THE COALITION FOR HEMOPHILIA B

FALL 2024

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

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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.

THE COALITION FOR HEMOPHILIA B HONORS DR. MINA NGUYEN-DRIVER AND DR. DANIELLE NANCE WITH THE ETERNAL SPIRIT AWARD

BY ERICA GARBER

Just sit right back, and you'll hear a tale...a tale of two extraordinary women being honored by the Coalition for Hemophilia B with the celebrated Eternal Spirit Award. On August 8, 2024, members and friends of the Coalition for Hemophilia B gathered at the Crest Hollow Country Club for a Gilligan's Island-themed awards gala. The night was filled with fun, music, and heartfelt moments. We honored Dr. Danielle Nance and Dr. Mina Nguyen-Driver with the Eternal Spirit Award, recognizing their dedication and contributions to the hemophilia B community.

The evening began with a lively cocktail hour, featuring live ukulele music by Phoebe Zara and delicious hors d'oeuvres, setting the stage for a night of fun and celebration. Renae Baker, our Master of Ceremonies, then officially welcomed everyone, leading the evening with warmth and enthusiasm. Her engaging presence guided the event, setting a perfect balance between honoring the serious achievements of the award recipients and embracing the lighthearted theme of the night. Her energy set the perfect tone for the festivities to come.

Following the welcome, guests enjoyed dinner and the awards ceremony to honor our incredible recipients. Kim Phelan, our CEO, joined us via Zoom to introduce our honorees, ensuring her presence was felt even though she could not attend in person due to COVID precautions. Ashley Zebley. shared her personal journey of getting the right diagnosis, highlighting how important advocacy and support are within our community.

Renae Baker, Farrah Muratovic, and I presented the Eternal Spirit Awards to Dr. Nance and Dr. Nguyen-Driver. The awards ceremony beautifully balanced the significance of the Eternal Spirit Award with the fun of the Gilligan's Island theme, reflecting our community's ability to embrace both joy and meaningful recognition.




S.S. MINNOW
















Each honoree received the Eternal Spirit Award, a symbol of their extraordinary dedication to the bleeding disorders community, along with a special quilt made by Alma Jewel. Alma added her own creative flair to the evening by attending in a handmade headpiece featuring the boat and island from Gilligan's Island. It was an honor to recognize the compassion and contributions of Dr. Nance and Dr. Nguyen-Driver, who both perfectly embody the spirit of dedication and joy that defines our community.

Dr. Mina Nguyen-Driver, whose personal connection to the community stems from her brother Mika who had hemophilia and passed away in 2003, is a Professor in the Department of Pediatrics at the University of Arkansas for Medical Sciences. With over 20 years of experience helping patients and families with bleeding disorders, she is known for her compassion and dedication. Dr. Nguyen-Driver has held roles at Children's Hospital Los Angeles and Oregon Health & Science University, gaining significant experience in rare diseases and behavioral health. Since May 2022, she has been a professor at UAMS, continuing her commitment to the bleeding disorders community. She regularly speaks at our events, sharing insights on coping, advocating, and building supportive relationships.

As a community member, Ashley Zebley reflects on her experience with Dr. Nguyen-Driver: "I was in a cold, sterile room, feeling like I had lost trust in the healthcare system, but Mina's empathy was exactly what I needed. She listens to patients as people, not just as appointments. Her commitment is truly inspiring, and I'm proud to call her a friend."

Dr. Danielle Nance is a board-certified hematologist at Banner MD Anderson Cancer Center, specializing in bleeding disorders, particularly women's health. With over 15 years of experience, she has been a dedicated advocate, educator, and healthcare provider for the bleeding disorders community. Dr. Nance is currently working towards establishing the first Adult Hemophilia Treatment Center in Arizona. Her compassion and dedication are shaped by her personal connection to hemophilia, as both she and her son are affected by it.

Zebley shared: "Dr. Nance's kindness and passion touched me deeply. After a difficult medical experience, she offered sincere compassion and guidance. I'm traveling over 1,100 miles to see her because of the trust she has instilled in me." Her empathy and understanding extend beyond the clinic, as she spends time in nature, enjoying activities such as bird watching and exploring the desert. Her passion for life is evident, both in her work and in her personal interests, which include a love for science fiction and dreams of space exploration.

Coalition member Leisa MacDougall expressed her gratitude for both of our honorees: "I've learned from Dr. Mina at many Coalition events, and her compassion for our community is amazing. Dr. Nance is also an incredible asset. I always learn something new and feel empowered by her presentations."

Rocky Williams, a long-time member and community leader shared, "Dr. Nance and Dr. Nguyen-Driver have both left a lasting impact on our community in their work, and their educational sessions at Coalition events have provided not just information, but real hope and inspiration for so many of us."

After honoring the Eternal Spirit Awardees, we moved on to recognize the achievements of our William Drohan Scholarship Awardees. Craig Drohan represented the Drohan family and presented the scholarship awardees, praising the honorees for their dedication to the bleeding disorders community. Each recipient demonstrated incredible dedication and passion for making a difference:

- Logan B. is currently enrolled at The University of Alabama, where he is double majoring in Economics and Finance. Recognized as an Academic All-American and Vice President of the Men's Volleyball team, Logan hopes to work in pharmaceutical sales for Hemophilia B factor products to help others, just as he has been helped.
- Max G. will attend UCLA to study Business Administration. Max has shown strong dedication as an Eagle Scout and former tennis team captain. He is passionate about fitness and personal growth and aims to join a startup company after graduation.
- Matthew H. is heading to Binghamton University (SUNY) to pursue a degree in Mechanical Engineering. Matthew is a talented jazz guitarist and National Merit Scholarship Commended Student. He aspires to invent safety solutions that mitigate everyday risks, combining his engineering skills with his love for music and photography.
- Jake P. is a senior at Long Island University, studying Artificial Intelligence. Jake is committed to advocacy for hemophilia and volunteer work. He is driven by a passion for combining engineering and medical science to solve healthcare challenges and improve lives.
- James S. will attend Tyler Junior College to study


Gilligan's Island




















Radiologic Technology, with a goal of transferring to Auburn University. Inspired by his experiences as a patient, James aims to provide compassionate care as a Radiology Technician and eventually own a medical imaging business serving rural communities. His passion for swimming and his experience in choir highlight his resilience and commitment.

- Steven S. is pursuing a graduate degree in Physical Therapy at Utica University, with minors in Entrepreneurship and Aging Studies. As a dedicated baseball player, Steven dreams of becoming a physical therapist for the New York Mets, using his passion for baseball and desire to give back to the hemophilia community.

These young scholars are inspired by the role models around them and are eager to make a positive impact in their fields.

We then gave a heartfelt champagne toast to Joe Pugliese on his retirement. Joe has had an incredible 46-year career, where he made significant contributions to the bleeding disorders community. Known as a "broker of deals" and a charismatic businessman, Joe's dedication to patients has been unwavering. His daughter spoke fondly of him, highlighting how he carries himself with confidence and is always there for others, making him someone people feel fortunate to have in their corner. Joe's advocacy and leadership have earned him a respected place in the community, and his work has positively impacted countless lives. We are deeply grateful for his years of service, and his legacy will continue to inspire us.

Post-awards, the fun continued with a costume contest recognizing attendees who fully embraced the Gilligan's Island theme by dressing up as their favorite characters from the show Craig for the Captain, Matthew for Mr. Howell, Tyshawn for Gilligan, Milinda for Ginger, Debbi for Marianne.

Rocky Williams brought the theme to life by wearing a gorilla suit, creating some fun chaos throughout the event. His antics had everyone laughing, perfectly capturing the playful spirit of the night. We also had a round of Gilligan's Island trivia, which reminded everyone of why the theme was so fitting—just like the characters on the show, our community knows the value of supporting one another through every unexpected twist and turn.

Wayne Cook, our Coalition president, who remains a key figure in our community serving as a leader and friend for many years, could not attend for medical reasons. However, he was represented by a life-sized cardboard cutout, that was a source of fun photo opportunities throughout the evening, including when the "Gingers" serenaded his cutout with a rendition of "I Wanna Be Loved By You," keeping his spirit present despite his absence.

The evening wrapped up with the announcement of silent auction winners, adding a final touch of excitement to the night. The dining room buzzed with excitement as Rocky Williams, Chris Villarreal, and I entertained the crowd during the raffle with a delightful comedy routine, enhanced by Joe Baker's musical interludes, creating laughter and adding a playful spirit to the evening.

On behalf of the Coalition and the families we serve, I want to thank all our generous sponsors. Diamond sponsors, including Pete Tadros, Senior Director of Marketing at CSL Behring, and Jane Smith from US Public Affairs & Patient Advocacy at Sanofi, spoke about their unwavering dedication to our community.

I would also like to extend a special thank you to our Gold sponsors: CVS Health, Hemophilia Alliance, Novo Nordisk, and The Alliance Pharmacy. Your support helps us raise funds for our scholarships and patient assistance programs, which directly impact the lives of those in the bleeding disorders community.

We are truly grateful to everyone who joined us and contributed to the magic of the Eternal Spirit Awards Gala. Your presence and support made this evening unforgettable. We look forward to continuing this journey together and hope to see you at our future events, where we can celebrate, connect, and honor our amazing community once again.

Special Thanks & Recognition

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BUILDING BONDS AND GROWING STRONGER: REFLECTIONS ON THE 2024 FALL MEN'S RETREAT

BY MATTHEW STONE

As the year draws to a close and the summer storms settle into memory, cooler weather arrives, inviting us to pause, gather our thoughts, and prepare for the winter ahead. It's the perfect time to reflect on our 2024 Fall Men's Retreat, held September 19-22 in sunny Orlando, Florida.

