family. Thank you for all that you do!"

"Grateful for the experience. It is great to be able to learn from the Amazing Dr. Clark!"

"Appreciate this educational session. I gained so much new knowledge!! Thank you!"

"Thank you for bringing all of the new, exciting updates to our community!"

"This session was really helpful. I enjoyed learning about the landscape of what's to come for hemophilia B."

"I loved the session and seeing everyone on Zoom was awesome! GRACIAS!"

"The presentation was so thorough and useful and shared in a way that is so understandable. I feel empowered and more educated than I did before this meeting. Thank you!"

GAME NIGHT EXTRAVAGANZA: A MULTILINGUAL CELEBRATION OF FUN AND LEARNING!

BY KATIE COLÓN

At the Coalition, we're still brimming with excitement for the new year and are thrilled to revisit our first event series of 2024: *Virtual Game Night!*

This series marked our inaugural venture into hosting events in Spanish. Our January 18th session was conducted entirely in Spanish, providing an enriching experience for our Spanish-speaking community members. This multilingual approach allowed us to reach a broader audience and create an inclusive space. Our English Virtual Game Night took place January 11th.

Each night was an unforgettable event filled with laughter, connections, and valuable insights. These gatherings were a testament to the power of games in fostering both fun and education within our community.

The events kicked off with a warm welcome to all participants, setting the tone for a night of camaraderie and shared joy. Our icebreaker questions provided the perfect opportunity for attendees to connect on a personal level, sharing their New Year's resolutions, discussing their origins, and even commenting on the local weather. These simple yet engaging prompts facilitated the creation of bonds within our diverse community.

Next up were the Kahoot Games. The games were tailored to cater to participants of all ages, creating an environment where families and individuals alike could come together for a night of shared enjoyment. The diverse range of activities ensured that everyone could find something that resonated with them, be it the thrill of a Kahoot challenge or the excitement of winning a raffle prize.

This segment was a unique blend of entertainment and learning, featuring games focused on hemophilia and various communities. Participants were not only testing their trivia skills but also expanding their knowledge on hemophilia B.

Adding an extra layer of excitement, the raffle segment had attendees on the edge of their seats. The raffle wasn't just about winning; it was a moment to celebrate and appreciate the sense of community that made the event truly special.

Game Night Extravaganza was more than just a series of games—it was a celebration of unity, diversity, and the shared joy of learning together. The success of this Game Night served as a testament to the enthusiasm and engagement of our community, and we are eager to build on this momentum.

Dana's Living Her Life with No Regrets!

BY SHELLY FISHER

When we visited, Dana had just celebrated her 35th birthday and 3rd wedding anniversary with her husband and toddler in California at Disneyland before heading to London to see her sister and niece. Hands down, her son's favorite parts of Disneyland were the rides, the train, and being introduced to Mickey Mouse. A highlight of her trip to London included taking a picture at Big Ben with her son, Benjamin, whose initials also just happen to spell B-I-G. "There's a song about the clock that we always sing to Ben, so that was super fun!"

After participating in Education Day at her local hemophilia chapter in Colorado this year, she was excited to meet new people in the Colorado community and search for volunteer opportunities after moving back to Denver from Virginia. "I'm still finding my way around right now, but I am looking forward to doing more."

Working from home for a financial advisory firm, Dana told me she splits her role between manager of client services and business development,and enjoys being a "jack of all trades." She shared that she spends her time in various pursuits from setting up accounts to compliance, and "likes that every day is different." Dana told me that spending time with her family is one of the most important things to her, and they get outside as much as possible by taking Captain Maverick Velociraptor, a Beagle mix otherwise known as "Mavie," to all of Denver's dog parks, kayaking, biking, swimming, and "wherever we can enjoy our time outdoors and spend time with our family."

One of two siblings, she confided that even though she had experienced bleeding symptoms throughout her life, her brother was the only one initially diagnosed with hemophilia B. The issues she experienced were normal for the women in her family and she "just dealt with them." Since women were not typically diagnosed with bleeding disorders at that time, it never occurred to Dana that hemophilia might be the cause of her symptoms.

After a medical procedure at the age of 23 resulted in an uncontrollable bleeding episode, it was her brother who advised her to get tested, and as a result, she was diagnosed with mild hemophilia B. Dana soon began the search for doctors capable of managing the symptoms

she had been experiencing her entire life, and found, to her dismay, that many doctors still did not believe that women had bleeding disorders.

It was only after finding the Colorado Hemophilia and Thrombosis Center that she discovered a medical staff familiar with women and bleeding disorders. Later, when Dana became pregnant, she was able to have a natural birth with no major complications after careful preparation and collaboration with the doctors there prior to the birth of her son.

When asked what advice she would give someone who has been newly diagnosed, she offered, “Find a female friend in the community. It’s helpful to have someone who is going through the same thing, or someone you can confide in and get feedback from.”

Dana also said, “Having family and friends in the bleeding disorders community to talk to has made all the difference. My mom taught my brother to be a great advocate for himself and he taught me the same thing.” She credits her brother for recognizing her symptoms and encouraging her to get diagnosed. “Thank God my brother was there for me. I don’t know where I would have been without him.”

What would her friends say is her superpower? “I have empathy. I try to understand how people deal with what they are dealing with, and I try to relate to that.”

Dana confided that she lives by a simple rule: “You only have one life. Don’t have any regrets.”

