

THE COALITION FOR HEMOPHILIA B



HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

SPRING 2022



**SYMPOSIUM
IN THIS TOGETHER
THE BEATS MUSIC PROGRAM**

**EMERGING THERAPIES
PORTRAITS OF PROGRESS**

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MISSION

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

SYMPOSIUM 2022: FIRST ANNUAL HYBRID SYMPOSIUM!

BY GLENN MONES

From May 19th to 22nd, The Coalition for Hemophilia B hosted its first-ever Hybrid Symposium. We were delighted to host over 500 in-person attendees from over 45 states, Puerto Rico and Europe at the Renaissance Orlando at SeaWorld in Florida. Additionally, we were joined virtually by 300 participants from 40 states plus Puerto Rico, Canada, and Australia.

During the height of the pandemic, we shifted to virtual events for the safety of our families, speakers, and sponsors. This year we were thrilled to be able to return to an in-person event by employing a variety of safety measures for everyone's protection. At the same time, we learned that many of our members still enjoyed the benefit of attending the event virtually, so we held sessions for them as well.

The Symposium featured a variety of amazing educational sessions led by experts in their fields. Many of these focused on advances in medical knowledge and treatment including *How Much FIX is in your Blood* with Dr. Robert Sidonio, and a panel on *Emerging Therapies* led by Brian O'Mahony of the World Federation of Hemophilia. Brian also shared his personal experience of being one of the first patients to receive gene therapy for hemophilia B.

Speakers who shared their personal stories were also an important part of the program. In *Unburying My Father*, Zander Masser took us on a moving journey of the life of his father, Randy, a photographer with hemophilia B.



A wide variety of sessions offered participants tools for living and improving quality of life. These included *Navigating Finances* with social worker Ellen Kachalsky, *Work/Life: The Five Buckets Principle* with Wendy Wollner, *Create Immediate and Lasting Joy* with Catherine Canadeo, and many others.

The speakers and sessions listed here represent just a fraction of the many offerings at the Symposium which held about 58 sessions for all ages and targeted sessions for specific groups including men, women, teens, parents, and others. Exhibit hours offered more opportunities for continued interaction and





then we closed out the final night with dinner, comedy entertainment, and a special appearance from our all-time favorite band, "THE BLEEDERS," who rocked the house!

We hosted 40 virtual sessions with 12 livestreamed and 28 that were virtual only, with speakers who went on Zoom, including activities and rap sessions. The virtual event ran from Thursday evening through late Sunday afternoon.

Thank you to all of our speakers, presenters, facilitators, and participants!

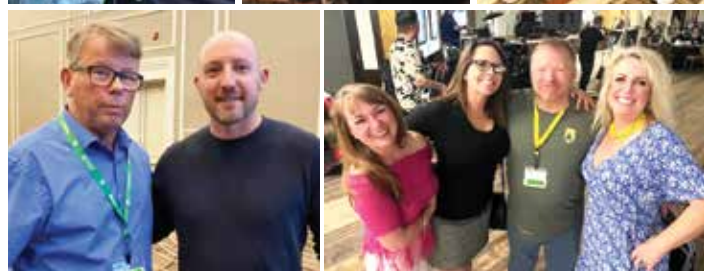


We are grateful to our many sponsors including CSL Behring, Medexus, and Pfizer (Platinum Level), Novo Nordisk and Sanofi (Gold Level), HEMA Biologics, Takeda and Tremeau (Silver Level), and others.

And thank you to our Virtual Sponsors!



Save the date for our next Symposium: March 16-19, 2023. Applications for travel scholarships are open now!







SYMPOSIUM COMMENTS

"Our first time going to the Symposium – loved it and highly recommend others to attend in person. We made incredible friends, learned new information to help advocate for our son, and he learned how to infuse himself!"

"First of all, thank you for the opportunity to attend the 2022 Symposium. In May 2021, we received the diagnosis of severe hemophilia B for our baby. Our world fell apart and we went through very difficult times emotionally as a family because we were totally unaware of the condition of hemophilia because we don't have it in our family. We began to look for information about hemophilia in order to manage this new stage that we had to live. A few months after diagnosis we found CHB which made me very excited. We began to receive information through email and we have a better picture of hemophilia. We were given the opportunity to attend the 2022 Symposium. Before going, I had mixed feelings such as excitement but at the same time fear. As soon as we arrived and started meeting other CHB families and members my heart filled with complete joy. They welcomed us with love and the most beautiful smiles I have ever seen. Meeting families and parents who have gone through the same moments as we have has filled us with a lot of hope and security. Knowing such a loving and understanding community exists made us feel that we have a safe harbor in our lives and even more in the life of our baby. The information we received throughout the Symposium has been of great help and we are excited for evolution in treatments that are beginning to reach hemophilia B patients."

"CHB members and CHB families...THANK YOU SO MUCH! Our first Symposium with you has been one of the best moments of our lives. They thought of everything. The organization was spectacular. The

information and your panelists were very helpful. Everything was BEAUTIFUL. It makes us very happy to be part of this beautiful community. Thank you for everything you do. And what a blessing to coincide on this path. Thank you for helping empower families. Thank you for everything! We are very grateful! We can't wait to see you at the next symposium."

"I didn't know what I was getting into or what to expect. Little did I know, it was everything I was looking for and everything I had been missing. I found knowledge, understanding, inspiration, HEALING, resources, sympathy, magnificent human beings, life-long friendships, a newfound family, a support system, and strength... just to name a few. There were tears and there was SO MUCH LAUGHTER! I am so incredibly grateful for this opportunity we got to share together. Words cannot explain all of the positivity, light and CLARITY this experience brought. THANK YOU for your acceptance with open arms. I cannot wait to see you all again...Until next time my brothers and sisters."

"We had such a great time at our first ever Symposium. We meet so many great people that are going through the same experiences as us, which is so nice to relate to. Our kids had a blast in the kids' care and we had a great time listening to great speakers. I didn't realize what a big event this was with over 500 attendees! That's amazing! We can't wait to come back!"

"Thank you for the scholarship to attend the conference last week. It was an incredible experience. My son learned how to infuse himself—he got it the very first try! I know it was a lot of work but thank you for creating this opportunity for our family."

"It was our first time at the Symposium one year after our baby's diagnosis. Attending the Symposium and meeting other families was one of the best experiences



we ever had. It helped us have more information and resources to work for a better quality of life for our baby and our family. Thanks for everything!”

“Symposium gave us more confidence, knowledge, and encouragement to be our own advocate and to not be afraid to ask questions. It provided connections to our factor medicine representatives who are now just a direct phone call. In addition, with new friends sharing how they self-infuse, my son decided to give it a try himself. The practice booth in the exhibit provided the infusion opportunity and I am proud to say my son now can self-infuse! He has more control and confidence of his well-being – he is only 13! This would not have been possible if we had missed this event – forever grateful for this Symposium. Greatest lesson learned, don’t hold your kids back due to your own personal fears. They are capable of so much more than you can imagine, even at a young age. Let them participate in their personal health and well-being. I have a new respect for my son and know we are a team.”

“The Symposium is such a great event for OUR COMMUNITY. It provides so much networking and information, not just for new families, but for families that have been in the community but are facing new challenges. I can’t thank you enough for providing this Symposium.”

“Thank you to all for all the hard work behind the scenes to make this possible. We are at the starting line of this journey with our two-year-old son and this was our first event. I am very thankful for all the information and support received from The Coalition for Hemophilia B family! Thank you for your kindness, dedication, and your service.”

“THANK YOU SO MUCH! We had a wonderful time seeing everyone and learning about what is happening in the hemophilia B community. Of great interest is the hope offered by emerging therapies. There is tremendous progress being made in gene therapy, and we look forward to a time when it provides a long-term solution to regular infusion. My son also enjoyed the opportunity to meet and talk to Robert Sidonio, Jr. It was a chance for him to meet one-on-one with a “source of information.” He’s considering studying biology in college next fall and this conversation couldn’t have been more timely!”

“Gatherings like this reinforce what a unique, but also very special community we are a part of. Thanks again to everyone who worked so hard to make this event what it is. For our family, it has added to both our knowledge base and our book of wonderful memories.”

“Thank you to the entire staff of the Coalition for an enriching educational and entertaining May 2022 Symposium experience. We are grateful for the opportunity to join the presentations virtually; we

learned a great deal of new information and greatly enjoyed the experience. Please continue to share the Symposium virtually so that those of us who can't travel can gain the knowledge and support we need."

"I truly did not know what to expect! Wow, we were so blessed to attend and meet some of our extended hemophilia family. The guest speakers, exhibitors, new friends & families, their stories, and strength! The Coalition for Hemophilia B is a wealth of knowledge. I met so many amazing people I now call friends, blessed is an understatement. I will forever be grateful."

"So it's hard to put a value on how important The Coalition for Hemophilia B is in my life. With so many programs out there, they help me focus on peace of mind, understanding, and hope, with their commitment to truly helping others not feel like they're less than a person. It's so amazing to have you all be there for us like family. Thanks, guys, for all the content like mental health, pain management with alternatives to healing, and living a confident sustainable future. I am proud to call you all brothers, sisters, mentors and leaders."

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1970

*First patients
ever receive
gene therapy*

1997


*First rFIX
products
approved
by FDA*

1999

*First gene
therapy
trial in hem B*

2018

*Late-stage
trials for
gene therapy
in hem B
underway*



EVERY STEP HAS BEEN EVOLVING THE SCIENCE OF GENE THERAPY IN HEMOPHILIA B

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NAVIGATING HEMOPHILIA B AS PARTNERS

The dynamics between partners in a relationship are an important part of health and quality of life, not only for the person with hemophilia but also for the partner. That's why during April 7-10, 2022, the Coalition hosted its first partner program called ***In This Together***.

The program featured tools for improving relationships, the key to learning each other's communication styles, morning exercise choices, rap sessions with hosts Carl and Gwyn Weixler, opportunities for interaction, and much more. We believe successful outcomes happen because of a strong support system, and we want to equip them with that moving forward.