We arrived in waves, filtering in from airports near and far, and were warmly welcomed at the Courtyard Marriott, our home for the weekend. We brought unique backgrounds, roles, and connections to the community, creating a vibrant tapestry of experiences. However, the shared journey united us—an opportunity to bond, laugh, and grow together, building knowledge and friendships that will carry us forward through the seasons to come.

Driven by our wonderful hosts Wayne Cook, President and longtime member of the Coalition and Rocky Williams, Community Relations Director they provided specialized discussion topics. Whether transitioning through life with hemophilia B with Wayne or Nurturing family and bonds with Rocky. Time was taken to allow the group to discuss and collaborate on how we all live our lives. The moments were easygoing and sometimes heartfelt, but every word had weight, and the group was there to hear it all and respect every word.

Moving forward it was time to get the blood flowing. Being a staple of the Men's Retreat backyard games were front and center once again allowing for a competitive spirit to flourish in the hotel's outdoor area. It didn't take long for outdoor activities to be set up and the group flooded in ready to dominate the field. Cornhole champions were crowned, and Kan Jam warriors fought for total victory. Competition was fierce with no one holding back still being kind to our joints all the same. There was nothing standing in the way of endless rounds of games. Except maybe dinner that was just about to take its hold onto all the hungry competitors.

For those tactile learners GutMonkey had you covered, coming in with a range of activities Joe Torrey, Communications Director at GutMonkey kept us on our toes. With an impromptu thumb wrestling gambit leading us into a rock, paper, scissors showdown. These games come bundled with deeper meanings, allowing us to connect and discuss the outcomes in more meaningful ways and even leading the group to have a serious debate if the rope in front of them was going

to straighten out or tie into a knot, a real brain twister.

Being such a tight-knit community, we are always there for each other. Thus, Lee Hall led us in a discussion of discovery in the power of community. Showing us the true strength of how deep relationships and support for one another empower us to fight the battles we couldn't face alone. As if that wasn't enough preparation, Donnie Akers didn't hold back any punches when giving us the legal tools for success and loading up our tool belt with tips and information on financial planning and magnifying the landscape of medical directives and benefits.

Rockstar social worker Tony Brown took center stage with an engaging open discussion on the many challenges men with hemophilia B face. He covered essential topics like pain management and maintaining a prophy schedule, while also addressing key issues for caregivers—such as fostering positive environments for affected children and navigating those early, often daunting, insurance battles. His open, conversational format encouraged us to share experiences and deepen our understanding of these crucial topics, fostering both learning and connection.

The Men's Retreat provides the older and younger generation of men with hemophilia B, the dads, and caregivers the chance to learn, share, and create long-lasting memories that stand the test of time. This community provides for each other, and it shows with the bonds and friendships we have come to see grow at these retreats. This shows with the conversations of support and guidance for the caregivers from our guys with hemophilia B. Whether new or long-time attendees, the men who gather here show us that the community is ever-growing, and bonds are ever forming.

A heartfelt thank you to our sponsor, Pfizer, for their generous support of this amazing event!

Pfizer

Comments:

"I really like the bonding everyone had , we all share different experiences, and we all brace each other with total support , very unique specially everyone comes from different places, but we get united as brothers."

I really liked the feeling of Family with all people at the event."

"I liked learning about the new forms of treatment options for hemo B's, & I REALLY appreciate & enjoyed getting the experience & perspective of the older crowd compared/contrasted to the younger crowd."

"The rap sessions were great, and I feel it was very therapeutic for a lot of the participants."

"I loved the conversations during mealtime, the movie, Donnie Akers presentation, and the pool."

"I really liked the comradery, reuniting with friends, and taking the opportunity to step outside of the box."

"I am always astonished by the stories of the previous generation. Their resilience in the face of terrible crisis, and the way they have molded that strife into a supportive community will never cease to surprise and amaze me."

"The men's retreat really provides me with the amazing opportunity to envelope myself in the hemo B community. I learn so much from the speakers and just from each other. I love the time that we have together. It is invaluable."

2024 FALL MEN'S RETREAT






















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“I’m embarrassed to

CALL IN SICK BECAUSE OF A BLEED.”

– Julian

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TFPI=tissue factor pathway inhibitor.
This patient story reflects the real-life experience of an individual diagnosed with hemophilia. Individual experiences may vary. This patient was compensated for their time creating this content.

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FOOTBALL, PHARMACY AND FIGHTING THE GOOD FIGHTS

An Interview with New York's Finest: Capt. Bill Patsakos

BY RENAE BAKER

Some people excel at getting things done, like Bill Patsakos. His accent and rapid-fire speech announce his proud Staten Island heritage, and he seems ever ready for quick and decisive action. Bill recently retired as captain of the Fire Department of New York. That's right; he is bona fide "New York's Finest!"

Bill also holds a Doctor of Pharmacy degree and for the past 23 years has worked in specialty pharmacy practicing in retail, hospital, and consulting. The last 12 years saw him working as a client relations executive for CVS Specialty and as a clinical specialist. Prior to these twin careers, Bill served in the United States Army.

Bill is an active member of The Coalition for Hemophilia B. He speaks on various health conditions and volunteers at Coalition events, but Bill does not have hemophilia.

After earning his medical degree, Bill worked in cardiology when he met his wife, Christine, a respiratory therapist. As they prepared to start a family, they knew that hemophilia B was a possible concern because Christine's father had passed from complications of the disorder.

Christine underwent genetic testing, and their queries were confirmed. It wasn't long before Bill changed his medical focus from cardiology to bleeding disorders.

Their sons, William Jr., Seth, Jake, and Aiden, have been encouraged to live physically active lives despite three of them having moderate hemophilia B. "We've been very fortunate because we've had access to medications, and our sons are not heavy bleeders," Bill says.

In fact, all four sons play contact sports, including baseball, football, basketball, and martial arts. "That's been our mission; that they would be educated about their condition and not take risks that are detrimental, but that they do not allow hemophilia to prevent them from any of their dreams."

So far, so good. William played college football and graduated. Seth and Jake are in college, and Aiden is about to attend college in the fall. "Aiden would be following William's footsteps. William, who doesn't have hemophilia, played college football for four years. Aiden is the number one receiver in New York, and he has multiple colleges recruiting him to play college football. It's a miraculous feat for someone with hemophilia, but he's a very talented football player," Bill marvels.

"Without medication and without science and without the Coalition and other organizations, that wouldn't be possible. These organizations were at the front of advocacy, political action, fundraising, working closely with pharmaceutical manufacturers to basically make hemophilia something that's manageable.

"I wanted to make a difference for my sons and other people who have this condition," Bill says. Oh, he makes a difference, all right! On top of his military, fire, and specialty pharmacy service, Bill somehow makes room to volunteer his time and talents.

He served as NYC's FEMA Red Card Medical Unit Leader in the FDNY Incident Management Team and United States Search and Rescue Task Force 1. He's responded to many hurricanes, earthquakes, and natural disasters. When Hurricane Maria devastated Puerto Rico, Bill helped locate all the hemophiliacs on the island and brought supplies to the HTC to be distributed to affected families there. He climbed on rooftops to help rebuild homes.

In April 2022, firefighters faced an unprecedented challenge: conducting search and rescue operations while under active artillery fire, a situation not seen since World War II. Then-FDNY Lieutenant Patsakos coordinated a major relief effort, collecting and shipping 60 tons of medical and war supplies to support the troops, doctors and firefighters in Ukraine.

Having played football from the time he was five to his high school career where his team won the 1990 New York City Championship, as soon as he was able, Bill started coaching his alma mater's team. "I'm still chasing that championship 30 years later. We came very close this year." He says, his determination undimmed.

Bill also volunteers at the various hemophilia organizations, including The Coalition for Hemophilia B. "I think the world of the Coalition and its staff! Kim Phelan (COO) is like a David Copperfield when it comes to resources! She's hired some really great, dedicated staff as well!"

When asked what drives his volunteerism, Bill relates, "I've been blessed. Honestly, it's very important to give back." His service in the military and FDNY helped inform his understanding of suffering in the world. "They exposed me to tremendous poverty and healthcare disparities," he reflects. This ignited a spark in Bill. "I'm pretty passionate about all people getting quality healthcare. I think a lot of it comes down to education. Someone may have financial means, someone else may not, but they have different barriers to healthcare."

Being deployed overseas during his military career and being an FDNY 9/11 responder gave Bill close-up views to trauma. "I've witnessed a lot of mental illness, post-traumatic stress, and substance abuse, which, as a country, we're not prepared to deal with. Many people are under-treated or not treated at all, because they are not first treating the mind. Those areas of trauma have opened my eyes to the shortcomings of our system." He believes the United States has the best system overall, but he sees room for improvement.

Shining a light on hemophilia-specific matters, Bill states, "One of the biggest challenges for anybody is maintaining gainful employment with an insurer that can cover you. If someone with hemophilia, or who has children with hemophilia, wants to change careers, they need to make sure there is no lapse in coverage, and that they get the same good coverage. Being a firefighter, and my wife being a respiratory therapist, we have the best coverage possible in the country, but the frightening question is always hovering, 'What if something happens to one of us? What'll happen to the kids?' As the boys have gotten older, they travel places. So, our biggest fear is not being there for possible major bleeds."