VOICE

WE'RE ALL IN THIS TOGETHER

Advocacy is the way this community expresses our “B Voice” in asking our elected officials and other leaders for the things we need to live happy, healthy lives. There are a number of ways you can get involved and express YOUR B voice! Our B Voice advocacy blog tracks developments related to the Affordable Care Act and speaks out on issues like the Children's Health Insurance Program (CHIP) and charitable premium assistance. You can also register as an advocate and join our advocacy efforts.

LEARN MORE: HEMOB.ORG/B-VOICE-ADVOCACY

ADVOCACY NEWS

TURNING A NO TO A YES — THE ART OF ADVOCACY

BY JAMES ROMANO

When we were young, we all developed strategies on how to convince our parents to turn "No" to "Yes" for something we dearly wanted: a later curfew, going to a party, or something we could not live without. As time goes on, the desire to go from "No" to "Yes" does not change, however our needs become larger. The situation becomes more serious especially for those living with a rare or chronic disease. Denial of treatments or services from health insurance providers is a frequent practice in the rare and chronic disease community. The key is to determine what motivation the decisionmaker needs to progress from "No" to "Yes." When dealing with an insurance company or a pharmacy benefit manager (PBM), we need to advocate for our own best interest. However, the insurance company is counting on you and patients like you to take "No" for an answer.

This article will provide a number of recommendations to pursue to get to "Yes." Those recommendations include:

- Document all conversations you have with an Insurance Provider of PBM.
- Document all coverage and access issues with your patient advocacy organization.
- Go through the Health Insurance Appeals Process.
- Appeal to the monetary self-interest of the insurance provider.
- Contact patient assistance organizations.
- Contact your elected representatives.

The first recommendation is to document all conversations you have with the health insurance provider or PBM. The patient or caregiver needs to document names, dates, and phone numbers. The patient should obtain the name of the person they are dealing with and note if they are with the insurance company or PBM. If the staff person does not provide a last name, please ask for an identification number. Plenty of times I have spoken with patients who tell me what they said to the company and relay the conversation. However, if you cannot provide details about who you spoke with, then it is as if the call never happened- it becomes a situation of he said/she said. Many insurance providers record the calls for quality assurance reasons and if you can share details, the company will go back and listen to the recording.

The second recommendation is to discuss the situation with your patient advocacy community. If you are denied a treatment or a procedure, the patient should immediately contact the patient advocacy organizations in their disease state to document the problem. If you are a member of the bleeding disorders community, you should contact The Coalition for Hemophilia B, the National Bleeding Disorders Foundation (NBDF), or the Hemophilia Federation of America (HFA). These organizations work closely together on coverage and treatment access issues and will want to work with you to document your situation to see if it fits with other trends the organizations are following.

The third recommendation is following through with the appeal. An appeal is applying to a higher level at the insurance provider for a reversal of the denial. Every insurance company has an appeals process. The Affordable Care Act (Public Law 111-148) guarantees an insurance appeals process. As indicated before, health insurance providers are counting on individuals not requesting nor filing an appeal. According to multiple sources, between 49% to 53% of health insurance denials are overturned on appeal. This is a process, and individuals need to work with your physician, treatment center staff, and patient advocacy group to accurately provide the information included on the appeals form.

The fourth recommendation to get to "Yes" is to appeal

to their self-interest. Patients should appeal to the self-interest of the health insurance provider. Health insurance providers are for-profit companies, the number one goal of these companies is to maximize profits while reducing claims. However, if by denying your claim or denying your treatment or procedure in the short term, the insurance provider is increasing costs in the long term- that is a persuasive argument to get to "Yes." The patient must provide the data to prove that hypothesis with information from the physician or the treatment centers. Data collection is very important to proving your claim.

To influence insurance providers in your state, influence the insurance regulators, such as the Insurance Commissioner. In certain states, insurance commissioners are appointed by the governor, in other states they are elected. Contacting your patient advocacy organization can assist with this process in reporting an issue you are having with your health insurance provider. Recently, the Coalition for Hemophilia B assisted a broader effort launched by a state chapter where an insurance company cut off negotiations with the provider network with the result that the hemophilia treatment center was no longer consider in-network. CHB signed a letter of support for patients being sent to the Department of Insurance discussing how patients were not receiving the care that they need. Patients can file complaints with the Department of Insurance in their state, however accurate information is essential.

The fifth recommendation is contacting patient assistance organizations or other health care attorneys. Patients can contact different patient assistance organizations in the bleeding disorders space or health care legal team. The Advocating for Chronic Conditions, Entitlements and Social Services (ACCESS) Program at Accessia Health could be an avenue for patients to receive advice regarding next steps to health insurance denials. The program also assists bleeding disorder patients in obtaining free disability legal aid program. Legal Assistance | Accessia Health.

The final recommendation is contacting your elected representative, if these other avenues are closed to you, I recommend meeting with your congressional office. Each representative has a local office with caseworkers to help constituents with issues they are facing. Individuals can make an appointment with a caseworker and meet with them to discuss the denial or the problem they are facing. A letter from a congressional office can be of great assistance in these matters. They can also provide advice for the next steps. I know a patient who worked on the personal staff of a Governor of a large state. The insurance provider would not cover his treatment for his disorder. The Governor sent a letter, and the insurance company reversed their position. A letter of concern from a policymaker can make all the difference.

In conclusion, document all dealings with your insurance provider or PBM. Contact your patient advocacy organizations to explain the situation and obtain their advice on moving forward. Work to file an appeal with the help of your physician or treatment center staff. The Coalition for Hemophilia B stands ready to work with you to become the best advocate for you and your family.