Some featured sessions and speakers included *The Ties That Bind, Part I and II* with Natalie Sayer, *Healing*

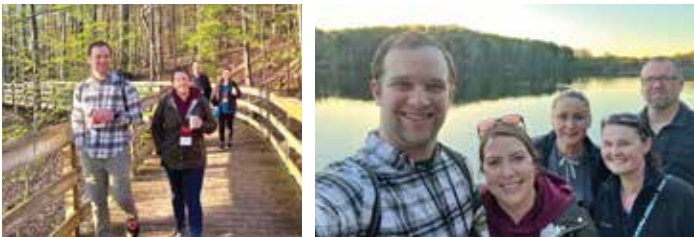


Connections: How to Improve Any Relationship with Catherine Canadeo, "Couples & Finances" with Matt Barkdall, *The Not so Newlywed Game*, men's, women's and partner breakout sessions, *Intimacy* with Karen Boyd and David Rushlow, and *Interactive Games to Deepen Understanding of Each Other* with Lee Kim. We closed the weekend with an outdoor bar-b-que dinner.

Thank you to our facilitators Carl and Gwyn Weixler, volunteers, staff, and speakers.

Many thanks to our generous sponsor, CSL Behring.

CSL Behring





COMMENTS

"Thank you for really bringing my partner and me closer. Thanks to some of the classes we had, we've really grown together in our communication and with the hemophilia community."



"The couples' retreat was a wonderful opportunity to connect with our partners as well as other couples in the Hemophilia B community. There were so many events, a variety of topics, and hands-on tools to improve our relationship. We cannot begin to express the generosity of sponsors and the organization and efforts of the Coalition. Thank you!"



"We had such a nice weekend reconnecting with each another and learning about our communication styles was life-changing. We really enjoyed getting to know other couples and how they have stayed strong together, throughout all the challenges and setbacks with hemophilia. Thank you!"



"Honestly this was an eye opener. I am a little embarrassed to say that I really did not know what my husband was going through until we had the rap sessions. Then we learned our own unique communication styles which was eye awakening. There was so much that I personally and my husband took away from this weekend. We learned new ways of communicating and we learned that it's OK to share with each other instead of trying to protect the other. We found a new support system of wonderful couples some who have been married a REALLY long, long time (which is encouraging)! Their shared experiences and wealth of knowledge was inspiring! They were also very honest and open sharing



that life is not always perfect and how we can best handle storms. And hearing all this has now helped us to be more honest and with that, we are stronger. Thank you! All the sessions were great and we are on a good path. I look forward to more programs like this."

"Having the opportunity to spend time without the kids allowed us to spend more together and time forming relationships with the other couples who face the same issues that we do."

"What a special program for my spouse and me to spend time strengthening our relationship. I appreciate connecting with others facing similar challenges and learning from each other. The variety of speakers touched on topics that are relevant and helpful. I definitely left with better tools to improve our relationship."

"The couples' weekend was excellent! It is so nice to be with others who understand and to have classes to share with others and learn. Much needed. Thank You!"

"Thank you to CSL Behring for sponsoring this event so couples within the community can attend. The topics that were discussed were just what we needed. One word to sum up the experience – phenomenal!"

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THE BEATS MUSIC PROGRAM CHANGES LIVES

BY RENAE BAKER

“I connected with my husband after he attended *The Beats Music Program* this week. His joyful and animated description of his experience made me cry. For the first time in all these years, he feels truly connected to our community, and The Beats did that. He has had moments he will never forget – playing guitar with session artists, teaching a kid a few chords last year and then getting to see that same kid play an entire song. Thank you!”

– Wife of *The Beats Music Program* participant

That was posted a week after the curtain came down on The Beats '22 held June 4–8 in Nashville, Tennessee.

I don't know who wrote that, but the way I see it, it could have been any number of people, because to be in the rooms where The Beats program happens is to feel the joy and change about which this wife writes. That is just what Kim Phelan and Wayne Cook envisioned when they set out to harness the power of music to improve the quality of life for the coalition members who





have interests not addressed in other programs. There is actual science behind how making music together creates cooperation, coordination, and empathy among people that may be felt for decades! Perhaps most telling are the faces of the returning Beats participants, and the gratification they feel by taking their fledgling blood brothers and sisters under their wings and welcoming them into a world of musical camaraderie.

One new participant had never sung in public before. She felt so supported by her bleeder bandmates that she stepped out of her safety zone and sang two additional songs with them, on stage, at the closing concert. She returned in '22 with a musical confidence far beyond what I saw before the mountaintop moments she experienced during The Beats '21. At the closing concert, she wowed us with the solo, *Crazy* and fronting the jam band with Rhiannon.

"I've developed a lot of confidence singing in front of people from The Beats program" she shares. "The program inspires me each day, because I was shown I have a talent I didn't know was there before!"

Committee member and participant, Shelby Smoak, highlights how The Beats program has the potential to do more than you might expect when it comes to your musical aspirations: "The Beats program gave me a drummer for my last record, a mix engineer for that same record, an amazing guitar player for future recordings, a drummer for live shows and recordings, and friends for life – how could you ever 'beat' that?" he asks.

Three-time returnee, Joe, experienced an organically inspired performance accident in '22 and found himself repeating, "Just go with it!" Go with it he did, finding his best song, the best way



to do it, and helping others do the same, whether by giving feedback or a helping hand getting onto the stage.

The Beats program usually begins on a Wednesday and ends on a Saturday, with a galvanizing trip to the Grand Ole Opry. Attendees are usually taken to give them inspiration before their final performance.

Our friend Dr. Robert Lawrence Friedman presented his popular session, *What's So Funny? The Rhythm Is Going to Get You!*, which combines laughter and rhythm, setting the tone for a fun, educational, and musical time for the next few days.

Coalition President, Wayne Cook, who seems perennially poised to speak eloquently, gave a warm welcome during our first of four delicious and healthy dinners. Then we shuttled to the Opry.

The next morning brought fellowship around the breakfast table, followed by The Beats favorite, Elec Simon, leading us in his motivational drum circle. Elec has become so revered in The Beats program that he now has a presence at Symposium for the tweens and teens.

The next three days saw a lineup of presentations that helped us grow in ways that encompassed mental and physical health, exploring music while living with hemophilia B. Anna Moss led a workshop in accomplishing big goals by tending to the small steps. She followed that up with a talk on the benefits of gratitude.

Our brother from the community, Max Feinstein, threw himself into the program, heart and soul, as always. He led workshops in time signatures, singing, and playing solo and sported his reliable intense focus and enthusiasm for musical communion, whether through sharing his knowledge, experience, original music, or



the Beats
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 COMMON CONNECTIONS, THE PATH TO RESILIENCE: FINDING CONFIDENCE IN YOURSELF AND MANAGING YOUR CONDITION

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performing with his Coalition friends.

Natalie Kukla presented her *Stop Waiting to Thrive* workshop, Wayne led a *Learning to Count* workshop and I led the *Befriending the Butterflies* workshop on harnessing the power of performance nerves.

Professional country musician, Trevor Martin, returned to The Beats for the second year in a row. He presented a couple of his original songs and discussed his process for song writing. Trevor is a perfect example of why The Beats music program is so important. You see, Trevor has hemophilia. His first trip to Nashville wasn't to make it in the music industry. He was flown there from Bowling Green, Kentucky at two days old, bleeding uncontrollably. Growing up with the physical restrictions of the bleeding disorder, he turned to music.

Still considered a fresh face on the country music scene, Trevor has been "making it," as a singer in some of the most prestigious venues in Nashville, on CMT (Country Music Television), and as an actor in film and television. He is an inspiring blood brother and all-round great guy.

With participants delving deeper and deeper into music, and some choosing to pursue music professionally, we held a panel discussion on touring and how to avoid pitfalls along the way. The discussion was billed as *Playing to the Limits: Music Performance and Self Care*. I moderated the panel which consisted of our homegrown, seasoned professionals, Wayne Cook, Shelby Smoak and Max Feinstein.

An annual favorite outing during the program is the home recording studio tour at Adam Smith's Recording Studio. Adam has outdone himself each year of The Beats, and during this year's experience in his studio,



he engineered the visiting participants performing a song, featuring our young Andrew, which Adam immediately produced. For the closing concert, we got to hear the world premiere of The Beat's 2022 *Happy!* It was truly special and a gift that will keep on giving.

A particularly touching moment happened via a Zoom presentation. Our own, Rick Starks, who had planned to join us for the program, caught the bug. Not showbiz; COVID-19. A mild case, but that meant he couldn't join us. He was prepared to not only participate as a student, but to present his story as an ambassador. We were disappointed, but we all gathered around a big monitor as he showed up, larger than life, on screen, to tell his story.

As Rick wrapped up, telling us how special this program and the Coalition are to him, a room full of his blood brothers and sisters held their phone lights up over their heads, marking the moment sacred.

In the middle of these illuminating workshops and discussions were the hands-on musical instruction.

This year, The Beats welcomed several young Coalition musicians, including 13–17-year-olds. One 13-year-old stretched his wings beyond his trumpet playing and took on piano during the program. For the concert, he performed *The Entertainer* and asked me to sing *Somewhere Over the Rainbow* so he could accompany me, which I considered a great compliment. He said his favorite part was hanging out and making new friends. He was impressed with the connections he made during his time in Nashville.

The concert began with our adopted Coalition member, "Ivory" Joe Turley, singing last year's finale, *Lean on Me*, as he played piano with attendee Chris next to

him playing bongos, and instructor Rich Adams on the drum kit. We all joined in singing and clapping. As the next act, Joe, took the mic, he commented on what a great “loosen up” it was. Indeed, it was, and it contrasted nicely with Joe’s very serious rendition of *Head Full of Doubt*, accompanied on piano and harmonica by Ivory Joe.

You might remember a group of our older members and the group they called “The Old Farts Band.” Well, they renamed themselves to simply “The Group” this year, as the average age of the group dropped considerably with the addition of a very talented teen guitar player.