Bill contemplates what he believes should be a concern for the bleeding disorder community. "Bleeding disorder organizations provide great resources, but they rely on funding that comes from pharmaceutical manufacturers who support these groups. If gene therapy creates a situation where many people no longer need to be on these medications, then that funding may dry up, and that could change access to other resources that we've fought so hard to make available to people. We cannot forget those who are unable to have gene therapy and still need support, resources and education and those on gene therapy need support as they will always have hemophilia, and we just don't know how long it will last. The organizations are important so we must not waiver in our commitment to them"

Bill values the friendships he's made within the bleeding disorder community. "Being a member of the military and fire service and being a football coach kind of puts me in one bucket of society. Without hemophilia, I probably never would've developed the deep relationships I have with musicians, academics, and others around the country. Hemophilia bonded us together because we have a common goal."

In that spirit, Bill puts out this call to action: "I think the members of these organizations need to realize the value they've received and pay it forward. We need to contribute more of our own money, resources, and time volunteering to keep these organizations and programs going. We can also reach out to our communities and ask them to contribute to something they were not even aware of. The Coalition is always looking for volunteers so reach out to them! If we each play a small part is makes a big difference."

What's next? "It's been a long career, and I love it, but it's time to figure out a new chapter in my life. It's always been a dream of mine to do medical missions, like Doctors Without Borders in poor countries."

Bill closes our interview with a story about Seth. "He's loved football his whole life. He played Little League when he was 12. The NFL Denver Broncos were preparing for the Super Bowl with Seattle and had gotten word that there was a boy in New York that had to take an IV every week to play football. They thought that was such a commitment that someone would do that for love of the game. When they won the AFC championship, they sent the signed football to our house. They were sending us to the Super Bowl! I mean the news was outside!

They interviewed Seth, and he gave a quote that stunned me. At 12 years old, he said, "I have hemophilia, but hemophilia doesn't have me." That's now been the mantra for our family and others. I think that's something we can hold onto. I don't care if it's hemophilia, drug addiction, post-traumatic stress, cancer, or something else; we have those conditions, but those conditions don't have control over our lives."

Thank you for your service, Bill!

VOICE
WE'RE ALL IN THIS TOGETHER

ADVOCACY NEWS

ADVOCACY UPDATE

BY JAMES ROMANO

The Coalition for Hemophilia B is strengthening its relationships with larger advocacy coalitions to support the needs of the chronic disease community. Just this past week, CHB signed onto three letters addressed to the Centers for Medicare and Medicaid Services (CMS), focusing on the needs of the broader advocacy community. A common theme throughout these letters is the advancement of health equity for underserved populations.

The first letter addresses the Medicare Physician Fee Schedule (MPFS), which is the list Medicare uses to reimburse physicians and providers for services under the program. The schedule covers payment rates for over 12,600 services. The letter, drafted by Families USA—a national healthcare advocacy coalition—and endorsed by several patient groups, emphasizes the importance of creating payment codes that promote health equity and deliver high-value primary care services. It also highlights the increased financial support needed for providers participating in the Medicare Shared Savings Program (MSSP), particularly those serving under-resourced communities. Lastly, it focuses on enhancing the connection between patients and providers accountable for both the cost and quality of care delivered.

The second letter pertains to the Hospital Outpatient Prospective Payment System (OPPS), which governs payments hospitals and community health centers receive for outpatient care provided to Medicare patients. Developed by the Community First Coalition, the comments focus on two key areas: improving hospital quality measurement to ensure accountability in advancing health equity, and addressing the maternal health crisis by establishing federal quality standards for obstetric services.

The third letter also relates to the OPPS and calls for the codification of 12 months of continuous coverage for children up to 19 years old in Medicaid and CHIP, while removing state authority to disenroll children for non-payment of enrollment fees or premiums. The letter advocates for extending the Medicaid "Four Walls" exception for Indian Health Services (IHS), tribal clinics, behavioral health services, and rural providers. It also calls for the eligibility and enrollment of formerly incarcerated individuals into Medicare.

The Coalition for Hemophilia B is eager to continue partnering with national advocacy coalitions, lending our voice and support to these critical issues that aim to improve the health of all Americans.

The *New Bully* on the Insurance Playground: *Alternative Funding Programs*

BY SHELBY SMOAK, Ph.D.

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In the ever-changing health insurance landscape, policies do as they always have – they change. The 'new kids on the block' in 2024 are *Alternative Funding Programs*, or AFPs. While AFPs staked out their street corner a few years' back, their adoption by Pharmacy Benefit Managers (PBMs) has them moving into the blocks and neighborhoods next to you. In 2023, about 14% of employers were utilizing AFP-style programs.[i] Let's take a look at AFPs and see how you might encounter them in your own health insurance plan.

What are AFPs?

AFPs are ***alternative funding programs*** that aim to flip the script on how specialty medications are paid for through your insurance plan. Intended to reduce the cost of expensive specialty medications like factor products, AFPs are sold to employers, especially for self-funded plans, and offers to save those company plans money through their third-party administrative services. The first AFP to enter the market, SaveOnSP, for example, advertises on their website's home page, "SaveOnSP can help reduce the financial burden."[ii] PrudentRx, now a big hitter with the CVS PBM, says plainly on their homepage: "PrudentRx was founded to help health plans of any size manage the rising cost of specialty medications."

How does a typical AFP work?

There are several steps involved in how an AFP operates within a health plan, so let's break it down:

1. An AFP will ask the health plan PBM to remove, or "carve-out," certain high-cost specialty medications from their formularies. Pointedly, those medications are no longer covered AT ALL by the health plan. These drugs are classified by the PBM as "non-essential" and removed from any health plan coverage. This allows the plan to bypass annual limits on a patient's out-of-pocket cost and leaves the patient either "uninsured" or "under-insured" for those medications, which is a key step in the AFP design.

2. Patients on these high-cost specialty medications are then referred to the AFP by the plan. A key point here is, at this point in the process, the patient is no longer being covered by the plan for those medications. Operating as a third-party to the plan, the AFP has full and total control. The AFP completely manages the patient's access to that specific medication.

3. The AFP will manage access to the medication in the following way:

    a. They will refer the patient to a charitable organization and represent the patient as "uninsured" or "under-insured" in order to access free drugs through a manufacturer patient assistance program.

    b. The charitable organization then refers the patient to a manufacturer copay assistance program and has the patient sign up for a copay card, which is then given to the AFP.

4. The AFP then strips all the money from the copay card and pockets it.

5. The AFP then authorizes for the drug to be dispensed to the patient for zero cost, or a nominal $10 – $25 dispense fee.

6. The AFP then shares a percentage of the transaction with the plan.

7. When a copay card is used, none of the money is applied to a patient's deductible or out-of-pocket expense.

This sounds like a copay-maximizer policy. Is it?

In many ways, an AFP looks like a copay-maximizer policy. In a maximizer, the copay card dollars are used as a way for the patient to access a specialty medication, and then those dollars are not applied to a patient's deductible or out-of-pocket. So an AFP does share that effect with the maximizer. However, the key difference is that a co-pay maximizer operates within the health plan and is still legally bound by maximum out-of-pocket amounts, which in a 2024 ACA plan is $9450 for an individual. An AFP operates completely outside of a health plan and has no limits on how much money they can take from a patient, or by proxy, from a patient's manufacturer copay card.

What are some examples of AFPs?

SaveOnSP was for the longest time the best-known AFP. It primarily works with the Express Scripts PBM. PrudentRx has risen to the top of the pack in 2024, largely as a result of their partnership arrangement with the CVS PBM. Other players include: ImpactRx, Paydhealth, RxFree4Me, SHARx, ScriptSourcing, Matrix Payer*, and Horton Group. (*The AFP Matrix Payer, is, in no way, affiliated with BioMatrix Specialty Infusion Pharmacy.)

Do AFPs help patients?

While health plans and PBMS will claim AFPs help patients access high-cost specialty drugs, the reality is much different.

Here are a few concerns with AFPs:

1. AFPs confuse patients. The messaging is unclear from the health plan who must tell a patient their drug is not covered and then must direct the patient to call the AFP. Often the patient may not fully understand why they are calling the AFP, nor has it been made abundantly clear that their medication is not covered by their health plan.

2. AFPs force patients onto charity care, which the patient may find they are ineligible for due to income limitations or other factors.

3. An AFP may force patients to sign up for manufacturer copay cards, which must be turned over to the AFP to receive medication approval. This adds a layer of complexity to accessing the medication, and often resulting in unnecessary delays in care.

4. The process for accessing medications through an AFP is burdensome to the patient. Call the AFP, call the manufacturer, call the AFP back with the manufacturer copay card information, etc. This leads to non-compliance as patients grow frustrated and sometimes give up, and may delay treatment if out of or running low on medication.

5. If a patient grows upset with an AFP, they have nobody to call for grievances. The health plan is out of the picture and is no longer liable for any delays in care, patient frustrations, or other issues with being able to access the medication.

With all these issues and problems, the only current conclusion is that rather than helping patients, AFPs seem to hinder a patient's ability to access their specialty medications, thus putting their health in jeopardy. But to give the AFP some credit, after a patient jumps through all these hoops, in most situations, even if delayed, the patient does receive their specialty medication.

Are AFPs legal?

The jury is still out on that. A few things raise flags on their legality. For one, they are taking the full extent of the copay dollars on a manufacture card, which in the case of factor products can be $12,000 – $24,000. For another, an AFP is misrepresenting a patient to a charitable organization as "uninsured," despite the patient having insurance.

Some AFPs have even resorted to using imported drugs as a means to obtain free medication, and this could violate US law.[iii] It appears AFPs have found a loophole in the health care system and are exploiting it for their own financial gain.

Are AFPs being challenged?

Yes. Johnson and Johnson filed a lawsuit against SaveOnSP in July 2023. The lawsuit included Accredo, Cigna, and ESI (the PBM for Cigna), and alleges that the three were in a "scheme designed to siphon money" from the Johnson and Johnson assistance program CarePath.[iv] AbbVie has filed a similar lawsuit alleging AFPs are "fraudulent" and "deceptive."