NORD PUSHES FOR RARE DISEASE ADVISORY COUNCILS AT THE STATE LEVEL

BY GLENN MONES

The National Organization for Rare Disorders (NORD) has made significant headway in their efforts to encourage the establishment of Rare Disease Advisory Councils (RDAC) around the country. RDACs are state-level advisory bodies that provide a platform for the rare disease community to have a stronger voice in state government. From providing information on the diagnostic journey, to making recommendations on state policies that improve access to affordable health care coverage and treatment, these councils serve as a tremendous opportunity for lawmakers and other decision makers to better understand the needs of their constituents with rare diseases. There are 27 RDACs nationwide, largely concentrated on the east coast, but NORD's Policy team is working to establish an RDAC in every state. To learn whether your state already has an RDAC or to join a coalition working to create one in your state, please visit NORD online. This information can be found at the following webpage. https://rarediseases.org/rare-disease-advisory-councils/map/

PHARMACY BENEFIT MANAGERS: THE SQUEEZE IS ON THE HEALTH CARE MIDDLEMEN

BY SHELBY SMOAK, PhD

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If you've been watching the news, you have likely heard some chatter about Congress investigating Pharmacy Benefit Managers (PBMs) and creating legislative bills to address concerns with PBMs. You may have also seen a frequent anti-PBM commercial where a patient tries to pick up her doctor–approved medication at a pharmacy. A man jumps in and takes it away, saying she can't have that particular medication; that she will have to use another one. She then replies, "But you're not my doctor." But, he is her PBM…

What are PBMs? What do they do? And why is Congress investigating them? Let's get started.

What is a PBM?

A pharmacy benefit manager, or PBM for short, is a third-party partner with a health insurance plan that provides prescription drug benefits to the plan members. PBMs function as an intermediary between the entities involved in getting prescription drugs from the manufacturer to the patient's individual insurance plans.

PBMs create drug formularies, establish drug inclusions and exclusions, and institute specialty drug-cost tiers; they negotiate drug rebates, create pharmacy networks, and handle the claim billing, processing, and payment for drug benefits.

When did PBMs start?

PBMs began operating in the 1960s when they helped insurers control drug spending by using formularies and administering drug claims. Their role expanded slightly in the 1970s and 1980s. It wasn't until the 1990s that PBMs began to change and expand more broadly. Initially, they were acquired by pharmaceutical companies, but the Federal Trade Commission (FTC) ended the practice, citing concerns over conflicts of interest.

Today, there are over 66 PBMs; however, the top three control almost 89% of the market. Many PBMs are also owned by an insurance provider, a position that echoes the FTC's concerns expressed several decades ago.[i]

What does a PBM do?

PBMs work in the background on prescription drugs. They play no role in the physical distribution of drugs, but handle payment and rebate negotiations between the manufacturers, wholesalers, pharmacies, and insurance plans.[i]

Do I have a PBM?

Yes. If you have a health insurance plan, you have a PBM. In commercial plans, the PBM may be more controlling and restrictive, but even government plans like Medicare use a third-party PBM to help manage prescription drugs for their members.

Is my bleeding disorder medication impacted by a PBM?

Maybe. Historically, PBMs have not been involved in injectables like bleeding disorder medications because these drugs fell almost exclusively on the medical benefit side of insurance benefits and were thus immune from pharmacy claims. But recently, coverage for products to treat bleeding disorders has fallen under prescription drugs, and thus PBMs.

With that, PBMs have made inclusions and exclusions of certain hemophilia and VWD products on their drug formulary. If a VWD, hemophilia A or hemophilia B product is excluded by your PBM, they may not pay for that particular product and will likely intend for you to use another medication.

This means if you are a patient subscriber and your PBM does not support the bleeding disorder medication you are currently using or intend to start using, it may not be covered. Check with your health insurance plan and PBM for more information.

What about copay accumulator adjusters and copay maximizers? Are those part of PBMs?

Yes! Copay accumulator adjusters and copay maximizers are operated by PBMs. When the PBM institutes a copay accumulator, the money paid by manufacturer copay cards for your infusion brand product does not count towards your deductible and out-of-pocket but is instead absorbed by the PBM. You are still responsible for those out-of-pocket cost-sharing amounts.

PBM INVESTIGATION AND THE LAW

Why is Congress investigating PBMs?

To answer that question, you must first understand how PBMs make their money.

How do PBMs make their money?

PBMs earn their money in three primary ways:[i]

1. Through an administrative fee for their services
2. Through "spread pricing," which is the difference between what is paid to pharmacies and the negotiated payment to health plans.
3. Through shared savings, which is the amount a PBM keeps from a discounted or rebated price from drug manufacturers.

PBMs also earn income by driving patients to in-house pharmacies or PBM-owned specialty pharmacies.

So then, why is Congress investigating PBMs?

According to testimony by a senior economics fellow, Karen Van Nuys, PhD, provided to the Senate Finance Committee this year, evidence shows that PBMs "leverage their position to extract profits in ways that are detrimental to patients, payers, and the drug innovation system more broadly."