Attendees Nathan and Robb who got up and knocked us out singing *Folsom Prison Blues*. Nathan came with his family to last year’s Beats concert and was so inspired from the audience that he took up the guitar and joined us this year! Our jaws dropped to learn that he’d only been playing guitar for a year. He’s a natural!

There were original songs which touched us including, *Dust Devil Boy* by Bryant. There were classics performed with professional gusto, including Zack singing *Are You Sure Hank Done It This Way?* Fifteen-year-old JC played the French horn, which was another first for The Beats.

Sam impressed us with how very far he’d come with his confidence singing, *Didn’t I?* Seventeen-year-old Dylan reprised *I Feel So Close to You Right Now*. Though there isn’t room to list all the acts, each and every one of them was touching. From soloists to groups to audience sing-alongs, a special time was had by all.

Sometimes the quietest people make the biggest impressions when it’s time to step outside of the safety zone. One quiet young man, stepped up onto the stage toward the end of the show, and sang *Don’t Stop Believin’* and absolutely brought down the house with his beautiful voice and full-blown commitment to the emotion the song needed. It



was the second time in the four days that the phone lights were waved above our heads. Credit also goes to Adam Smith, who supported him and provided a safety net while accompanying him on piano.

As sometimes happens during the afterhours jam sessions, a new song is born among The Beats participants. I’m elated to say that this year’s Beats program was the incubator for another one, and we were privileged to witness the first performance of The Group’s new, *And the Beats Go On!* You can find this performance and Adam Smith’s version of *Happy!* on The Coalition for Hemophilia B’s Facebook page, and I expect you’ll see YouTube videos before long!

We want to thank CSL Behring for sponsoring at the top Impresario level sponsor, Novo Nordisk for sponsoring at the Producer level, Medexus Pharma and Sanofi for sponsoring at the Conductor level, and CVS Specialty for sponsoring at the Performer level! The growth and enrichment that are possible because of your generosity cannot be overstated!

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and our comfort in sharing it.”

“The Beats program has given me the tools to have better opportunities by giving me confidence I didn’t know I had in me. Every music coach helps us tap into so much love and passion and helps us to create a great relaxed state of mind to elevate our music skills.”

“Great times and performances all around this year! I’m so proud of all the singers and players... lots of talent, determination and soul... and all the positive support is fantastic!”

“It means the world to me. It’s a chance for us to get together and express ourselves through music. It also gives those a chance to be a part of a program that does not revolve around athletics. It opens so many doors for individuals.”

“The Beats program is a event I look forward to every year, I love seeing new faces and coming together through music and creating a special bond with my fellow hemophiliac family and learning from each other little things that help improve our craft of playing the instruments of our choice. It’s such a pleasure to see the enthusiasm each year that this event provides by sharing a common love for each other through music and hemophilia doesn’t place any limitation to participate in this event.”

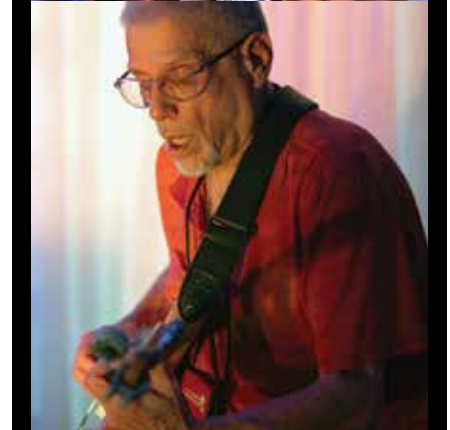
COMMENTS

“This program has given me and many other people with bleeding disorders another type of voice to say the important things inside our hearts that we live with every day.”

“This is a truly fantastic program! There is no other program like it! The Beats program is a very original and organized event for people of all ages and all talents. It brings people close together through the form of what they love and that is music. Thank you for doing this!”

“The Beats Program is one of the highlights of my year. It’s a chance to meet up with old friends, make new ones, and play a whole lot of music with some really talented people. I feel like I come away from it a better musician with a tighter connection the hemophilia B community. Thanks to the Coalition for putting it on and to the sponsors who make it possible.”

“The Beats program allowed me to meet still more blood brothers and sisters as well as some caregivers. The Coalition excels in bringing people together in positive and enjoyable settings. Music is a common source of comfort in our community. While teaching us in specific areas the Coalition fosters exchange of knowledge and stories which advance both our musical skill



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GETTING BRAVE IN THE WAVES: MY WEEK AT GEN IX ADVOCACY

BY RICK STARKS

San Diego...what a location for Generation IX Advocacy 2022 held April 14-17! It was so exciting to finally reunite with old friends and new acquaintances, in person, after three years.



The Coalition for Hemophilia B and Gutmonkey, as always, chose a prime location: Camp Surf, a YMCA camp just south of San Diego. It is located right on the beach, with spectacular views and amazing sunsets. It was a fitting backdrop for this year's Gen IX Advocacy theme: WAVES!

The theme meant not just waves in the ocean, but also riding the waves of curiosity, hope, and connections. It meant understanding the tides of the bleeding disorders community throughout history and the present. It meant connecting with our dreams, our strengths, and our future as advocates for positive change.

This year's fantastic program, as in years past, focused on helping adults with hemophilia B escape the norms of the everyday and engage with others to explore and define the meaning of advocacy. That sometimes means challenging yourself emotionally and physically, and boy did we! We explored how we can listen, play, and collaborate amidst the changing tides of policy, healthcare, insurance, access and more. The next few days were spent with much laughter, along with opportunities to learn about the ocean, tides, and waves.

The Generation IX Project, launched in 2014 by The Coalition for Hemophilia B, Medexus Pharma, and Gutmonkey, is a unique outdoor educational program designed specifically for and by those living with hemophilia B. The highlight for many was learning to surf and boogie board. At this session, I was

most touched by everyone's desire to help each other,



going out of the way to make others feel comfortable and accepted. There was true camaraderie amongst the group. All too soon, the weekend was over. After Tai Chi, we departed on Easter morning and were surprised to be sent off by the largest Easter bunny ever...Pat (Big Dog) Torrey!

Saying goodbye to brothers and sisters, new and old, was the most difficult part. I am looking forward to getting together again next year at another amazing Gen IX event.

A special Thank you to our Sponsor MEDEXUS PHARMA.



COMMENTS

"GEN IX means family, community and support. This program has been an essential foundation for me as a community member, advocate and health professional. Without opportunities, that programs like Gen IX provide, I most likely would not be so invested in my community and actively building a career with the intention to help people with rare diseases."

"I want to thank everyone again for arranging and coordinating the awesome event at Camp Surf this past week. I appreciated the opportunity to see members of the community, The Coalition, and GutMonkey who I haven't seen in person in close to three years.



It was nice to have a return to normalcy after so long in the pandemic and I look forward to more events and opportunities in the future."

"It's been a great experience! The staff and community have been extremely helpful!" "Fun bonding again after two years, with old friends and making new ones. I enjoyed the exciting beach time, learning to boogie board and surf! It was a great opportunity to learn about hemophilia and how to advocate!"

"Each Generation IX program is uniquely designed to meet leadership and advocacy skills to meet the hemophilia B community. I have attended several Gen IX events in person and Virtually. I can say proudly that Gen IX activities and lessons helped form me into a better person and leader. Thanks to CHB and Medexus for their generous sponsorship of this wonderful program."

"GenIX is connection, understanding of one another, growth, and family. These are the things that bring me back every opportunity I get! GenIX Leadership was amazing, as it was so relevant focusing on regrowth and what that means for us, as the world is going through its own regrowth after a year of covid."

"To me, Gen IX is not only a way for me to connect with others with hemophilia B, but also an opportunity to step back, look deeply into my life and find ways to improve it and then explore how to carry that into the larger hemophilia community."

"Gen IX was fun, interactive and a great way to connect with other people with hemophilia B! Gutmonkey's programming is always filled with surprises and depth that has me unpacking it long after the program ends."

"Gen IX means family to me". "I thought this program was very interesting and important because we came together and connected". "It was amazing and fun. I loved it"

"There are so many things inspiring about this weekend. From the facilities and environment to the programming and activities. What stands out the most is the people you get to share the experience with, who have become, I hope, lifelong friends."

"My favorite things were learning to surf, laying out on the beach, watching the sunrise and sunsets. The most inspirational thing was watching everyone push their limits!"

TO VIEW MORE PHOTOS VISIT OUR FACEBOOK PAGE UNDER "ALBUMS" OR OUR WEBSITE: HEMOB.ORG



SPRING INTO WELLNESS

BY GLENN MONES

On Saturday afternoons of April 2nd, 9th, 23rd, and 30th, we presented our latest *Spring into Wellness* series. Each program in the series is designed to introduce participants to a variety of health and wellness exercise and topics.

We begin each series with wonderful exercise movement from Tai Chi to learning new forms of yoga, etc. We love to introduce you to new exercise experiences that will put a spring into your step. We also introduce some pretty cool ways of cooking and some special and some secret healthy recipes demonstrated this series by the Coalition's President Wayne Cook.

We have a special guest speaker at every program who are experts in their fields. Speakers and topics included:

- *Living Intentionally in Terms of Your Behavior, Thoughts and Spirit*" with Debbie de la Riva LPC
- *Finding Happiness with Stress Management Tools* with Catherine Canadeo, CHHC
- *The Power of Laughter and Play* with Dr. Robert Friedman
- *Cooking Healthy and Delicious Meals for Better Living* with Wayne Cook
- And several other incredible physical fitness sessions!

The Coalition for Hemophilia B believes that health and wellness go beyond learning about hemophilia and extends to a variety of topics that look at all aspects of a member's wholeness and well-being.

Thank you again to our sponsor Sanofi and to our awesome speakers!

LET'S GET MOVING! • • •

Spring seems to breathe new life into us. It cleanses our spirit and our hearts. We feel a renewal of our own energies emerging.

PUT A LITTLE SPRING IN YOUR STEP WITH THIS WELLNESS SERIES!