States like Tennessee are working to fold AFP practices into their push for Copay Accumulator legislation, but unfortunately, states that currently have accumulator laws will be immune to AFP policies. AFPs have effectively undermined all current state copay accumulator bans.

Even though an AFP may appear as a maximizer, it is not, and is thus not legally bound to state maximizer law restrictions.

There is not yet a federal or national challenge to AFPs.

What can I do if my plan has an AFP?

Since most of these AFP vendors target self-funded plans, its likely your employer/Human Resources Department selected the AFP based on the savings they were promised without necessarily fully understanding its negative impact. Consider bringing it to their attention and see if they are able to make a change mid-year; if not, your information may encourage them to not select an AFP vendor the following year.

In some cases, your employer might be able to move your factor medication from a pharmacy PBM benefit to a medical benefit. Making this switch will remove you from the AFP which is only triggered on the pharmacy side by the PBM.

For additional questions, feel free to reach out to education@biomatrixsprx.com, or contact your local BioMatrix TAE (Territory Account Executive).

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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.

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RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

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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WITH ACCESS, THERE IS OPPORTUNITY

BY ARIANA BELLAN AND IRENE KALEKYE

Over 75% of people around the world with bleeding disorders don't have access to medication. This is the unfortunate reality for many of Save One Life's beneficiaries.

For those who have infrequent access to treatment, it often takes hours to reach the nearest hospital. Without treatment, their quality of life is drastically lowered. They are living in pain, forced to miss school or work, which in turn creates a cycle of poverty. Project SHARE is a program of Save One Life that seeks to improve access to treatment by sending factor directly to patients with bleeding disorders in developing countries. The hope is to allow them to have a better quality of life in order to achieve their dreams.

You have the opportunity to take part in this goal. Project SHARE's inventory comes directly from donations of unused, in-date factor. Reasons for donating include switching brands, developing an inhibitor, changing dosage, and more. By donating your unwanted factor to Save One Life, you can make a difference - one person at a time.

Save One Life is a nonprofit whose mission is focused on empowering people affected by bleeding disorders in developing countries through direct financial assistance and access to medical treatment. Their reach spans globally, having partners in 15 developing countries. This allows a Save One Life to hear about patients in need and send life-saving medicine through Project SHARE. The support they provide leads to many heartwarming stories like Saidi's, a young boy with hemophilia from Kenya.

Saidi's story, provided by our partner in Kenya, details the experience of growing up with hemophilia in a developing country, having little access to treatment, and the impact of Project SHARE:

In the remote coastal town of Kikoneni, in the southern region of Kwale lives a young boy named Saidi, who has been battling hemophilia type B since birth. Saidi's journey has been one of resilience and hope, thanks to the remarkable work of Project SHARE and Save One Life's programs.

For years, Saidi's family faced immense challenges in accessing the necessary IX treatment. The strenuous 74-kilometer journey from their rural home to Mombasa General Hospital, coupled with the steep travel costs of approximately 3,000 Kenyan shillings ($23 USD) plus a consultation fee of ksh 1400 ($10 USD), made acquiring the life-saving medication a daunting task.

However, a ray of hope emerged when Project SHARE extended its reach to Saidi's community. Through Save One Life, Saidi's father now has the opportunity to collect the vital Factor IX treatment from Malindi Ukunda, a nearby town accessible after crossing the ferry. This game-changing initiative has not only alleviated the financial burden on the family but has also significantly improved Saidi's access to medical care. The once-prohibitive travel costs have been reduced to a mere 1,500 Kenyan shillings, allowing Saidi's father, Swaleh, to acquire the treatment with relative ease.

This newfound accessibility has transformed Saidi's life, enabling him to attend school regularly without prolonged absences due to a lack of medication. No longer does Saidi have to endure distressing weeks or even months away from his studies, as access to the essential factor IX treatment is now within reach. The burden of hemophilia, once a constant struggle, has been lifted, allowing Saidi to embrace his education and pursue his dreams with renewed strength.

Through the unwavering efforts of Project Share and Save One Life, families like Saidi's in remote corners of Kenya are experiencing a remarkable transformation.

Access to life-saving treatments is no longer a privilege reserved for the fortunate few, but a reality that empowers individuals like Saidi to thrive and contribute to their communities.

To help children and families around the world like Saidi's, consider donating your excess factor to Save One Life. Visit: https://saveonelife.net/how-you-can-help/project-share/donate-factor

Read more success stories in Project SHARE's 2023 report: Project SHARE Report 2023.pdf
Learn more about Project SHARE here: https://saveonelife.net/how-you-can-help/factor

"My life has changed for the better because of you, I live happy days without pain and without fear. I thank you with all my heart."

- *Mohamed,* Morocco

Living with Von Willebrand Disease Type 2B: A Patient's Journey

BY JENNIFER DEGLOPPER

Living with a bleeding disorder is an intricate, exhausting, and often lonely experience, as one patient with Von Willebrand Disease (VWD) Type 2B shares in her candid reflection. Jennifer Hastie's story is not only a testament to resilience but also highlights the need for proper diagnosis, treatment, and advocacy within the bleeding disorder community.

A Family Legacy of Bleeding

Jennifer's journey began early. "My oldest and youngest daughters have it too," she says. "My mother likely has it, but she refused to be tested so as not to be blamed, as she put it." This generational thread underscores the genetic nature of VWD, though her own path to diagnosis was anything but straightforward.

A Lifetime Without Answers

"I had my first surgery for a hernia at age four," she recalls. "The doctor told my parents I should never take aspirin, but they never followed up." For decades, she endured symptoms without an official diagnosis, avoiding aspirin and NSAIDs on instinct. It wasn't until age 56—following extensive genetic testing—that she finally received a formal diagnosis.

Her daughters were diagnosed much earlier, at 5 years and 18 months old. "At first, my oldest was typed as Type 1, but they re-typed her later based on her sister's diagnosis and follow-up labs."

The Challenges of Diagnosis

Securing a diagnosis was an uphill battle. "I saw four hematologists in Tampa before I found one who took me seriously," she shares. "It was frustrating knowing that my symptoms were just as severe as my daughters', but without medical records—since they weren't digital back then—there was no proof."

The complexity of VWD Type 2B, where stress, exercise, and other factors can skew test results, made things worse. "I was genetically tested for 2B twice, and both times it showed I had it. But my hematologist initially didn't believe the results, thinking the lab wasn't

reputable." Complicating matters further, prior labs dismissed her diagnosis due to elevated levels that masked her bleeding tendency.

Living with VWD Type 2B: A Painful Reality

The physical toll of VWD 2B was severe. "I had horrific nosebleeds that lasted all night and heavy periods that could stretch for nearly three

months," she recounts. "At school, I'd bleed through my jeans and have to tie a jacket around my waist to hide the stains."

The menstrual bleeding left her physically and emotionally drained. "By the end of my longest period, I was so exhausted I thought I would die." Miscarriages and anemia compounded her challenges, while her struggles with ulcers in college forced her to take a break from her studies.

Despite these hardships, she persisted. "I underwent a total knee replacement in 2020, but my hematologist at the time didn't think factor treatment was necessary. Instead, I was prescribed baby aspirin and Meloxicam." Her knee deteriorated over time, and it wasn't until years later, when her new hematologist reviewed post-surgery imaging, that the true extent of her condition—ongoing hemarthrosis—was confirmed.

Misconceptions and Misdiagnoses: The Battle for Recognition

Navigating misconceptions about her disorder was one of the toughest parts of Jennifer's journey. "I was told by three different Tampa hematologists that I couldn't possibly have a bleeding disorder because my levels were 'normal,'" she says. "I tried explaining how stress and exercise could raise my levels, but they dismissed me."

It was only after years of persistence that she found answers. "When my oldest daughter switched to my primary care provider, the nurse practitioner connected the dots. She asked if I had the same bleeding disorder as my daughter and immediately ordered a genetic test. That test confirmed I had VWD Type 2B." Reflecting on the journey, she adds, "It's fascinating—and frustrating—that none of the hematologists I saw ever thought to run genetic tests."

Advice to the Not Yet Diagnosed: Don't Give Up

Asked what advice she would give to someone who is not yet diagnosed, her message is clear: "Don't give up." She emphasizes the importance of documenting symptoms. "Take pictures of your bruises. Outline them with a permanent marker. If they grow beyond the marked area, you'll have visual proof for your doctor." Documentation, she believes, is critical. "Upload these pictures to the patient portal—once they're part of your medical record, they're harder to ignore."

For her, the need for early intervention is also paramount. "If I had access to factor earlier in life, I wouldn't be in the physical shape I'm in today. I use a cane now, and my knee is permanently damaged. I also have neuropathy in my legs, making me prone to falls."

A Message of Hope and Persistence

Despite years of misdiagnoses and dismissals, she urges others to persist in their search for proper care. "You live in your body every day—you are the expert. Don't let anyone tell you otherwise." She acknowledges the emotional toll of advocacy but encourages patients not to give up. "There were many times I wanted to quit, but I kept pushing through, and now I'm glad I did."

For her, finding the right hematologist made all the difference as has prophy. "There are many great doctors out there—you just need to keep looking until you find the one who will listen."

Jennifer's story is a powerful reminder of the importance of self-advocacy, persistence, and proper care in managing chronic health conditions. "For me, getting the right treatment wasn't just life-changing—it was life-affirming."

HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

2024 Nobel Prize and Hemophilia

10/7/24 The 2024 Nobel Prize in Physiology or Medicine was awarded to two scientists for the discovery of microRNA and the determination of its role in gene regulation. So, what's that got to do with hemophilia? Possibly a lot. One of the many mysteries in hemophilia is that a small number of affected patients appear to have normal factor VIII or IX genes without any mutations. Then, why do they have hemophilia? Why do they have lower levels of factor activity in their blood? In many of these cases, it probably comes down to regulation of the processes that produce the clotting factor proteins.

The actual production of a protein involves a very complex process that we don't fully understand. First, most genes, including those for the clotting factors have regulatory (control) sequences, usually at the beginning of the gene. The regulatory sequences are sections of DNA that tell a cell whether, when and how much of the clotting factor to make.

For instance, the factor IX gene has a regulatory sequence that tells liver cells, but not other cells, to make factor IX. It also controls when and how much factor IX to make, in order to keep the amount of factor IX in the bloodstream at the desired level. If there is a defect in the regulatory sequence, the body might not make enough (or too much) factor IX, even if the rest of the gene has the correct sequence to make normal factor IX.

Once the regulatory section of the gene tells the cell to go ahead and make a factor IX molecule, the first step is "transcription" in which enzymes in the nucleus of the cell make an RNA copy of the DNA gene. That copy, called messenger RNA (mRNA), is secreted out of the nucleus into the main body of the cell where the rest of the cell's protein-making apparatus is located. The mRNA is basically a template or recipe that tells the cell which amino acids to string together in what order to make the protein.

This is where microRNA comes into play. The mRNA copy of the gene must be modified to make it suitable for use by the cell's protein-making apparatus. MicroRNAs interact with the mRNA to control the modification process. Therefore, if there are defects in the microRNA, the modification process can go awry so the body ends up not being able to make the protein, even if the original gene is OK.

The origin of these small, microRNAs come with an interesting backstory about scientists wrongly jumping to conclusions. In the mid-20th century, when researchers were first figuring out how genes work, they realized that we had much more DNA in our cells than was needed to make all the proteins in the body. They decided that all the extra DNA must not have a function and named it "junk DNA."

That decision turned out to be rather short-sighted. Now we know that the junk DNA has a number of uses, one of which is to make microRNAs. Today, we're realizing that there is almost nothing in the human body that is not functional, and in fact, that most things actually have several different uses. [2024 Nobel Prize announcement and Jankowska KI et al., Int J Mol Sci, 21, 3598, 2020]

Cognitive and Behavioral Outcomes in Children with Hemophilia

5/8/24 The eTHINK study looked at cognitive, behavioral and adaptive functions in children and young adults with hemophilia A or B in the US. The study included551 male children (433 As and 101 Bs plus 17 subjects whose disorder was not specified) from 21 US HTCs. The subjects had hemophilia of any severity, with or without inhibitors, and their ages ranged from 1 – 21 years. They found that performance on cognitive tests was generally comparable to or better than age-matched children in the general population, with a few exceptions. Two age groups, 4 – 5 and 10 – 21 years, performed worse in attention measures and processing speed. The authors also found no effect of hemophilia severity.

They did find a higher prevalence of attention-deficit/hyperactivity disorder (ADHD) and learning disorders

than in the general population, especially in boys aged 13 – 17 years. This shows the importance of screening for ADHD and similar conditions. Interestingly, children on prophylactic treatment showed fewer ADHD symptoms and emotional problems than those receiving on-demand treatment.

Two other interesting findings are that children whose mothers had higher education levels tended to have higher intellectual function and better attention. Similarly, lower family income was associated with lower intellectual, attention, executive, behavioral and emotional function and adaptive skills. Executive function is the ability to set and carry out goals. These findings suggest the importance of the child's environment and point to the need to consider that in treating patients.

These results were significantly better than those found in a similar study performed over 30 years ago and probably reflect improvements in hemophilia treatment. The authors point out that the study did not include females because there was insufficient data available and that further research is needed to determine whether there is a difference in the cognitive and behavioral effects between male and female children. [Mrakotsky C et al., J. Pediatr, online ahead of print 5/28/24]

Women with Hemophilia Have More Depression, Anxiety and Fatigue than Men

8/27/24 A group of researchers in the US looked at differences between men and women with hemophilia in terms of quality of life, and mental and physical health using questionnaires. In 118 men and 21 women with hemophilia A or B, the average age was 36.9 years. Approximately 26.3% of the men and 85.7% of the women had mild hemophilia. They found that the women were much more likely to experience depression, anxiety and fatigue than the men. There were no significant differences in the other outcome measures. The authors suggest that this highlights the need for mental health services to be integrated into the care of women with hemophilia.

Whether this difference is due to hemophilia requires more study. As Dr. Christine Kempton, one of the authors reports: "It was not surprising that we saw greater amounts of depression, anxiety, and fatigue; these are higher in women in the general population. We don't know if the differences are due to gender alone, hemophilia alone, or the interaction between the two." [Kempton CL et al., Haemophilia, online ahead of print 8/27/24]

Hemophilia Carriers Have QoL Comparable to Women with VWD and Other Bleeding Disorders

9/4/24 A group from India looked at quality of life (QoL) and bleeding profile in hemophilia carriers compared to women with established bleeding disorders including von Willebrand Disease (vWD) and factor VII, X and XIII deficiencies. Using questionnaires, they found that a significant number of the carriers had problematic bleeding tendencies and poorer QoL, in line with what is found in women with accepted bleeding disorders. [Radhakrishnan N et al., Cureus, 16(9), e68636, 2024]

Awareness and Diagnosis of Heavy Menstrual Bleeding Among Physicians and Patients

8/20/24 An international group of researchers looked at the awareness and perception of heavy menstrual bleeding (HMB) in women and girls in the US, UK, China, France, Germany, Oman and Saudia Arabia. HMB accounts for 18 – 30% of outpatient gynecological visits, and approximately one in five women with HMB have underlying bleeding disorders. They found that a major barrier to care is a lack of awareness and understanding of HMB by healthcare professionals (HCPs).

They found that many HCPs do not conduct investigations for women/girls complaining of HMB, and 22% of general practitioners (GPs) lack confidence in the management of HMB. Only 8% of GPs use screening tools to evaluate menstrual blood loss, and only 13% of GPs and 15% of OB/GYNs look for underlying bleeding disorders. In contrast, 76% of menstruating women/girls believed they could recognize HMB symptoms "well." However, 23% of these women/girls would not seek medical advice for abnormal/prolonged menstruation even though it disrupts their lives. The authors conclude that "There is a need for standardized clinical criteria to promote efficient diagnosis and management." [Kadir RA et al., Haemophilia, online ahead of print 8/20/24]

IDELVION®
Coagulation Factor IX (Recombinant), Albumin Fusion Protein

CONFIDENCE IN BLEED PROTECTION COMES FROM KNOWING YOUR FACTOR IX LEVELS

With IDELVION, Hemophilia B patients of all ages, can have reliable, high and sustained factor IX trough levels.

Knowing your factor levels, means knowing whether you're protected against future bleeds.

ONLY IDELVION DELIVERS

7 and 14 DAY DOSING* FLEXIBILITY
FDA APPROVED FOR ADOLESCENTS AND ADULTS

+ **20%** STEADY-STATE **TROUGH LEVELS**
WITH 7-DAY PROPHYLACTIC USE†

+ **0** SPONTANEOUS **BLEEDS**‡

KNOW YOUR FACTOR IX LEVEL. PROTECT YOUR FUTURE.

* Once well-controlled (1 month without spontaneous bleeding or requiring dose adjustments on a weekly dose of ≤40 IU/kg), people 12 years and older can be transitioned to 14-day dosing.
† The average dose for adolescents and adults receiving prophylaxis every 7 days was 37 IU/kg.
‡ The median AsBR for people who started on 7- or 14-day prophylaxis was 0. For people who switched to prophylaxis from on-demand, the median AsBR was 0.7.
AsBR=annualized spontaneous bleed rate.

IMPORTANT SAFETY INFORMATION

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

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

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

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

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

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

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

You are encouraged to report negative side effects of prescription drugs to the FDA.

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

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

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

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CSL Behring

HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Fall 2024 - 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.

HEMA Biologics Starting Phase III Study of Sevenfact in Major Surgery

6/7/24 HEMA Biologics distributes Sevenfact, an activated factor VII treatment for hemophilia A and B patients with inhibitors. The product is manufactured by LFB, a French pharmaceutical company. LFB is currently enrolling subjects with inhibitors for a Phase III study of Sevenfact use during major surgery. The study will be conducted in several US and international sites. [ClinicalTrials.gov Study NCT05695391]

TiumBio Approved for Phase Ib Study for TU7710 for Inhibitor Patients

7/16/24 TiumBio, a Korean biotech, is developing TU7710, a longer-acting activated factor VII (FVIIa) product for treatment of hemophilia A and B patients with inhibitors. TU7710 consists of a FVIIa molecule fused to the protein transferrin. Transferrin is a common plasma protein that transports iron around the body. Transferrin-fusion gives TU7710 a half-life about six to seven times longer than NovoSeven.

Tium has now been approved for a Phase Ib clinical study in Europe to study the safety, dosage and pharmacokinetics of TU7710. [TiumBio press release 7/16/24]

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.

Novo Nordisk's Alhemo Recommended for Approval in EU for Inhibitor Patients

10/18/24 Novo Nordisk is developing Alhemo (concizumab), an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) as a rebalancing agent. Concizumab is a daily subcutaneous injection for treatment of hemophilia A and B patients, with or without inhibitors. Alhemo has already been approved in Canada, Australia and Switzerland for treatment of hemophilia A and B patients, 12 years of age or older, but only for those with inhibitors.