In other words, PBMs negotiate drug prices that are often more favorable (profitable) to themselves than more affordable for patients, and PBMs take money from the healthcare ecosystem that might be better served in drug development. Van Nuys also claims that PBMs increase drug costs and, as example, states that Medicare pays almost 21% more for the same drugs that can be purchased at Costco.[ii]

Going back to how PBMs earn money, some economists argue that the structure inherently favors PBMs seeking a higher drug price from a manufacturer because they could then increase the rebate amount and thus increase the PBM's earnings. Furthermore, as another testifier to Congress put it, "PBMs force manufacturers to raise their list price, in exchange for formulary placement."[iii]

Remember, PBMs include and exclude drugs from their formularies. If a PBM excludes a manufacturer's drug, not only will patients lose access to that drug, but manufacturers would, necessarily, lose revenue from those potential drug sales.

Congress is investigating the truth of these matters and trying to answer the question: Do PBMs increase drug costs?

Are there any proposed legislative bills to address the PBM issue?

Yes, there are several proposed bills in Congress and the Senate. Here are a few:

HELP Copays Act (H.R. 830|S. 1375):
"Help Ensure Lower Patient Copays Act"

Seeks to require any third-party payment, financial assistance, product discounts or vouchers, copay assistance or other reductions to apply to a patient's out-of-pocket expenses.[iv, v]

Patients Before Middlemen (PBM) Act: (S. 1967)

Seeks to prohibit PBM compensation based on the price of the drug as a condition of entering into a contract with Medicare Part D plan.[vi]

Strengthening Pharmacy Access for Seniors Act (S. 2405):

Seeks to increase transparency measures on PBMs and provide seniors with additional options to fill their prescriptions. The bill would end limitations or restrictions that PBMs often place on certain medications without the PBM clarifying its reasoning.[vii]

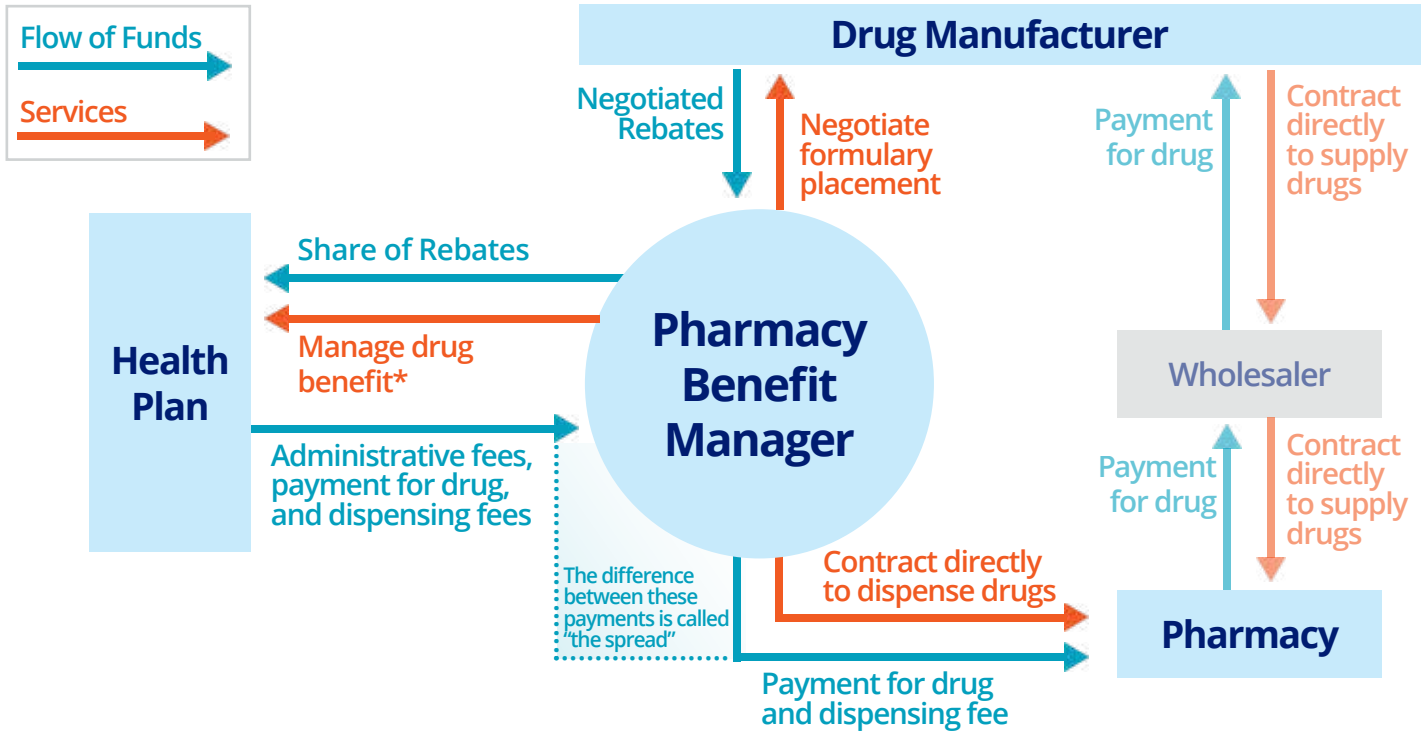
Pharmacy Benefit Manager Transparency Act of 2023 (H.R. 2816|S.127):

These bills would make "spread pricing" (charging the plan a different amount than the PBM reimburses the pharmacy) illegal and would end "clawbacking" reimbursement payments made to pharmacies.[viii, ix] Pharmacies do not "pay" the PBM, the PBM "clawsback" or deducts paid claim funds directly from the pharmacy's bank account often without notice and/or with questionable reasoning, often weeks or months later.