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JOIN US FOR AN HOUR OF HEALTH AND WELLNESS!

FUN FOR ALL AGES. 3-100

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Catherine Canadeo



Debbie de la Riva



Robert Friedman



Wayne Cook

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HOW DOES
THIS FACTOR IN?

To find out about a prescription
option, talk to your doctor or visit
[OnceWeeklyForHemophiliaB.com](https://www.OnceWeeklyForHemophiliaB.com)

YOU TURNED 26 NOW WHAT?

BY SAM LATHROP

If like me, you have recently turned 26, you may have approached a milestone “transition to adulthood” birthday with a variety of emotions. Fear, anxiety, trepidation, reluctance, and a realization that suddenly, you will become the “master of your own fate.”

As a bleeder, in your first 26 years of life you are reliant on your caregivers in a way that people without a chronic illness can't comprehend. This isn't the fault of the “chronically well” but of the way the healthcare system is set up, especially for people with “rare” conditions like hemophilia B. As we learn how to navigate this system, we learn the things to say and ways to say them to help ensure our access to the best treatment available.

Long before you are 26, you may even find yourself in the role of educator as you're speaking with representatives at your insurance provider. This is something I learned by watching my folks do it on more occasions than can be counted on both hands. The system has rules that can be rigid, but there is also a degree of flexibility, and we have rights. When we're not getting what we need, there are things we can do to change the situation. My major focus in this article is to describe, based on my personal experience, some of the things you'll need to know when transitioning off the insurance of your parents or other caregiver once you turn 26.

First, and most importantly – don't panic! So many people are turning 26 as you are reading this article; and of course a portion of them have a chronic illness, even hemophilia B. This means that you are absolutely not alone in whatever you might be feeling. This also means that there are people that you can reach out to for help if you need any – and hopefully this article is a good starting place.

The unfortunate truth is that you will be dealing with incredibly frustrating situations that are sometimes out of your control. You may have to deal with multiple people at your specialty pharmacy and at your insurance company. Do not be discouraged if one representative doesn't know anything about



your specific situation. Hopefully there should be enough notes in your file, so the rep has at least some background on your history..

Keep your own notes and be prepared to “fill in the blanks.” Take as much time as you need to explain what you need. Be as civil and patient with them as you can but be forthright and unapologetic at the same time. You are your own advocate and a rep's role in assisting you is a big part of their actual job. There will be instances where you will need to talk to a supervisor who knows more about your current insurance/ pharmacy situation than the rep you were originally connected with.

Be confident in your ask and know that you are not creating any undue hassle for them. This is an instance where it is okay to be a little bit “selfish.” You are asking for your life-saving medicine! Be prepared to be a little forceful if your needs are not being met. Receiving your medicine is your primary goal here and a big reason behind insurance.

Now this brings us to talk about insurance. Many people receive insurance from their employer which is usually ideal. ACA plans also known as Obamacare is another option but the quality and availability of the plans vary from state to state. Some in the community find they are able to obtain Medicaid, which is a program funded by the Federal government but administered by the individual states. Eligibility is mostly based on income (it has to be very low), and the threshold varies from state to state. Medicaid is also an option, but again, eligibility varies from state to state and the income requirement is very low.

The social worker at your treatment center or someone at your local chapter may be able to help you determine which options are best for you. Your national

organizations, including The Coalition for Hemophilia B, also have information and other resources that may be helpful.

Do not feel shame for using government programs if you need them. A mark of a civilization is its ability to care for the people with the greatest needs and WE need some Factor IX in us to be able to contribute to that civilization. If anyone tries to tell you otherwise – ignore the ignorance. Take advantage of programs that are aid and other programs available in your state. If you need help applying, there are plenty of people who can assist you – just ask! Just be patient and keep your treatment center’s information and proof of diagnosis handy as you will need them again and again. Processing time varies but follow up if you do not hear back within a reasonable time.

In addition to needing a treatment center (HTC) you will need a specialty pharmacy that will actually provide you with your product. You can’t just get factor at the corner Walgreens. Depending on your coverage and your state, you may have only one option or you may have several, so be sure to ask and make sure the one you choose is covered by your insurance, so you don’t receive any “surprise bills.” Often, your HTC will have their own specialty pharmacy that can dispense your medication. If they do, this may be a good option to consider as it also helps fund enhanced services at the HTC. Private options may also be available, and many of

these companies support programs and services in the community.

Your HTC is required by law to provide you with information about your pharmacy options. Your specialty pharmacy should also provide you with ancillary items such as gauze pads, syringes, and butterflies. You can ask for them every time. It’s good to have some extra supplies as backups for vacations or whatnot. It is 100% legal to do this and you shouldn’t feel bad about asking for more gauze or needles. To complete the package, be sure to ask your pharmacy for a new sharps container (get a giant one, trust me). Hopefully you’ve still got the box from your last one, but if not, your pharmacy can also take care of it for you. They’ll either send you a box or ask you to drop it off at the HTC.

There you have it! You must do everything you can to advocate for yourself, especially once you are officially in control of your hemophilia. I hope my article has helped guide you! Hopefully, after this process, you will have your first shipment of factor as an “adult.” From start to finish, it is in your hands now. Be confident in your ability to talk to health professionals. You have survived things some of these people can’t imagine – and you will continue to survive them! You have the tools to do it! And remember – wherever you go, whoever you are at the time, you will always be part of the Bleeder family.



“
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IMPORTANT THINGS YOU
CAN DO ON THE EARTH
IS TO LET PEOPLE KNOW
THEY ARE NOT ALONE.



SHANNON L. ALDER

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

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

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BY GLENN MONES

ADVOCACY NEWS: COST OF ACA PLANS MAY INCREASE FOR THOSE AT HIGHER INCOME

An important feature of the Affordable Care Act (ACA or Obamacare) subsidies made available to Americans opting for the plans. The impact of these subsidies has varied from state to state depending upon how the plans have been implemented in each state. However, the amount of subsidy is generally keyed to income, with those above four times the Federal Poverty Level (FPL) receiving substantially less or even no assistance.

That was the case until the passage by Congress of the American Rescue Plan Act (ARPA), part of the many bills designed to address the cost of the COVID crisis. ARPA expanded subsidy eligibility, now capping what people

with higher incomes pay for a silver plan premium at 8.5% of their income. However, ARPA is due to expire at the end of the year, again making ACA plans less “affordable” for many Americans. There is strong support for extending the subsidies at least for another year.

You can learn more about this issue in a post from the Kaiser Family Foundation at *Falling off the Subsidy Cliff: How ACA Premiums Would Change for People Losing Rescue Plan Subsidies* | KFF. The Coalition for Hemophilia B will also provide additional information as it becomes available.

WASHINGTON DAYS AND BEYOND: EFFORTS TO ELIMINATE ACCUMULATOR ADJUSTERS MOVE FORWARD AT THE FEDERAL AND STATE LEVELS

The Coalition for Hemophilia B has been working with the broader bleeding disorder community and with a range of other healthcare advocacy groups to eliminate the use of accumulator adjuster programs by insurers. This issue was front and center at NHF's Washington Days, held virtually.

Accumulator adjuster programs are used with increasing frequency by insurance companies to force patients to pay more for their coverage. These companies do not allow those who receive copay assistance from pharmaceutical companies, nonprofit organizations and other entities to count what they receive against their out of pocket maximum. This can cost a family or individual thousands of dollars in additional costs. To address this problem, members of the House of Representatives have introduced a piece of legislation called the Help Ensure Lower

Patient (HELP) Copays Act or HR 5801. Because of the community's efforts, what started as 8 original cosponsors has now grown to 33. Additional sponsors are still needed however, and a companion bill in the Senate is also needed.

Although the problem will ultimately best be solved by this federal approach, chapters and patient groups at the state level have been working with their state legislatures to provide an interim fix. We recently reported that the State of Washington has become the 13th state to pass legislation preventing insurers from instituting accumulator adjuster programs. This has the effect of not only protecting patients in those states without having to wait as long, but also adds momentum to the national effort. The Coalition for Hemophilia B will continue to provide the community with updates on this and other important issues.

EMERGING THERAPIES

BY DR. DAVID CLARK

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

A number of the studies described below were presented at the annual meetings of the European Association for Haemophilia and Allied Disorders (EAHAD), February 2–4, 2022, the World Federation of Hemophilia (WFH), May 8–12, 2022, and the American Society of Gene + Cell Therapy (ASGCT), May 16–19, 2022. Abstracts (summaries) of the studies are available on the websites of the three organizations.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using. The improvements include longer half-lives and delivery by subcutaneous injection.

What's Happening at Catalyst Biosciences?

5/23/22 Catalyst Biosciences has been developing several new products for hemophilia treatment. They include a higher-activity longer-lasting subcutaneous variant factor IX in Phase II studies, a higher-activity longer-lasting subcutaneous factor VIIa in Phase III studies for treatment of inhibitor patients, and a gene therapy based on their variant factor IX. The products have been progressing well, giving promising results. However, in November 2021, they announced that they were discontinuing work on their hemophilia products in order to focus on their complement therapeutics. The complement system is part of the immune system. Then in February, they announced that they had hired a financial advisor to help them sell off their hemophilia assets and to help them in exploring "strategic alternatives."