Alhemo has now been recommended for approval in the European Union (EU) by the European Medicine Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP), also for A and B patients, 12 years of age or older, also only those with inhibitors. If the European Commission (EC) concurs with the CHMP recommendation, Alhemo would become the first non-bypassing agent for inhibitor treatment licensed in the EU. The U.S. FDA is currently also reviewing a license application for Alhemo. [Novo Nordisk press release 10/18/24]

Pfizer's Hympavzi Approved by FDA

10/11/24 Pfizer is developing Hympavzi (marstacimab), an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) as a rebalancing agent. Marstacimab is a once-weekly subcutaneous injection delivered via pre-filled syringe or auto-injector pen for treatment of hemophilia A and B patients, with or without inhibitors. FDA has now approved Hympavzi for treatment of hemophilia A and B patients, 12 years of age or older, but only for those without inhibitors. Licensure of Hympavzi for inhibitor patients is still pending. In addition, Hympavzi was also recommended on 9/19/24 for approval in the European Union (EU) by the European Medicine Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP), also for A and B patients, 12 years of age or older, without inhibitors. [Pfizer press release 10/11/24]

10/25/24 Pfizer has given Hympavzi an annual cost of $795,600, which they say is comparable to the prophylactic treatments for hemophilia A and B now on the market. They have also set up a copay assistance program to help patients who have trouble affording their medications. Hympavzi is expected to be available around the end of 2024 or early in 2025. [Hemophilia News Today 10/25/24]

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.

Be Bio Awarded Fast Track Designation by FDA and also Raises $82 Million in Funding

10/22/24 Be Biopharma is developing BE-101, a cell therapy for hemophilia B in which a patient's B cells are genetically modified to produce factor IX. The treatment involves harvesting B cells from the patient's bloodstream and then modifying them in the laboratory to contain a normal factor IX gene. The modified B cells are then transplanted back into the patient's bloodstream where they will continuously produce factor IX. Be Bio is currently conducting a Phase I/II clinical study to test the product.

The treatment is expected to be long-lasting, since B cells have a half-life of about 17 years and can also reproduce to generate additional factor IX-producing B cells. In addition, unlike the current gene therapy products, which can only be taken once, the product should be re-dosable.

In September, 2024, Be Bio was awarded Fast Track Designation by the FDA. According to FDA, "Fast track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier." The company also announced that they have secured an additional $82 million in financing to support the development of BE-101. [Be Bio press release 10/22/24]

Pfizer Loses Patent Infringement Suit to UniQure

10/29/24 Both Pfizer's Beqvez and CSL's Hemgenix use the higher-activity Padua variant of factor IX in their gene therapies. However, uniQure, the original developer of Hemgenix, owns various patents on the Padua variant. Pfizer challenged the European patent in court as invalid and has also challenged the Padua variant patents in the US and Canada. Now a judge in the UK has ruled that uniQure's European patent is valid and that Pfizer infringes it with their gene therapy. What happens next remains to be seen. It is business and law, not something as simple as science!

CSL has an exclusive license from uniQure for the Padua patents. That would seem to preclude uniQure from further licensing the patent to Pfizer, but in business everything is negotiable. We'll also need to wait and see what the US and Canadian courts think. The poor market performance of all of the approved hemophilia gene therapies could give the companies second thoughts about spending a lot more money defending their positions. Stay tuned. [Life Sciences Intellectual Property Review article 10/29/24]

HEALTH EQUITY IN THE HEMOPHILIA B COMMUNITY: PERSPECTIVES OF PATIENTS' RECAP

BY MARTA THOMAS

The quest for health equity remains a cornerstone of modern healthcare, particularly for communities grappling with chronic conditions like hemophilia B. In March of 2023, The Coalition for Hemophilia B, together with Upequity, launched an important research initiative. On February 8, 2024, Dr. Taneasha Washington presented the pivotal findings of the *Health Equity in the Hemophilia B Community* research project to the Coalition patient community.

This comprehensive study, informed by the perspectives of 609 participants, aimed not only to highlight the disparities within the healthcare system but also to forge a path toward equitable care. Integral to this endeavor were the advisory council members, Fel Echandi, Meisha Douglas, Dr. Fernando Sanchez, and Royal Smith, whose significant contributions were vital throughout all stages of the project.

The study's findings brought to the forefront the voices of a diverse range of individuals living with hemophilia B, including a notably active demographic of young adults aged 25 to 34. These individuals, often in the throes of significant life transitions, face the dual challenge of managing their condition and navigating their personal and professional lives.

The study also recognized the varied needs of all age groups affected by this condition. Not just of those reaching adulthood but to older adults managing long-term health considerations, the healthcare system's capacity to provide seamless and adaptive care is critical to ensuring the well-being of every person living with hemophilia B.

A revelation from the survey was the financial burden borne by patients: a significant majority faced out-of-pocket expenses for treatment on top of their insurance coverage. This economic strain represents a broader issue of affordability and accessibility in healthcare that is unsustainable. The financial challenges are not simply numbers on a bill but rather indicative of the sacrifices patients make, which can impact their quality of life and long-term health outcomes.

The ability to access specialized care is yet another hurdle that many in the hemophilia B community face. Survey participants in the study indicated instances of being denied access to a hemophilia specialist based on their gender, race or geographic location, revealing gaps in equity, quality, availability, and geographic distribution of services, which are crucial for the management of this condition.

The study also highlighted the need for improved education among emergency care providers. The project identified a significant learning curve in the treatment of hemophilia B, emphasizing the need for specialized training to prevent life-threatening complications during emergencies.

In the realm of clinical trials, a key recommendation in the study calls for the creation of resources to demystify the process for patients. By breaking down the complexities into accessible information, these guides can empower patients to make informed decisions about their participation in clinical trials.

The research also shone a light on the distinctive healthcare experiences of women with hemophilia B, who often face barriers in diagnosis and treatment due to historical gender biases. Addressing these challenges requires a concerted effort to ensure that women's health needs are recognized and met with the same urgency and dedication as those of their male counterparts.

Advocacy organizations were recognized for their instrumental role in driving change toward health equity. These groups bridge the gap between patients and the broader health system, ensuring that patient voices are amplified and their needs are addressed. Recommendations include expanding education and resources to aid in navigating insurance complexities and raising awareness of women's challenges with bleeding disorders.

To ensure that the insights from this study reach as many people as possible, a robust plan is in place to share the findings widely. By spreading the word, we will contribute to building a more equitable healthcare system for hemophilia B. This research project represents a beacon of hope and determination, a catalyst urging collaboration among stakeholders to address equity gaps in the hemophilia B community.

The journey toward health equity in the hemophilia B community is both challenging and inspiring. It requires a collective response where individuals, healthcare providers, policymakers, and advocacy groups unite to transform the healthcare landscape. This project is not the conclusion but the commencement of an ongoing effort to ensure that health equity is not an aspirational goal but a realized one for every individual affected by hemophilia B.

As we look to the future, we extend our heartfelt thanks to our sponsors CSL Behring, Pfizer, and Sanofi. Their support underscores the collaborative spirit needed to foster systemic change. With these partnerships, the hemophilia B community is well-positioned to advocate for and achieve the healthcare equity it deserves.

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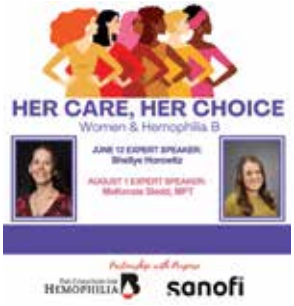
HER CARE, HER CHOICE

BY MARTA THOMAS AND ALYSHA MCCABE



The virtual Her Care, Her Choice series, dedicated to women's empowerment and education, continued with two impactful sessions held on June 12 and August 1. These sessions provided valuable insights and tools for navigating healthcare challenges with confidence.

On June 12th, Shellye Horowitz led a session on "Partnering with Providers for Care: Sharing Your Patient Story." Shellye has an impressive background with 25 years in K-12 public education, where she held roles including principal and school counselor. Shellye is also deeply connected to the bleeding disorders community with six generations of hemophilia A in her family.

The session was designed to help participants communicate better with their healthcare providers. Shellye emphasized the importance of working together with your healthcare team to get the best care possible. She shared practical tips on how to tell your patient story effectively, which can help providers understand your needs and tailor their care accordingly. Shellye's advice included keeping a health journal to track symptoms, treatments, and responses, which you can refer to during appointments. She also highlighted the importance of taking pictures to document physical symptoms and changes. These photos can provide visual evidence that supports your descriptions, making it easier for your healthcare provider to understand your condition.

Throughout the session, Shellye encouraged participants to think about their own healthcare experiences and how they could advocate for themselves more effectively. She gave examples of how to construct a concise and effective patient narrative, ensuring that all relevant information is conveyed without overwhelming the provider or yourself.

Shellye also shared her own journey with hemophilia, adding a personal touch that resonated with many attendees. Her story underscored the importance of persistence and self-advocacy in navigating the healthcare system. She emphasized that every patient's story is unique and valuable, and that sharing these stories can lead to better understanding and improved care. Her insights into the barriers women often face hit home with many participants, reinforcing the importance of continued efforts to promote equity in healthcare. Shellye's session was a powerful reminder of the importance of patient advocacy and the impact that sharing one's story can have on the quality of care received. Her expertise and personal connection to the bleeding disorders community made the event both informative and inspirational.

On August 1, Makenzie Sledd, MPT, led the session on "Joint Health for Women." A physical therapist at St. Louis Children's Hospital, Makenzie has expanded her practice to include adults and children with bleeding disorders. Her energetic and approachable style helped participants engage with her, gleaning insights on joint and musculoskeletal health.