Health Care Price Transparency Act (H.R. 4822|S. 1130):

Aims to give transparency and access to pricing information on prescription drugs. Insurance plans must publish the in- and out-of-network charges for covered items and services and the negotiated prices for covered prescription drugs. Plans must provide a tool for consumers to search for this cost information.[x, xi]

ROLE OF A PHARMACY BENEFIT MANAGER IN PROVIDING SERVICES AND FLOW OF FUNDS FOR PRESCRIPTION DRUGS



* Includes establishing formulary and patient adherence programs and implementing utilization management tools - such as prior authorization, step therapy, and tiering - to steer patients toward certain drugs on formulary.

Source: Elizabeth Seeley and Aaron S. Kesselheim, *Pharmacy Benefit Managers: Practices, Controversies, and What Lies Ahead* (Commonwealth Fund, Mar. 2019). http://doi.org/10.26099/n60j-0886

Hidden Fee Disclosure Act (H.R. 4508):

Requires that providers give patients transparent cost data from price comparisons before providing treatment. Aims to bolster requirements for PBMs to disclose compensation to plan sponsors and other fiduciaries.[xii]

What's going on in Florida regarding PBMs?

So glad you asked! Florida is the first state to pass legislation restricting PBM operations within the state. The bill increases oversight of PBM operations and stops a PBM from requiring patients to use an in-house or mail-order pharmacy also owned by the PBM. Its intent is to return business to local pharmacies.[xiii]

How is this going to affect me and my medication?

For the time being, nothing will change. Florida residents on state plans may see some differences next year through fewer restrictions and increased pharmacy choices for their prescriptions. The Congressional bills are yet in the future but can result in positive outcomes for patients should they gain support and become law.

What about the Federal Trade Commission (FTC)?

The Federal Trade Commission is also investigating PBMs for anti-competitive practices, the same issue that caused them to intervene in the 1990s with pharmaceutical manufacturers and their own PBMs. The six largest PBMs have been issued "compulsory orders." These require those PBMs to provide information and records of their business practices.

The FTC has indicated it is seeking information on leveraging fees and clawbacks to non-affiliated

pharmacies; steering patients to affiliated pharmacies; using opaque reimbursement methods; and negotiating rebates with pharma manufacturers that impact patient drug cost.

The FTC has also withdrawn its past letters of advocacy for PBMs and has deemed their past conclusions unreliable at this point. Basically, the FTC is saying that what they determined ten or more years ago about PBMs is no longer valid; they want to re-investigate PBMs and draw a more current conclusion regarding anti-competitive behavior.[xiv]

What can I do?

If you want to learn more about PBMs, reach out to the BioMatrix Education Team at education@biomatrixsprx.com. You can also contact your Congressional representatives in support.

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LET'S PLAY NINE: 2022 AWARDEE UPDATE

BY ROCKY WILLIAMS

Among the after-school activities that shape the lives of teens and preteens with hemophilia B, few things mix skill, precision, and friendship quite like golf. Beyond the well-kept greens and the satisfying thud of hitting a ball just right, golf allows you to express yourself while learning important life skills.

The Coalition provides the Let's Play Nine Golf Scholarship. It is uniquely designed for young golf fans living with hemophilia B, ages 7 to 19. This scholarship, offered by CHB, can be used for golf lessons or for clubs. It's not just about acknowledging the skills of these young golfers; it's also about inspiring those keen on discovering the joys of golf. Let's take a look at our 2022 scholarship recipients and their latest achievements!

In Zander's world, it's not just about golf; it's about flipping the script on hemophilia challenges. Zander loves to play golf with his family, particularly when he can beat his dad (hah!). He has been golfing for six years or so, and he outgrew his clubs. With the scholarship, Zander now has new clubs and continues to get better at golf and is living a fuller life. "Just because you have hemophilia doesn't prevent you from doing sports," said Zander. "Or what you enjoy, you might have barriers, but you can overcome them. I'm blessed to have parents to be my supporters and voice for me."

With every swing, Zander's story is a reminder of the spirit that keeps the love for the game alive. But, he's not the only one! Montana's gratitude for the golf scholarship spills out of him. "The sport of golf gives people with differing physical abilities the opportunity to participate and live an active lifestyle," Montana said.

Montana has long had an appreciation for sports and has found that he has quite the knack for golf. Having hemophilia has not stopped Montana from pursuing his dreams. He learned to self-infuse at the age of six in large part because his closest treatment center was 1,200 miles away. He fell in love with sports at a young age too! He particularly appreciates the golf scholarship as he transitions from high school to college sports. "The golf scholarship has benefited my golf game in allowing me to improve on my skill, especially because I now have golf clubs that fit my height," Montana said.

Allie also shares her story. "Golf means a lot to me." Let's dive into why – it's not just about hitting balls into holes. She says, "I can golf and show people that you can do things with hemophilia. You don't have to miss out on fun activities. You can get good exercise and still be safe."

Golf brings her a sense of pride and accomplishment. With school competitions on the horizon, having her own set of clubs is like unlocking a treasure trove of practice sessions. According to Allie, "Winning the golf scholarship was so exciting. I am too young to start competing at school, but having a set of clubs of my own allows me to get a lot of practice."

Most importantly for Allie, golf is family time for her. "My favorite thing is that my mom likes to golf. We can have Allie and Mom time to catch up while we are out golfing and enjoying spending time with each other. I also could pass on some of those skills to my siblings or other people and show them how fun golf is."