Well, now it may be that hemophilia actually is their strategic alternative. Catalyst announced that they have sold their complement portfolio to Vertex Pharmaceuticals. They don't say anything specifically in their press release about a decision on their hemophilia products, but in the "About Catalyst Biosciences" section that is a normal part of business press releases they talk about their hemophilia products as though they are still under development. Stay tuned! [Catalyst press releases 11/12/21, 2/17/22 and 5/23/22]



HEMA Biologics Reports on Studies of Sevenfact

2/3/22 and 5/10/22 HEMA



Biologics has developed a new activated factor VII product (FVIIa; Sevenfact) for treatment of bleeding in patients with hemophilia A or B and inhibitors, which was licensed by FDA in 2020. They presented papers at both EAHAD and WFH reporting on results for pain relief from their Phase III studies. They found that pain from a bleeding episode consistently decreased over the 24 hours after Sevenfact infusion. The median time to pain relief was three hours in older subjects (≥ 12 years of age) and five hours in younger subjects (< 12 years). They speculate that the longer time in younger patients may be due to the difficulty in assessing pain in that group. [EAHAD abstract PO099 and WFH abstract PP-30 (1159191)]

At WFH, HEMA also reported on the safety of Sevenfact. Over 2674 infusions in 67 subjects, 115 adverse events were recorded but only eight were considered treatment-related. These included four cases of infusion site discomfort, two cases of infusion site hematomas, one case of dizziness and one case of increased body temperature. No instances of thrombosis and no sensitivity reactions or inhibitors to Sevenfact were seen. [WFH abstract LR-01.02 (1159194)]

4/27/22 Sevenfact is currently only approved for treating adolescents and adults older than 12 years of age. They recently published the results of a study of safety and efficacy in children under twelve. They found safe and effective treatment and control of bleeding with 97 – 98% treatment success after 24 hours, depending on dose. [Pipe SW et al., Haemophilia, online ahead of print 4/27/22]

BeneFix Actually Has a Longer Half-Life

4/14/22 BeneFix from Pfizer was the first recombinant factor IX product licensed by FDA.



When it was approved in 1997, there was not as much focus on the half-lives of products as there is today. The developers did a more limited pharmacokinetic study (a study to determine half-life and clearance of a drug), using only a limited number of data points for each patient. They found average half-lives as shown in the table below. Since the results agreed well with the generally-accepted half-life value of plasma-derived factor IX, 18 – 24 hours, most people assumed they were correct. These are the values shown in the tables in the direction insert, the prescribing information leaflet that comes with every vial of BeneFix.

A few years ago, however, Pfizer did a study that showed that patients could be treated with a once-weekly dose of 100 IU/kg, more like the dosage patterns for the extended half-life factor IX products. Physicians have also seen other evidence that suggests that the half-life of BeneFix might be longer than originally thought. Therefore, a group of researchers from France and Canada did a study to look at the half-life using more extensive sampling and advanced statistical analysis. Their results showed significantly higher half-lives as shown in the table. They also found that after 72 hours, the maximum time observed in the original BeneFix study, factor IX levels reach a plateau with values between 2 and 5% of normal lasting for several days.

These results suggest that the half-life of BeneFix is much longer than previously known, which better matches clinical experience. Note that while these new results have been published in a peer-reviewed journal, they have not been submitted to or approved by FDA. [Tardy B et al., Haemophilia, online ahead of print 4/14/22]

Original and Revised Average Half-Life of BeneFix

Age, years	Average Half-Life Shown in Direction Insert, hours	Average Half-Life from Tardy et al. Study, hours
<2	15.6	36.9 (ages 0.8–12 years)
2 – <6	16.7	
6 – <12	16.3	
>12	23.1	49.9

REBALANCING AGENTS

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

Novo Nordisk Provides Updates on Concizumab

2/3/22 Novo Nordisk is developing concizumab, a monoclonal antibody that binds to and inhibits the anticoagulant tissue factor pathway inhibitor (TFPI).



This reduces the amount of anti-clotting activity in the coagulation system and helps to rebalance the system. Last year their Phase III clinical study was placed on hold after three subjects developed thrombotic complications after being given factor products to treat breakthrough bleeds.

One of the issues for all rebalancing agents is how to treat breakthrough bleeds, that is, bleeds that occur even though one is taking one of these agents. Several organizations developing rebalancing agents have seen problems with thrombosis (unwanted, dangerous clotting) after treating breakthrough bleeds with factor or bypassing agents.

At EAHAD, Novo presented two papers discussing treatment of breakthrough bleeds and modifications to the dosing regimen for their Phase III study to reduce the risk of thrombosis. [EAHAD abstracts PO005 and PO091]

Novo also presented results from several subjects in their Phase II studies who underwent surgeries or diagnostic procedures while on concizumab. Most of the surgeries went well with no evidence of thrombosis and only mild/moderate bleeding. However, one subject experienced severe bleeding and another experienced later bleeding after a difficult dental extraction. It may be that concizumab and other rebalancing agents could provide adequate bleed control in everyday life but need additional factor coverage during events like surgery. [EAHAD abstracts PO095 and PO129]

4/16/22 The first published paper on concizumab dates to 2012. A researcher in Italy recently published a review of all the 40+ papers that have been published so far. [Pasca S, J Blood Med, 13:191–199, 2022]

Pfizer Gives Updates on Marstacimab

3/16/22 Pfizer is developing marstacimab, another monoclonal antibody that binds to and inhibits the anticoagulant tissue factor pathway inhibitor (TFPI). This reduces the amount of anti-clotting activity in the coagulation system and helps to rebalance the system.



As above for concizumab, treating breakthrough bleeds is an issue. It is especially a question for inhibitor patients who are usually treated with either activated factor VII (FVIIa; NovoSeven or Sevenfact) or activated prothrombin complex (aPCC; FEIBA). Both contain activated clotting factors that could lead to thrombosis (unwanted clotting) without the control provided by the anticoagulants.

Pfizer performed both laboratory (in vitro) and mouse (in vivo) experiments looking at thrombin generation, a marker for clotting. In normal and hemophilic (both A and B) plasmas they showed that marstacimab plus either FVIIa or aPCC did not lead to excessive thrombin generation. They confirmed these findings in a mouse bleeding model. This gives more confidence that treating breakthrough bleeds in inhibitor patients on marstacimab may not lead to dangerous thrombosis. [Pittman DD et al., Res Pract Thromb Haemost, online ahead of print 3/16/22]

2/3/22 At EAHAD, Pfizer also announced that they are planning a Phase III study of marstacimab in children ages 1 to <18 years with either hemophilia A or B, with or without inhibitors. The currently ongoing Phase III study of marstacimab is limited to subjects 18 years or older. The new study is scheduled to start in November 2022. [EAHAD abstract PO122]

Sanofi Reports Updates for Fitusiran Development



2/3/22 and 5/10/22 Sanofi is developing fitusiran, a drug that reduces the amount of the anticoagulant antithrombin being made by the body. Lowering antithrombin levels appears to rebalance the clotting system. Fitusiran is a monthly subcutaneous injection. At the EAHAD and WFH meetings, they presented results of studies on the improvements in Health-Related Quality of Life (HRQoL) of patients receiving fitusiran in their clinical studies. Patients with inhibitors (EAHAD) or without inhibitors (WFH), all showed improvement in all aspects of HRQoL while taking fitusiran. [EAHAD abstract PO067 and WFH abstract LR-10.02 (1155651)]

Another study at EAHAD showed that the reduced antithrombin levels in the blood of patients on fitusiran do not affect routine factor VIII or IX assays. [EAHAD abstract PO007]

GENE THERAPY

Gene therapy is the process of inserting new, normal factor IX genes into the body to allow it to produce its own normal factor IX.

China's Belief BioMed Announces Results of Phase I Gene Therapy Study

5/19/22 Belief BioMed is developing BBM-H901, an AAV-based gene therapy for hemophilia B using the Padua factor IX variant. They recently published a report on the results of their Phase I study. In ten subjects, they found an average factor IX level of 36.9%, 58 weeks after treatment. There were no serious adverse events and no evidence of inhibitor formation. Two of the subjects developed liver inflammation, which reduced their factor IX production to 7% and 12%, respectively. [Xue F et al., Lancet Haematol., online ahead of print 5/19/22]

Catalyst Presents on Gene Therapy




5/17/22 Catalyst Biosciences is developing a gene therapy for hemophilia B using their high-potency factor IX variant. The variant, dalcinonacog alfa or Dalca, is also being developed as a stand-alone factor IX concentrate. Although Catalyst Biosciences announced last November that they were leaving the hemophilia field, more recent developments suggest that may not happen – see the first story above. At ASGCT, they presented results from a study of two different modified AAV vectors in mice and in non-human primates, rhesus macaques. They found that modifying the AAV vector can make as much as a ten-fold difference in factor IX production. Their goal in using modified vectors and a high-potency factor IX gene is to lower the dose of vector. This could substantially reduce the cost, lower the potential liver toxicity and decrease the risk of adverse immune reactions for the treatment. [ASGCT abstract 699]

FDA Accepts CSL/ uniQure License Application for Gene Therapy



5/24/22 FDA accepted CSL Behring's Biological License Application (BLA) for its hemophilia B gene therapy etranacogene dezaparvovec. This is the first hemophilia B gene therapy to apply for licensure in the U.S. The product was originally developed by uniQure, who will manufacture the product for CSL. CSL had previously submitted a Marketing Authorization Application (MAA) to the European Union (EU) in March. FDA has accepted the BLA for priority review, which means that it should get a more rapid consideration for licensure. [uniQure press release 5/24/22]



Tell us about
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in your life.

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

Together, they're the Heroines of Hemophilia B.