Makenzie's down to earth approach stressed the importance of physical activities to maintain joint health while offering tips to help you succeed in realistic ways. She asked participants to consider the following when planning physical activities: What makes you happy + Physical activities that don't make you angry = Success!

Participants shared some ideas such as crocheting while walking on the treadmill or listening to an audiobook while taking a walk outside or inviting a friend to join you on a walk for some social bonding and physical activity. Makenzie also suggested creating SMART goals for continued success. SMART goals are an outline for setting goals that are specific, measurable, achievable, relevant, and time bound. The framework helps ensure that goals are attainable within a certain time frame and are carefully planned out, executable, and trackable.

Participants had the opportunity to share personal experiences and ask questions about specific concerns. From these interactions, it was clear that Makenzie is a true advocate for women and the community and strives to provide patients with the knowledge and tools they need to be successful on their own.

Wrapping up the night, Gha'il Rhodes Benjamin returned to lead participants in another session of community reflection and empowerment. Gha'il began with stretches and a guided meditation session. She encouraged participants to check in with themselves and highlighted the importance for doing regular check ins and acknowledging your truths. Gha'il helped to create a calm and connective atmosphere for all.

After each meeting, participants expressed their appreciation for the speakers' insights and the practical strategies they provided. The sessions left attendees feeling empowered to take a more active role in their healthcare and inspired to share their stories with confidence. The sense of camaraderie and support within the hemophilia B community was evident, reinforcing the message that no one must navigate their health journey alone.

Reflecting on the success of these events, it's clear that gatherings like these are crucial. They offer a blend of education, support, and social interaction that is essential for managing hemophilia B. By sharing their stories, individuals can advocate for themselves and others, paving the way for a more understanding and responsive healthcare system.

We would also like to extend our heartfelt thanks to our sponsor, Sanofi, who's generous support made these events possible. Their commitment to the bleeding disorders community is greatly appreciated.

sanofi

Comments: June 12th

"Thank you for these events. It's nice to stay connected and learn."

"Thank you for having this space for us women. We appreciate it."

"Loved hearing from Shellye! Her encouragement to document everything is what I needed!"

August 1st

"Love the connection with my hemo B ladies. Uplifting and inspiring!"

"The PT angle about "just move" is very powerful."

"This was a great reminder to check in with myself."

"Thank you! I was reminded to make a list to take to my PT appointment next month."

WELL-BEING, RELAXATION, AND MOVEMENT FORO LATINO DE HEMOFILIA B

BY LAURA ECHANDI

On August 6th, we had the pleasure of welcoming Corazón Tierra as a host for our Latino Hemophilia B forum group's Zoom meeting. Corazón is a talented interdisciplinary dance artist, poet, writer, and educator specializing in dance and somatic movement. Her passion lies in helping children, women, and families cultivate a deeper connection between body and mind.

During the session, we connected as parents, partners, and children navigating the daily stress of living with hemophilia B. Through Corazón's guidance, we explored breathing techniques and healing movements, using dance as a tool to recharge and find relaxation amidst life's challenges.

Dance offers more than just a fun way to move—it's a powerful form of exercise with numerous health benefits. It strengthens the heart, improves muscle tone and bone health, and enhances balance. Additionally, physical movement encourages the release of dopamine, serotonin, and endorphins, boosting mood, promoting relaxation, and improving sleep quality. We encourage everyone to apply what they learned during this session. Caring for a bleeding disorder means caring for both the body and mind. By reducing stress and strengthening the body, we can improve our overall well-being.

We look forward to seeing you at our next meetings as we continue to focus on health, movement, and self-care—together.

2024 NBDF CONFERENCE

BY ROCKY WILLIAMS

This year's Bleeding Disorders Conference, held by the National Bleeding Disorders Foundation, took place from September 12-15 in Atlanta, Georgia!

Wayne, Rocky, Erica, Farrah, and April had an incredible time representing The Coalition for Hemophilia B at the conference. Thank you to everyone who visited our booth. It was so great to see everyone and to participate in this amazing event!

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FAMILIA DE SANGRE

BY LAURA ECHANDI

The Coalition for Hemophilia B was honored to participate for the first time in Southern California's largest blood disorders event, ***Familia De Sangre,*** held from September 6-8 at the Anaheim Marriott Hotel in Orange County. The entire event was conducted in Spanish, making it a culturally enriching experience.

Organized by the Hemophilia Foundations of Southern California, Northern California, San Diego, and Central California, the event brought together over 700 Spanish-speaking participants. Attendees engaged in a variety of educational workshops, covering topics such as mental health, the latest medical advancements, healthy cooking, and advocacy efforts.
We were delighted to see enthusiastic participation from not only the hemophilia B community but also individuals representing other blood disorders. The event provided a unique opportunity to share cultural traditions, including vibrant costumes and customs that reflected the richness of participants' heritage.

The experience culminated with a full day of fun at Disneyland, creating lasting memories for everyone involved. We look forward to reuniting with the community at next year's event!





NATIONAL CONFERENCE FOR WOMEN AND TEENS

BY JENNIFER DEGLOPPER

From October 4–6, Farrah exhibited at the National Conference for Women and Teens with hemophilia and rare factor deficiencies, organized by the Hemophilia Foundation of Michigan (HFM) and held both in Detroit and online. I attended the event as a participant. The conference was exactly what powerful, inspiring, and full of connection.

Shari, HFM's education and program services director, flawlessly led the event. The sessions were invaluable, covering critical topics such as *Self-Advocacy in the Emergency Department* and *Understanding Factor Levels and Bleeding in Hemophilia Genotype Positive Females*. We were privileged to learn from rock-star presenters like Jill Johnsen, MD, from the University of Washington Center for Bleeding Disorders, and Lynn Malec, MD, associate director of the Comprehensive Center for Bleeding Disorders at Versiti Blood Center of Wisconsin and associate professor at the Medical College of Wisconsin.


The Coalition for Hemophilia B was happy to be invited to exhibit. We look forward to next year!

RESUME WRITING WORKSHOP

BY ROCKY WILLIAMS

On Tuesday, October 8, our very own Dr. April Willis hosted an engaging virtual Resume Writing Workshop, offering participants expert advice on how to craft standout resumes. Dr. Willis covered the dos and don'ts of resume writing, including tips on avoiding common mistakes and highlighting measurable achievements. Attendees asked questions and receiving personalized feedback on improving their resumes.
The workshop also featured LinkedIn tips and strategies, with Dr. Willis guiding participants on optimizing profiles for networking and attracting recruiters. To keep things interactive, participants played word scramble games, reinforcing key concepts in a fun way. The session wrapped up with an example of a resume showcasing all the best practices discussed.

Overall, it was an insightful and educational evening, blending valuable career advice with engaging learning games. Participants left feeling empowered and ready to update their resumes and LinkedIn profiles.

UPCOMING EVENTS

Visit our website to find more Upcoming Events!

www.hemob.org

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> SHANNON L. ALDER

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

MEET ISABELLE: TEEN ADVOCATE AND COUNTY FAIR PRINCESS!

BY SHELLY FISHER

Although we only spoke over the phone, Isabelle's kindness and warmth shone through in her voice throughout our conversation. Despite her busy schedule as a Pickaway County Jr. Fair Princess, she made time to talk, and it quickly became evident that her title meant much more to her than just a crown.

"I'd like to use this role as an advocate to bring awareness to young women with bleeding disorders," she explained.

Well-spoken and thoughtful, Isabelle shared that she had often felt isolated as a female hemophilia patient—a reality she is determined to change for other young women in the future.

When we spoke again after school started, and I asked her how it was going, her optimistic reply was, "So far, so good." This belied the fact that she had just spent the first three weeks of the year recovering from an emergency surgery that followed a tonsillectomy due to complications involving a bleed. Undaunted and with a jam-packed extracurricular itinerary ahead of her, Isabelle was eager to start her first year of high school.

Like most students, she was looking forward to seeing her friends in class, and even though she would be splitting her time between 4-H, FFA, the Ohio State Fashion Board and the Pickaway County Jr. Fair Board, Isabelle indicated that she would definitely have time to focus on her favorite subject - English. "I love books and writing so much!"

Isabelle felt she was known for her passion and drive among her peers, and not surprising at all; she thought her friends might describe her as caring, loving, and

confident. She also added they might say she was sometimes dramatic.

Though this is her first year to be a member of the junior fair board, FFA, and state fashion board, this ninth grader is a 10-year member of her 4-H Club. During that time, Isabelle completed multiple community service projects, and served as the treasurer, secretary and vice president. In addition, she won 5 state awards and one national award as well.

Diagnosed at 18 months old when she went in for a surgery, Isabelle doesn't remember a time when she

didn't have a bleeding disorder, and she has some advice for anyone newly diagnosed. "Don't freak out. You are not alone. Get involved in a local bleeding community." Isabelle's advice to others is based on her own experiences within the bleeding communities. After attending a recent CHB event, the impact was life-changing for her. She shared, "I love learning about other bleeding disorders, and I was excited to see new products, especially the sub q injections for hemophilia B. It opened my eyes to the bigger community, and I finally got to meet other woman with hemophilia B, which gave me so much encouragement!"

Isabelle shared that her favorite part of a conference is learning how she can help other teens with bleeding disorders, so it's no surprise that she is currently a teen bleeding disorder advocate for the state of Ohio. This role has allowed her to participate in House Days and serve as a teen representative to select conventions, in addition to presenting to teens, and both selecting and training the 2025 teen advocates.