As we celebrate these remarkable stories, we extend our heartfelt gratitude to our sponsors: CSL BEHRING, MEDEXUS, NOVO NORDISK, CVS HEALTH, and THE WINGMAN FOUNDATION. Their support echoes beyond the greens and affirms that the spirit of golf knows no bounds!

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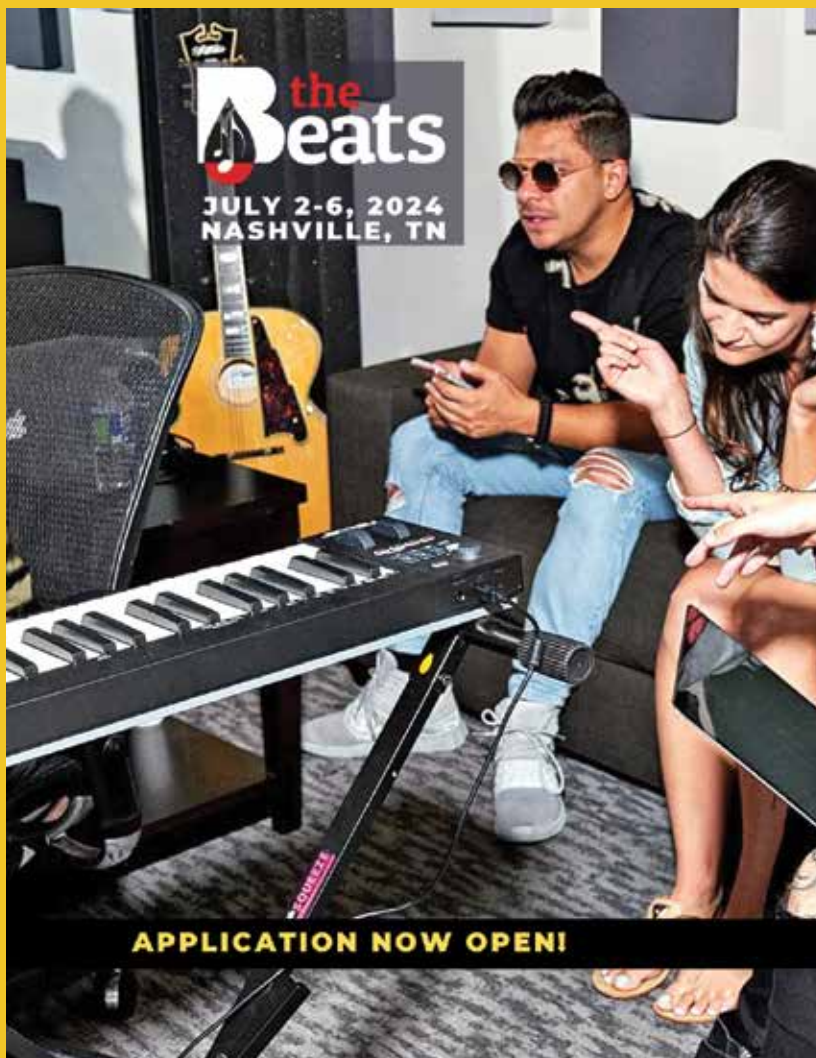

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SUPERHEROES OF HEMOPHILIA: A VIRTUAL TEEN EVENT TO REMEMBER

BY JC

Last December, I had the incredible opportunity to co-host a virtual teen event with CHB, and let me tell you, it was nothing short of stellar. Partnering with Kaley, we put on a really outstanding event that I am sure left a lasting impact.

As the virtual room filled up with participants, the excitement was amazing. We kicked off the event with a warm welcome to everyone who joined in. It was great to see so many of us come together from all across the country – to share stories, have fun, connect, and learn from one another.

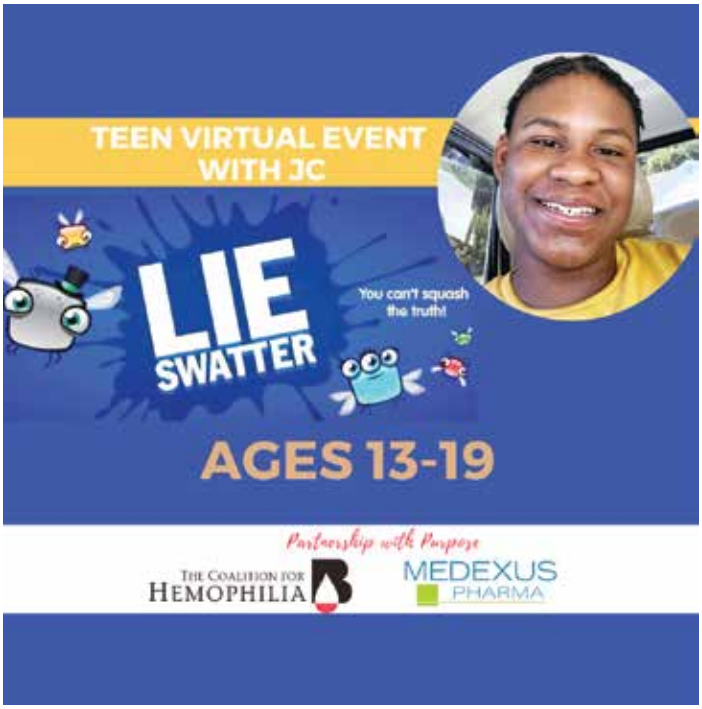
I took the lead breaking the ice by sharing my own aspirations for the semester ahead – aiming for straight As. My strategy involves a laser focus on my studies and dedicating time to practice areas that are not as easy for me. Little did I know that this was just the beginning of all of the stories that we would share with each other.