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2/3/22 At EAHAD, CSL and uniQure presented the latest data from their Phase III study of etranacogene dezaparvovec. In 54 adult male subjects with severe or moderately severe (<2% of normal FIX) hemophilia B, they found an average factor IX level of 39.0% after 6 months and 36.9% after 18 months. The study included subjects with pre-existing antibodies (31 subjects) to the AAV5 vector, and no correlation between subsequent factor production and antibody levels was found, except in one patient with a very high antibody level. All except that patient were able to discontinue prophylaxis. During the six-month lead-in period when all subjects were on routine prophylaxis, they found an annualized bleed rate (ABR) of 4.18. That was reduced to an ABR of 1.51 after treatment. The subjects' factor IX consumption decreased from an average of 257,000 IU/year during prophylaxis to about 8500 IU/year after treatment. After treatment, 37 subjects experienced 92 treatment-related adverse events (AEs), 80% of which were mild. The only serious AE seen was determined not to be related to the treatment. [EAHAD abstract PO143]

At EAHAD, CSL also presented data from three subjects in their Phase II study who had been followed for 2.5 years. The subjects, who had $\leq 1\%$ factor IX levels before treatment, increased to an average of 31% after six weeks. At 2.5 years, they had levels of 37.1%, 54.4% and 58.6%, respectively. [EAHAD abstract PO098]

5/17/22 At ASGCT, the researchers presented data on Health-Related Quality of Life improvements for the subjects in the study. They found statistically-significant improvements in all aspects. [ASGCT abstract 1192]

Freeline Reports on Phase IIb Study

3/31/22 Freeline Therapeutics is developing a gene therapy treatment for hemophilia B called FLT180a that uses an AAV vector with a Padua variant factor IX gene. They recently completed a Phase I/IIa dose ranging study and have selected a vector dose that should put subjects in the normal range of factor IX expression. They are currently starting a Phase IIb dose confirmation study to verify that selection. They enrolled the first patient in March and expect to complete enrollment in the first half of 2022. If all goes well, they expect to begin a Phase III study in the first half of 2023. [Freeline press release 3/31/22]



Pfizer Reports on Factor IX Assays for Gene Therapy



5/10/22 Pfizer is also developing a gene therapy treatment for hemophilia B. One of the important aspects for comparing gene therapies is the factor IX level achieved by the patient. However, the high-potency factor IX variants used in the gene therapies can give different results in different tests. At WFH, Pfizer presented results from subjects in their Phase I/IIa study showing that different types of assays can give quite different values, in one case a six-fold difference in factor IX levels. This shows the need to standardize factor assays and potentially develop a more consistent factor IX assay that can be used with gene therapy patients. [WFH abstract LR-09.03]

Takeda's Gene Therapy Plans



5/4/22 Baxter was once developing a gene therapy treatment for hemophilia B. When they spun off their hemophilia business as Baxalta, the gene therapy project went with it. However, when Shire bought Baxalta, they discontinued the project because the product did not give consistent results from patient to patient. One of the important principles in the pharmaceutical industry is that a product should have a predictable, reproducible effect in patients. Thus, when Takeda bought Shire, it came with no ongoing hemophilia B gene therapy project.

Note that it is not only Baxter/Baxalta's gene therapy treatment that has this problem; they all do. For instance, a gene therapy treatment might produce factor IX levels of 35% in one patient but 55% in another. How do you market a product when you can't tell the patient ahead of time what his response will be? How is a payer going to react to that? We'll find out, since the other companies developing gene therapies for hemophilia B are still going ahead full steam.

Meanwhile, Takeda has decided to get serious about gene therapy. Since early 2020, they have spent billions of dollars buying up small biotech companies that have expertise in various aspects of gene therapy and related fields. However, Takeda is a large company with interests in many medical fields, so we don't know yet whether any of these new resources will be targeted at hemophilia.

One of the most interesting moves is a \$2 billion deal with Code Biotherapeutics to develop virus-free gene therapy treatments. A virus-free treatment could be an important product. It would eliminate the issues with immune rejection of treatments and allow repeated dosing. [Fierce Biotech article, 5/4/22]

BONES AND JOINTS AND PAIN, OH MY!

BY DR. DAVID CLARK

Not that long ago, the main research topic in hemophilia was product safety. Without treatment, many people with hemophilia did not live beyond their teens. That was overcome when clotting factor concentrates purified from human plasma were developed. They opened up a real future for patients, for awhile... Then in the 1980s people with hemophilia

started coming down with unusual diseases. That turned out to be AIDS, caused by the HIV virus, which contaminated many plasma donation pools. Over ten thousand hemophilia patients and family members died of AIDS. That tragedy sparked a push to develop safer plasma-derived products and then recombinant products not made from plasma. Finally, we have safe life-giving products, and hemophilia patients live normal lifespans.

So, what's left to do? A lot! When you get to my age, you realize that living a long life is not necessarily the main goal – you want quality of life (QoL), too. QoL has become the focus of much of today's research. Just getting you to old age is not enough. We aren't really successful just because we can keep you alive even though you're curled up in a wheelchair unable to walk. We want you running and jumping and enjoying yourself.

One of the most important aspects of QoL is being mobile. Bone and joint health are crucial. We thought that once everyone was on prophylaxis with safe easier-to-use factor products, all the joint problems would go away. Unfortunately, that hasn't been the case. Three recent papers presented at the annual meeting of the European Association for Haemophilia and Allied Disorders (EAHAD), Feb. 2 – 4, 2022, showed that joint damage is still occurring: 1) in about one-third of patients on prophylaxis, 2) even in the absence of apparent joint bleeds and 3) even in mild and moderate patients despite a low frequency of bleeds. [EAHAD abstracts PO083, PO084 and PO085]

What researchers think is happening is called subclinical bleeding, or microbleeds. "Subclinical" means that the



bleeding is not apparent to the patient or physician. The bleeds are so small that there is no pain and no feeling of having a bleed. However, there is still enough blood getting into the joint to cause trouble. A paper at the World Federation of Hemophilia (WFH) annual meeting, May 8–12, 2022, describes an MRI study of hemophilic joints that suggests that

about 20% of joints have damage due to microbleeds, without any outward signs of ever having had a joint bleed. [WFH abstract PP-14 (1157875)]

Joints are lubricated with synovial fluid. Earlier on, the thought was that maybe the blood leaking into a joint just diluted the synovial fluid so much that it lost its slipperiness. That could cause physical damage by wearing away the cartilage in the joint, similarly to what would happen if you put water in your car instead of oil. Today, after much more research, we don't think joint damage has a physical cause like that; it's more due to changes in the biochemistry going on inside the joint because of molecules coming in with the blood.

Joint health is also important for women who are carriers or who have hemophilia. They are often developing joint damage without knowing it. Women with severe hemophilia usually know they are bleeding, but carriers and those with mild/moderate disease rarely have noticeable bleeds, yet often develop joint damage, apparently from microbleeds. This is one major reason women should try to get treated. Although you might be living a seemingly normal life, you may be piling up joint damage that will affect you in later. [Citla-Sridhar D et al., Haemophilia, online ahead of print 4/13/22]

Joints are not the only issue. In fact, recent research has shown that joint problems tend to go along with low bone density. People with hemophilia tend to develop low bone mass or bone density, known as osteoporosis, which can lead to fractures. This has historically been thought to be caused by prolonged immobility, repeated joint bleeds and lower physical activity levels. More recently, we have seen that prophylactic, but not

on-demand, treatment with clotting factors improves bone density. Therefore, deficiencies of either factor VIII or IX may be involved, not just low physical activity. Factor VIII, which is deficient in people with hemophilia A, has definitely been shown to be involved, but the situation for factor IX and hemophilia B is less clear and needs more research.

Women who are either carriers or have hemophilia may be even more at risk, since women tend to lose bone density with age anyway. Again, more reason for them to try to get treated. Even if they don't have significant bleeding problems, they may benefit from prophylactic factor to preserve their bones. The same is true of men with mild or moderate disease who aren't on prophylaxis.

This brings up the question of the new non-factor therapies being developed to treat hemophilia. They seem to do a good job restoring the ability of the blood to clot, but will their lack of factor VIII or IX lead to other problems? We tend to think that the main job of clotting factors is to form clots, but most proteins in the body actually have more than one function. Factor IX, for instance, is thought to be involved in wound healing. Will the use of a rebalancing agent to restore clotting have a negative effect on healing or some other body process because of a lack of factor IX? We don't know yet, but we may find out in the near future as these products come onto the market.

Finally, what would joint damage be without pain? Current thinking is that pain is a signal to us that there is a problem in our body. But what about the chronic pain that afflicts so many people? "We know we have a

problem – you don't need to keep reminding us." As I wrote in the last issue, we are learning more and more about pain and finding that some of our concepts may not be correct. Chronic pain may be a malfunction in the nervous system. It might get stuck in the "ON" condition, even though the pain is no longer needed.

This was demonstrated by a recent study from Belgium and Germany. The researchers looked at 30 adult patients with hemophilia who had ankle damage. They assessed the structure of the 60 ankles by both MRI and ultrasound, while also assessing pain by several methods. What they found is that there was no correlation between ankle structural changes and pain. That is, in the subjects with ankle pain, the pain was not actually coming from the ankle. They propose that the pain is actually due to a dysfunction in the nervous system, but this needs further study. [Roussel NA et al., Haemophilia, online ahead of print 3/16/22]

People with hemophilia need to pay attention to this. Osteoporosis is a silent disease. Many people don't know they have it until something breaks. Talk to your doctor or HTC. There is a simple X-ray test called a DEXA scan that can measure your bone density. If you do have a problem, there are a number of lifestyle changes and medical treatments that can help.

Now that some of the major issues in hemophilia have been resolved, researchers can focus on some of the other aspects that affect quality of life. These include the crippling joint damage, bone density loss and pain that many patients suffer. We are making progress that hopefully will result in hemophilia patients not only living long but living well.

FROM AWARENESS TO ACTION

BY APRIL WILLIS, PH.D.

In partnership with more than 1600 brands, nonprofits, government agencies and cultural leaders, The Coalition for Hemophilia B was proud to be a partner in Mental Health Action Day on May 19th. On this day, we encouraged and empowered people to take the next step for #MentalHealthAction. Go to MentalHealthActionDay.org to learn more and join our effort to shift from awareness to action on mental health.

Head to MentalHealthisHealth.us to get started with ways that you can take action for yourself or a friend on Mental Health Action Day. There is no-one-size-fits-all action, but rather, this is an open source effort for all who want to use their megaphones to drive our culture of mental health from awareness to action.



RAISING A CHILD WITH HEMOPHILIA: WHEN OUR BEST DOESN'T SEEM GOOD ENOUGH

BY MATTHEW BARKDULL, MS, MBA, LMFT, MEDFT

"When something goes wrong with children's bodies, the reverberations penetrate deep into the consciousness of families and health professionals alike."