As a young woman with hemophilia B, Isabelle is no stranger to adversity. What's her go-to source for courage to face any obstacle? "Philippians 4:13 is my power Bible verse. It says I can do all things through Christ who strengthens me. This reminds me I am not alone and when life is rough I have God to hold me up. He has my back."
In addition to her faith, Isabelle has a strong support team, and hematologist Dr. Amy Dunn is one of the most important members. "She has always treated me as a patient of hemophilia, not just a girl carrier." Isabelle counts her parents as her strongest supporters, and she was quick to indicate her gratitude because "they always support my crazy ideas and let me live my life regardless of my disorders."

A credit to her generation and the hemophilia community, Isabelle is a unique individual. With a heart for advocacy, eloquent expression, and a passion to improve the lives of those around her, I'm glad she will be using her superpowers for good.

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.

EMPOWERING ADVOCACY: A TEEN'S JOURNEY WITH HEMOPHILIA B AND THE COALITION

BY CONNOR

I was so excited when Rocky asked me to cohost the teen event! It was such a great opportunity to bring Bracketeering, a Jackbox game where teens vote on the funniest answer for a prompt, to make the game night part extra special. Rocky really empowered me to take charge organizing the game—we kicked things off with music playing and introducing the teens that registered to sign up. The teens were so engaged, and it was amazing to see how much fun everyone had. The energy in the room was fantastic, and the laughter and camaraderie made the night truly memorable.

Rocky's guidance helped my passion and creativity really shine through, and it's clear how much I enjoy being involved in the coalition. I'm already talking about how excited I am to do it all again at the next event!

We also had some moments to focus on mental health. We were fortunate to have Matt Barkdull lead the event in a rap session where we all talked about what grade we were in and what we were excited about in the upcoming year. From what I could see, the teens found it incredibly helpful. It was so rewarding to see them connect not just with the games, but with each other on a deeper level.

Overall, the night was a huge success, and I'm so proud to have been a part of it. I'm already looking forward to the next event and continuing to work with such an amazing team.

HOSTING MY FIRST VIRTUAL TEEN GAME NIGHT FOR THE HEMOPHILIA B COMMUNITY

BY JAYDEN MACDOUGALL

Hey everyone! I recently had the awesome opportunity to host a virtual game night for the hemophilia B community, and I wanted to share how much fun it was! It was such an honor to bring everyone together and hang out, even if it was over Zoom. I decided to play *Wheel of Enormous Proportions* from Jackbox Games on Oct 2nd, and let me tell you—it was a blast!

We used Zoom, which worked great because everyone could easily join from their phones or computers. I picked *Wheel of Enormous Proportions* because it's super fun and easy for anyone to play. The game is all about answering trivia questions to earn slices for a giant spinning wheel. Then, you spin the wheel and hope it lands on your slice to get points. It's so much fun watching the wheel spin and seeing who gets lucky (or not, haha).

Before we started, I made sure to explain the rules so everyone knew what to do, but most people caught on pretty fast. It was awesome to see everyone having such a good time, laughing, and joking around in the chat. Even though some people got really competitive, it was all in good fun!

Honestly, the best part for me was just hanging out with everyone. It was cool to chat with other people who understand what it's like living with hemophilia B. We shared stories and gave each other advice, which made it even more special. Plus, it was great to have a break from the serious stuff and just relax.

I had so much fun playing the game myself! Every time the wheel was spun, the suspense was real. I was cheering when I got points and laughing when I didn't. It was exactly what I needed—a night of fun with new friends.

Hosting this game night was such an honor for me because I know how important it is to feel connected, especially when you're dealing with something like hemophilia B. Being able to put this together and give everyone a chance to just have fun and forget about everything for a while was really special. It felt great knowing I could bring some joy to our community.

After how well this event went, I'm already thinking about planning another game night soon. If you missed out this time, don't worry—there will be plenty more opportunities to join in the fun! I can't wait to see everyone again and maybe even try out some new games.

Thanks to everyone who joined, and if you didn't, I hope to see you next time! Let's keep having fun and supporting each other!

MEET CONNOR: HE MAY LOVE STUDYING THE PAST, BUT HE DEFINITELY HAS HIS EYES ON A BRIGHT FUTURE!

BY SHELLY FISHER

Connor was about ten days away from wrapping his junior year and looking forward to being a senior when we visited. He shared, "You don't have to take any final exams when you're a senior, and I've heard it's the best year of your life." As the treasurer of his school's student council, chief editor of the yearbook club, and a member in the select choir, he was finishing strong with a few irons in the fire.

Active on his school's student council, he attends all the meetings with the school's board members and dean, and then he communicates changes to existing policy to the student body. He said, "They know I will tell them what I really think about something."

As chief editor, Connor was excited to show me the final version of his yearbook, complete with a picture of his school and a traditional logo of their mascot. The yearbook committee's votes decided the cover, and the legacy mascot had won out over a dragon.

It was no surprise when Connor confided that his rich baritone voice had earned him a spot in the bass section of the select choir. He was anticipating a competition at the end of the week, and although he had performed in many concerts, this was to be his first experience at a contest representing his school. With a lot of the choir members in their first year of participation, Connor felt it would be an invaluable learning experience for everyone. He was also looking forward to celebrating at an amusement park called The Great Escape afterwards.

When the conversion turned to academics, Connor told me he was a history and U.S. government fan. With a penchant for historical research, current events and global news, the soon to be senior hopes to have a future either in politics, or work as a CNN news anchor. When I noted that his voice was perfect for broadcasting, he said, "I get that a lot. Everyone wants me to go into television and radio." He also shared, "In my school, I'm the political guy. Whenever something is going on in the news, I'm the first to tell everybody."

In addition to enjoying some debate opportunities in his history class, Connor shared that he has really enjoyed all his classes this year and has even been on the high honor roll for all three quarters of the school year. He particularly enjoyed a math class as well where he felt the lessons were applicable to real life and allowed him to participate in a mock stock market. He shared that "he did pretty well" and would like to try his hand at the real stock market one day. Connor also enjoyed the reading list for English and noted The Great Gatsby as




one of his favorites.

When asked how his friends might describe him, he said, "I'm not quite the popular kid, but I'm not the loner guy either. I talk to everyone about the news, sports, and music, and I think they would say that I am a good person." After following up with a question about the type of music he discusses with friends, he listed Frank Sinatra, Dean Martin, the Beach Boys and classic rock as some of his favorite artists.

Connor has hemophilia B and an inhibitor, but that does not stop him. He enjoys gym class activities like flag football and pickleball and confided, "I try to do as much as I can, and I play like I'm in a sport." He also enjoys watching sports and is a huge Green Bay Packers and New York Yankees fan. Connor is also a great sport because he congratulated me on my home team, the Texas Rangers' World Series win last year.

Though he didn't feel he should accept an offer from the Make-A-Wish program, his father encouraged him to do so, and he asked to go to a Yankees game. "We played the Toronto Blue Jays and won. Completely different from watching at home and seeing them play in a stadium. I definitely had goosebumps during the 7th inning stretch when they played a patriotic song." When asked who his favorite player was, he said without hesitation, "Aaron Judge, it's gotta be Aaron Judge."

Connor's diagnosis came early around 18 months when he was learning to walk and the bruises that were a result of falling refused to heal. He also learned that he had an inhibitor to factor IX. As a result, he now takes AlphaNine SD, and travels to his clinic an hour and a half weekly to get it. In addition, he goes for a monthly 4-hour infusion of intravenous hemoglobin (IVG).

When asked how he would encourage someone who had just been diagnosed, Connor had this to say: "Even going through all that I do for treatment, I still strength through. I want to be a story to tell and a role model for all the young-uns. I want to tell them not to let it stop them. You just gotta keep your head up. Your diagnosis might sidetrack you, but don't let it overcome you. There's been a lot of new medicine in the last few years. It's going to get better. Don't let it control you. Be who you are and are going to be - be the astronaut, be the firefighter, be the cop."

Connor's top priority as a politician would be leveraging a political role to "work with the FDA, CDC and WHO to get content to the community and promote progress to find a cure. Not many people even know what hemophilia is."

After finding out about The Coalition for Hemophilia B from his doctor, he headed to the CHB Symposium in Dallas in April of 2024. He shared, "I met a lot of great people that made you feel like a human person and not a disorder. I've only met two other guys with severe hemophilia B and an inhibitor, and it was down at the show in Dallas." He was proud to say that after finding out more about hemophilia B, his biology teacher asked him to lead a lesson on it for his class at school. "Not that many people know what it is." Connor counted his time at the ranch riding horses, shooting bows, and swinging a lasso with his CHB group as a core memory that meant a lot to him, and he's looking forward to next year in Orlando. "Rocky's a great guy, and I really appreciated having some input into the next symposium."

True to his love of history and music preferences, Connor offered the following when I asked if he had a quote, or song that he often thought of as a mantra for his life. "Ain't That a Kick in the Head by Frank Sinatra. Of course this had to happen to me. And Franklin Delano Roosevelt said, 'The only thing we have to fear is fear itself.' Don't be afraid of who you are, just be who you are and don't let the disorder take control of you."

Connor has an amazing support team, and he wanted to thank them all for the specific role they have played in supporting him since his diagnosis. "My grandma took care of my PICC line, and my dad has been with me for all the driving and everything. I want to thank Dr. Halligam. Dr. Porter retired and she was my hematologist since I was little. She always treated me like a person. I want to say thanks to my grandma for also stepping in when my father was at work."

In closing, he added, "I just want to thank CHB for making me feel like a human being again and not as a disorder."

B inspired!

Stories and artwork from teens in the Hemophilia B Community

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IN THIS ISSUE:

MEET CONNOR

MEET ISABELLE

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!