Participants chimed in, sharing their personal journeys and the innovative ways they tackled their goals. From daily musical instrument practice to master tricky notes, to strategic video game sessions aimed at improvement, the room was buzzing with enthusiasm. It was super cool! It was also a reminder that everyone has their unique struggles and victories, and together, we share and inspire resilience.

As we turned to our games, we learned a ton of trivia with Are You Smarter Than Your Hemophilia B. We learned about both hemophilia and about advocacy. We learned that Queen Victoria was a carrier of hemophilia B, that the first factor IX product licensed in the US was in the 1960s, and we even learned what CBER stands for- the Center for Biologics Evaluation and Research!

We also played some Kahoot! and Lie Swatter on Jackbox. Lie Swatter is a game where we share outlandish facts and players guess whether each fact is actually true or just made up. It's basically a gigantic game of Two Truths and a Lie, only way more fun. It was neat to see what everyone came up with.

Hosting the event turned out to be not only fun but an invaluable learning experience for me. Confidence in public speaking, connecting with people of different ages, and engaging effectively during a presentation,



these were skills I worked on throughout the event.

I want to express my gratitude to Medexus for providing me with the opportunity to co-host this event. It was more than just an event; it was a gathering of superheroes – individuals facing challenges head-on and emerging victorious. Together, we celebrated our uniqueness and strengthened the bonds that make our community truly exceptional.

Here's to more events that inspire, connect, and remind us that we are all superheroes in our own right.

MEDEXUS PHARMA

COMMENTS:

"The best part was getting to know more about each other and strengthening our bond."

"I always look forward to these events. Thank you for giving us this space to all come together."

IZZY IS FINDING HER VOICE BOTH ON AND OFF THE STAGE!

BY SHELLY FISHER

In between practices and performances with her alternative rock band, Izzy took some time to talk about her passion for music, her love of science, and the recent diagnosis that surprised her family and friends. This junior is definitely finding her voice both on and off stage, and she has big plans to use her gifts to advocate for other teens.

A first-time participant at The Beats Program in Nashville, TN, this young vocalist enjoyed collaborating with other artists and was most excited about singing a duet for one of the performances. In addition to singing, Izzy also writes songs, plays piano, guitar and the ukulele, and since last June, has been the lead singer for Total Luminosity, an alternative rock band that was formed due to her participation in The Bach to Rock program at Virginia Beach. Managed by the owner of the program, her band has already played at notable venues such as the Bunker Brew Pub, so The Beats Program was the perfect event for Izzy in many ways!

When our conversation turned to her school year, it was no surprise that the 16-year-old had earned spots in two advanced choirs – the Madrigals Choir and the Salem Select Choir, and had the opportunity to sing acapella on several occasions. Izzy seemed equally excited to talk about her volunteer work with the beginning choir and summer music camps where she taught choir and ukulele. “I love to teach music and see the outcome it has on people. Music has always been my thing that I really love teaching people. It just feels good to know that you helped somebody.”

In addition to helping with the beginning choir, she has a penchant for succulents and participates in the gardening club, is a photographer on the yearbook staff, and has a particular interest in science. After taking a high school science course in the eighth grade just so she could pick an additional science in high school, she was looking forward to an advanced class. “I’m excited that I get to take chemistry next semester.”

In college, Izzy hopes to major in music with a focus on vocal performance and would like to be a vocal instructor some day. “When words fail, music speaks. I have a genuine belief that music can change lives. All of the choir teachers I have had have made me feel that I want to be one as well because they inspired me to do better and help

other people."

When asked how her friends might describe her, she giggled. "They would probably describe me as goofy. I am introverted, but also outgoing. I turn into a whole different person on stage."

After being diagnosed with hemophilila B at 14 years of age, Izzy began attend events in the hemophilia community. She shares, "The Coalition for Hemophilia B has been invaluable and even though there are other teens with hemophilia, it's easy to forget that you're not the only one." She also says that her experience in the hemophilia community and particularly in The Beats program has been really great. Izzy "feels more connected with other individuals living with hemophilia B" and that the Coalition has truly provided support every step of the way.

A strong proponent for advocacy, "Advocating for yourself and others is so important. I'm always trying to advocate for myself, like when I go to my 6-month appointments, I know what I am going to say and what I am going to ask about, and I know how to answer the questions that are asked of me. I always try to stay as educated as I possibly can. One thing I really want to do more of is advocate for women and teens who have hemophilia, especially female teens. At these events, sometimes I feel like I don't hear anything about teen girls." When asked if she would like to speak about this topic at an event, she quickly answered, "I would love to do that!"

When asked if there was anyone who had been particularly supportive after she received her diagnosis, the answer came without hesitation. "I definitely would say my brother. He was very shocked when I told him. I came home from the doctor's and I was like, you will not believe what I just got diagnosed with."

Even before she was diagnosed, she credits her brother, Brandon, with helping her understand the disorder itself. Having been diagnosed at birth, he was no stranger to hemophilia B and knew exactly what she needed to hear and how to help her manage her treatments.

What's her advice for someone newly diagnosed with hemophilia B? "You're not alone. Even though it feels really hard right now, the more you educate yourself, the easier it will become. It's important to advocate for yourself so you can speak on your own behalf."

The lead singer for Total Luminosity is just getting started with her own voice and we look forward to hearing a lot more from her!