– Susan McDaniel, Jeri Hepworth, and William Doherty, Medical Family Therapists

How many of you have sat next to the bedside of a child who is suffering from a raspy cough, congested sinuses, warming temperature, or upset stomach? Stressful, isn't it? Getting sick is a strikingly predictable feature of childhood. Managing an acute health problem is bad enough but a growing number of parents are facing the stress of raising children with chronic health conditions. In fact, an estimated 10 million children suffer from a type of chronic health condition.

Caregiver burnout is a common reality among parents. Despite the advances in hemophilia treatment to the point that many children are living fairly normal lives, parents may still commonly experience the following:

- Parental guilt
- Treatment anxiety
- Grief over the child's loss of "normal" childhood
- Vulnerability to health professionals and the medical milieu

Parents must understand feelings of stress, anxiety, and caregiver fatigue are real. Misunderstood, feelings of burnout can spin parents into a vortex of poor self-esteem, self-doubt, and unyielding shame. Sometimes it's okay to take a step back and say, "This is hard," or "This has been a hard day" or "I'm not sure what the future holds." Acknowledging one's feelings is an amazingly healthy behavior to exhibit.

So how do we best handle the day-to-day demands of a child's chronic difficulties?

- Accept your child's diagnosis. Denial stops the natural progression of grieving patterns that are vital for the body to experience. Denial also erects a barricade to treatment that may be necessary and hinders assistance that can greatly ease the burdens of caregivers. Accepting hemophilia opens doors to healing and accessing services that can be a boon to the family system.
- Put the child's diagnosis in its place. Hemophilia can unbalance family life in a major way. The "well" children can be yanked into our peripheral vision and ignored while efforts continue to focus on the diagnosed child. Family activities are delayed or are entirely forgotten as demands of caregiving take center stage. Putting hemophilia in its place can come in different forms such as preparing for activities that

are not planned around treatment. Other suggestions include spending one-on-one time with unaffected siblings, actually involving the other kids during "infusion time", arranging for "me time" by asking a friend or family member to watch the kids so you can take a drive by yourself, read a book, or anything else that you enjoy. Taking care of yourself is essential so you can feel up to the task of taking care of others.

- The family system is so much more than hemophilia. Although hemophilia does have its place, it can almost always be handled as any of the other issues that families face. Consider:
- Promoting open communication. Share your thoughts and feelings. Keeping an open dialogue with children can go a long way. When your children need to share their feelings, don't criticize or stop them...just LISTEN to them. Hear what's on their minds. Empathize with them and communicate that it's difficult for you too. Come up with a solution even if it's just a walk around the block.
- Collaborating with health professionals and schools. The ability to act goes a long way when it comes to managing a bleeding disorder. We want to have some control over at least some aspects of our lives. Healthy advocating gives a great deal of relief and a measured sense of control to parents. A warning, however. Don't be too over-zealous where feelings get hurt and bridges are burned. If you're unsure how to properly navigate and discuss these issues with professionals, seek out the guidance of a medical social worker, a Coalition member, community members, or another trained professional that can help you better understand and support appropriate processes.
- Keep up the great work, my fellow parents in the hemophilia community! You're doing better than you think!



women & girls with hemophilia

WE'RE IN THIS
together



articles to support, educate, and empower

The Birth of a Mother:

Part 1

BY CASSANDRA STARKS

As we all know, the sea of hemophilia can be frightening, mysterious and stormy. While I came to know my own hemophilia through high school and college sports, it was only shortly after I gave birth to my son that I began to understand hemophilia through the eyes of a mother.



When my son was born, he was absolutely perfect. With factor, a bit of Pitocin after delivery to prevent hemorrhaging, and a very detailed birth plan, I gave birth to Clyde naturally, without any interventions after nine hours of labor. By the end of his first day, however, he started to show the first signs of his bleeding disorder as we discovered a quickly growing hematoma on the back of his head. Filled with fear, grief and love, my husband and I watched helplessly as the nurses gave Clyde his first dose of factor. This was my first glance at the true strength, selflessness and dedication of the nurses.

Over the next several months I almost forgot about Clyde's hemophilia as he did not have any bruising or other complications whatsoever. Then, just as he was beginning to learn to crawl, Clyde woke up restless and crying late one night. After a quick evaluation, we realized one of his knees was very swollen, at least twice the size of his other leg. Although he had been a little extra fussy the last few days and not eating as much as usual, there were no other warning signs.



This began the landslide of decisions we had to make, and that every hemophilia parent has to

make for their child. I was hesitant to jump right on prophylaxis treatment as I knew how difficult it could be for him as an infant to get weekly infusions. At the same time, I knew firsthand how painful and damaging joint bleeds could be.

After much discussion with the nurses and doctors at our local HTC, we decided to begin regular infusions to prevent any additional major bleeding. This began the weekly trips to the nearby hospital. Each trip I was so nervous, praying that it would only take one or two pokes to find a vein. For a couple of months, the nurses were quite successful. I found ways to minimize the trauma Clyde had to undergo, including laying on the table and breastfeeding him during his infusions and sharing some chamomile tea just before to help us both relax.

Then, suddenly Clyde blew four veins in one session. We went home without factor and one of the blown veins in his foot turned into a bleed. His little foot was swollen, puffy, and darkly colored purple and blue. Two days later, we tried to infuse again but were unsuccessful, having blown three more veins.

The nurses urged us to have a port implanted, and while I disliked the idea of Clyde going through surgery at 11 months old, it was the only option left to ensure that he would be able to receive his much-needed factor.

A few days later, filled with fear, I handed Clyde off to the nurses to ease him to sleep in preparation for the surgery. The procedure took longer than expected as



they had difficulties getting the IV started, but thankfully his surgery and recovery were both very smooth and uneventful.

Now, Clyde's infusions are relatively simple. At 18 months old, we use numbing cream to numb his port, and I still breastfeed him to comfort him during the infusion. Swaddled, he lays completely still, rarely fusses and will sometimes even fall asleep during it. He has had a couple more bleeds but recovered well from both after treatment. While I still inwardly flinch every time he bumps his head or falls, I'm slowly learning about his hemophilia and what types of things cause him to bleed.



The transition from a woman with hemophilia to the

mother of a son with severe hemophilia has been very challenging and yet offered many opportunities for me to blossom into a better human being. Watching the resiliency and dedication of the nurses and Clyde during each infusion has taught me my own strength.

The separation during Clyde's surgery taught me how much of a blessing he really is, and every bleed has taught me that life is truly mysterious and uncontrollable. While having years of experience dealing with my own bleeding disorder has been incredibly helpful in knowing what to expect in my son's hemophilia, there are still many unknowns, and I am so grateful for this community to help walk me through it.

Note: This article was written in Dec 2020. Shortly after it was written, Clyde stopped breastfeeding during his infusions by his own choice and I began learning how to access his port.

Now, at 3 years old, Clyde is sitting up, unrestrained for his infusions and watches me infuse him in a small mirror. While his infusions aren't his favorite thing, as he'd much rather "go play," he is very cooperative with the whole procedure.

On February 27, 2022, we welcomed into our family our second child, Edith Louise Sever. She came very suddenly and peacefully without any complications after a 2-hour, painless, natural labor.

Look for Part 2 of this article soon!

WORLD HEMOPHILIA DAY 2022

BY GLENN MONES

April 17, 2022 was World Hemophilia Day. The theme of the event this year was "Access for All: Partnership. Policy. Progress."

This important event is about bringing the global bleeding disorders community together. By raising awareness and bringing hemophilia and other inherited bleeding disorders to the attention of policymakers, we can increase sustainable and equitable access to care and treatment.

There are many ways you can bring attention to hemophilia and other inherited bleeding disorders all over the world. Whether you are a person with hemophilia B or another bleeding disorder, you can use social media and many other channels to get the word out.

The Coalition for Hemophilia B is proud to be a donor of WHF's Humanitarian Program, sponsor patients with hemophilia through *Save One Life* and to support the documentary *Bombardier Blood*. Near or far we are all connected.

The World Federation of Hemophilia has many tools to explain World Hemophilia Day and help promote the event and related programs. For more information, please visit <https://wfh.org/world-hemophilia-day/>



DON'T STAND ON THE SIDELINES

LEARN IF RIXUBIS®
MAY BE RIGHT FOR YOU

Visit RIXUBIS.com to learn more

➤ 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®:

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.

RIXUBIS
[COAGULATION FACTOR IX
(RECOMBINANT)]

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.

What else should I know about RIXUBIS?

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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PORTRAITS OF PROGRESS

On Friday, June 10, 2022, we were honored to see members of our community, including our President Wayne Cook, represented in this incredible art exhibit, **Portraits of Progress**.

This celebration of how far the medical industry has come since the 1940s showcases patient, caregiver, healthcare provider stories as well as clinicians, scientists and the history of clinical advancements.

This iconic event ran from June 11–19, 2022 in Soho, New York. Follow this link to see when the exhibit will be in your area or national conference.

<https://www.portraitsofprogress.com/?fbclid=IwAR1bz-jNiOOV7BV4q70gKLAIC2Vr7pVdzYmiS6IBZoUSeNuPrYhCI7QIlml>



CSL Behring

UNBURYS MY FATHER BOOK RELEASE

BY KIM PHELAN



On Sunday, June 12, 2022, Zander Masser held a book reading about his father, Randy's life with 14 close friends

and family. There were plenty of tissues on hand as each one read an excerpt from the book, *Unburying My Father*. We felt the strength of Zander's mother as she came to grips with, her husband's decline in health due to the tainted blood products people with hemophilia received in the 1980s.

We heard Ed Rogoff share his love for his dear friend he lost too early in life. He tried to fight back the tears of the pain he was so clearly reliving while reading from the book. Also felt was the loss of so many he knew with hemophilia and how he survived when they did not. Zander shared the eulogy he wrote for his dad that called for more tissues quickly being passed among attendees. One

story from a baseball coach hit a home run with me. A local kids' baseball field was named after Randy. Randy loved baseball but couldn't play - what he could do was take photos of the kids, and that was his gift. He had team spirit!