ANDREW'S GOT THE HACK TO LIFE— PURSUE YOUR GOALS, STAY ACTIVE, AND KEEP YOUR FRIENDS CLOSE!

BY SHELLY FISHER

A junior at Northwestern State University in Louisiana, Andrew was anticipating a full course load in computer science for the spring semester when we spoke. This avid fan of technology and jack-of-all-trades sports fanatic talked about his degree, staying active, and the importance of friends. With a year to go before graduation, he seemed to have the perfect hack for life.

With an interest in computers that began in high school, Andrew was excited to share that he had recently disassembled and reassembled an entire computer and was looking forward to upgrading it for school and video games. He was particularly looking forward to his web development class because he felt it would be "different and challenging," however, it was clear that his true passion was computer security and virus protection. He described having to "pick a lock" on a computer for a group presentation to demonstrate how hacking occurs in order to prevent it. Already anticipating courses for his senior year, he told me his objective was to "secure a stable job" when he graduates.

When he's not contemplating the world of information technology, the computer science major told me that he enjoys spending time with friends, and he felt they might describe him as "a good basketball player who always goes for the 3-pointer," and "the friend who always stands by them." In addition to basketball, he enjoys playing a variety of recreational sports including golf, soccer, and tennis, but with his best score of 48 on 9 holes at the nearby Northwestern Hills Golf Course, golf appeared to be his favorite.

"I started playing golf about nine years ago after my

grandmother got me into it, and I played my first tournament when I was 13." When asked if he played it safe on the golf course, or went straight for the green, he shared, "I try to take the short cuts and if there isn't one, I make one!"

Andrew also told me that he likes to read when he has the time and does his best to keep up with the latest edition of Time or People. He listed The Hunger Games trilogy as his favorite books, but, spoiler alert, this sci-fi fan did not recommend the movie. "I wasn't really impressed with it." Also, a fan of classical literature, he told me he finds the line, "fair is foul, and foul is fair" interesting, and counts MacBeth as his favorite work by William Shakespeare.

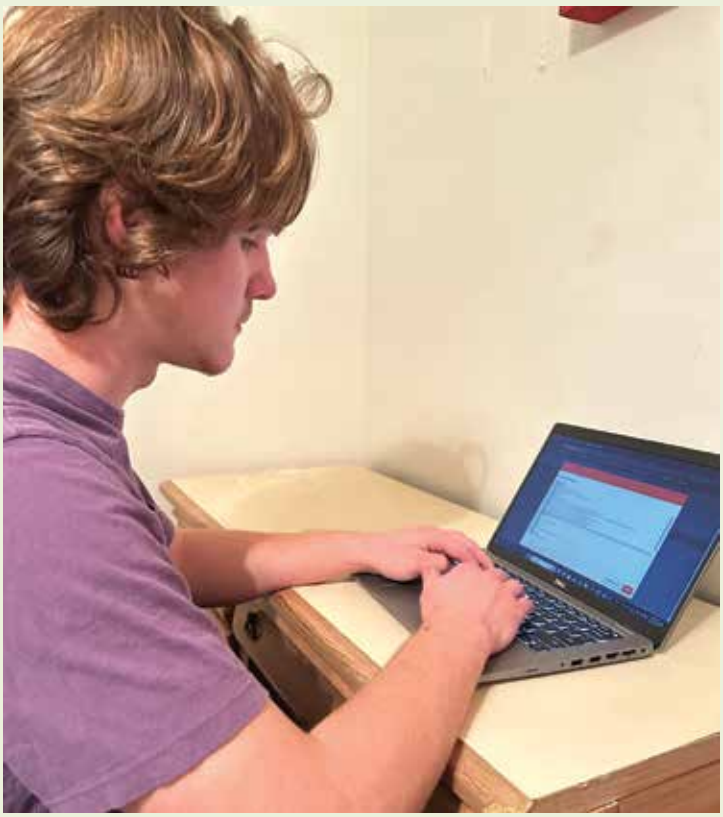
When our conversation turned to his diagnosis, he shared that after an injury left a bruise on his head that wouldn't heal and had to be removed surgically, his doctor discovered he had hemophilia at the age of 14. Andrew confided, "It was kind of a rough time," and required him to "do stuff carefully" moving forward. Without any family history and subsequent negative test results for his mother and younger brother, it was determined that his diagnosis was the result of genetic mutation.

After his family physician retired, Andrew began seeing a hematologist for on-demand treatment for his mild hemophilia, but it was his mother who was his biggest support after his diagnosis. "My mother helps me out and is supportive. She studied and learned about hemophilia. We had never even heard of it." They both attended their local bleeding disorders chapter's events

in Alexandria and Andrew recalls being particularly interested in gene therapy.

Today, Andrew is grateful that he continues to meet new people and have new experiences with his hemophilia B family. "The Coalition for Hemophilia B changed me as it's good to know that I'm not alone in the world with hemophilia. It's helpful to know how others handle life with a bleeding disorder and makes me confident to handle the world ahead of me with hemophilia B."

When asked what advice he would give to someone who had just been diagnosed, he offered that people with hemophilia can still do what they want, they just have to know what they can handle and to be careful. "Make direct choices, make sure you're careful, and don't make choices that are dangerous." Sounds like the perfect life hack to me!



B inspired!

Stories and artwork from teens in the Hemophilia B Community

WINTER 2023

IN THIS ISSUE:

IZZY IS FINDING HER VOICE!

ANDREW'S GOT THE HACK TO LIFE!

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!