As we progress and move forward, we must always remember the past and honor those who went through so much. This book is a gift in multiple ways - it shares some of the most beautiful photographs I have ever seen. It shares the raw story of one family, what they went through, and the healing that took place on multiple levels after many years. This is a book everyone should all read!



100% SAM

AN INTERVIEW WITH SAM BY RENAE BAKER

He had never imagined such an out-of-this-world idea but there he was! He hadn't even time to process the possibility before he was guided onto the ice and handed a hockey stick. It was easier than he would have thought, and it felt a bit like flying.



You may know him as “Sammy,” because he has been a Coalition member since he was a kid. He’s 25 years old now, and because I see so many references to him as Sammy, I just had to clarify: “Three years ago, you told me that you prefer to be called “Sam. Is that still true?”

“Yes. 100%,” he states emphatically.

So there we have it! If you want to respect his wishes, please call him “Sam.”

When I caught up with Sam, he was coming down from a couple of weeks being “in and out of hospitals,” after an accident that left him with a badly fractured femur. My thoughts went to – “Oh no! Was it a hockey accident? How did it happen?”

“No. I fell off my bathtub,” Sam shakes his head. “I wish it was from hockey! That would’ve made me feel extra cool.” The head shaking continues, now with laughter. He explains that his leg will be in the knee mobilizer for quite some time; a summer project for which he hadn’t signed up! At least hockey season is over, and he won’t be missing any practices or games, which would have hurt more than the broken leg.

Sam is a handsome twenty-five-year-old man with dark, wavy hair and sparkling, brown eyes that invite you into his world. “Shy” by his own admission, he is often serious and quiet, but when he smiles, he lights up a room, and

his laughter lifts the spirits of many. You may know that Sam cruises around in a wheelchair, but you might not know that the reason is that – along with hemophilia B – Sam was diagnosed with spina bifida at birth. According to the CDC, spina bifida is a type of neural tube defect that can happen anywhere along the spine if the neural tube does not close all the way. This often results in damage to the spinal cord and nerves.



You may be thinking, “But didn’t you just say something about ice hockey?” That’s right. Sam has played ice hockey for the past 4+ years.

“A coach and a friend of mine talked it over and thought it would be a good idea,” Sam remembers. Having someone in a wheelchair on the ice was a new idea to all of them, but they decided to give it a try!

In what world is an ice hockey team able to give something like that a try? In SNAP Sports world! SNAP is an acronym for Special Needs Athletes and Peers. SNAP Sports is a Valencia-based non-profit organization providing sports-based activities for athletes with special needs with the strategic bonus of peer-based partner training. Kurt, one of the coaches of the Valencia SNAP Flyers, allowed Sam to come and watch a practice.

"And then, all of the sudden, he made me part of the team!" Sam exclaims, "I was like, 'Wow! OK!'"

Still trying to wrap my mind around how this played out, I ask, "So, basically, they just brought you out there. In your chair. And they said, 'Let's see how this goes.'"

"Yup!" Sam is smiling to see the incredulity he felt that day reflected in my face.

"...and here's a hockey stick!"

"Yup!" And we both burst out laughing!

"I'm like, 'OK!'" Sam says with energy. "Talk about throwing caution to the wind!"

I asked him how it felt when he first got out on the ice.

"It was super, super-duper exciting," he states with a peace about him that might seem incongruous if it weren't for the lambent light also flickering in his eyes. "It was not at all like anything I'd ever done before."

Sam speaks admiringly of the coaches of the team, which is comprised of people with different special needs. He explains that a couple of the players are vision-impaired, so the coaches will help guide them in the right directions and away from danger. These coaches push Sam's chair while he navigates the hockey stick. I imagine aloud that these coaches are very special people.

"They are," he affirms with a serious nod.

"The season runs from October through April. Most of the season is spent practicing," Sam reports. All of that practice culminates in four games. This season, the Valencia Flyers won two and lost two; a turnout with which Sam is pleased.

The practice and game time also fosters meaningful relationships. "I have a LOT of friends (from the league,)" he says. "They are dear relationships. Everyone is very kind and nice and loving to each other."

At any given time, on the ice, there are four or five coaches, and strong relationships are formed with the coaches of his team as well as coaches and members of other SNAP teams.

Sam enjoys his ice time with the Valencia SNAP Flyers so well that, when the season is over, "It's so boring," he laments. He is currently on summer break from Southeastern University, a Christ-centered institution in Valencia, CA. He is obtaining a bachelor's degree in Ministerial Leadership. He is preparing to be a youth minister. A pastor at the non-denominational church at which he is a member saw potential in Sam. She asked him a few questions and then told him about this college program, thinking it was a very good fit for him.

Now, halfway through the program, he reflects that it has been both challenging and enlightening. "It definitely takes me out of my safety zone," he says, soberly. He has already delivered a few children's sermons at his home church. Though I am confident that this congregation feels mightily blessed to hear Sam's sermons, he admits to severe stage fright. Happily, I Sam joined us in Nashville for the 4th annual The Beats program, this summer, where I had the privilege of working with him on music and stage fright!



As he earns credits toward his degree at SEU, Sam is also attaining hard-won wisdom from life lessons that he believes apply universally. One he wants to share with us pertains to advocacy. He recalls a particularly difficult time, as a new college student, when he was struggling academically. "I knew I needed help, but I



didn't tell anyone. I didn't speak up for myself," he winces. As Sam's grades and attitude in class declined, a meeting was called between his professor, his mother and Sam.

"Expectedly, my mom was upset," Sam squirms at the memory. He was now being ordered to reach out for help when he needed it. "It was hard at first, but I said to myself 'You have to start doing this,' and sure enough, when I did, it worked. I'm so happy now! I realize that, if I'd spoken up earlier, I would've gotten the help I needed sooner."

Advocating for himself was not an easy act, but the fact that his studies have been going smoothly since that uncomfortable confrontation gives Sam the confidence to urge us to self-advocate. "When you need help with something, don't just stay quiet. Speak up for yourself."

Another piece of advice Sam offers is this: "Don't let hemophilia stop you from doing

what you're doing. Sure, it might come with some challenges, but don't let it stop you." We unpack this a bit more, and Sam inspires me with the idea that just persevering in the act of living your life the way you choose to is a form of advocacy in itself.

Sam tells me that every morning that he wakes up, he feels blessed to simply be breathing.

"Are you just really in touch with the idea that it could be otherwise?" I ask.

"Yes."

"What do you think keeps you so presently mindful of that fact?" I press.

"I just keep reminding myself of why I'm here; what my purpose is."

"I think of you as touching the lives of other people in such a positive way," I express to Sam. "Do you believe that you have a positive impact on other people?"

"100%!" he owns.

"What are your hopes for your interactions with people?"

"Just that I can encourage them every day; anyone who crosses my path."

I know this guy, and I assure you – that is 100% Sam.

B AMAZING

A huge shout out to Bill Patsakos and the FDNY team for loading up donated medical supplies going to Ukraine! We're proud to see our hemophilia B community making a difference in the world!



VIRTUAL MEETINGS “ON THE ROAD” WITH THE COALITION FOR HEMOPHILIA B

VIRTUAL MEETINGS ON THE ROAD

The Coalition for Hemophilia B's **Family Meetings on the Road** is our way of “bringing the Coalition to you” when you can't come to the Coalition. During the height of the pandemic, we introduced virtual versions of these popular one-day events which bring interactive educational programs and speakers to the local level.

The last series of the 2022 virtual programs consisted of nine events held January 15th, 22nd, 29th, February 5th and 19th, and March 5th, 12th, 19th and 26th.

Eight programs were sponsored by CSL Behring and one by Novo Nordisk. We grouped participants according to regions of the country so that they could interact with other community members from the same area in smaller group settings. The programs were extremely successful, with many attendees choosing to attend more than one meeting.

While not every program was identical, all participants had the opportunity to hear from a variety of experts who shared information community members can apply to their own lives. Popular sessions included the *Importance of Rhythm, Laughter and Joy* with

Robert Friedman, MA, *Navigating Finances* with Ellen Kachalsky LMSW, ACSW, *Memory Kaleidoscope* with Lee Kim, *Tai Chi* with Rick and Cassandra Starks. Each program is designed to get

you moving, gain support and connection and learn in engaging and interactive ways. It has been our pleasure to spend time with all of you and we loved going over time on many occasions as we were immersed in final rounds of specially designed Kahoot!

As always, the programs were very popular and the feedback excellent. Thank you to our sponsors, speakers, staff and participants, and stay tuned for more programs, both in person and virtual, in the coming year.



CREATIVE TIPS & HEMO HACKS WORKSHOPS FOR JOB SEEKERS BY ROCKY WILLIAMS

Community member Christian Harris hosted well-attended and engaging sessions on “nine factors to get the job you want and build your career.” The sessions were sponsored by Medexus. Both were loaded with very practical advice that job-seekers can put to immediate use in their own lives.

One of the useful tips Christian offered: you've got three seconds to communicate who you are when networking or interviewing. What do you say? Does your resume accurately reflect your experience and your aspirations?

These and other ideas sparked a lively discussion. Christian shared his substantial knowledge of job hunting, applications,

and career building. His interactive presentations were hugely valuable and thought-provoking! A special thank you to our sponsor Medexus.



COMMENTS

“The program was great. I really liked the handy tips and illustrative examples from different job types. I also liked the interactive portions, where all participants were engaged and there was open discussion related to resumes and much more. I personally learned several handy tips from the speaker as well as from fellow participants.”

“It was very informative and useful in getting a career ready in any field that you want to specialize in. Amazing tips and tricks!”



MENTAL HEALTH FIRST AID: MAKING OUR COMMUNITY TRAINED AND READY

BY GLENN MONES

Many people think of chronic conditions as being entirely physical. However, we have learned over time that there is often a significant mental component that can manifest itself as anxiety, depression or in several other ways. This effect has been exacerbated by the impact of the COVID pandemic, including families forced to stay at home, missed school and work, economic hardship and many others. When these issues are present, they can in turn worsen physical health, creating a vicious cycle.

That's why The Coalition for Hemophilia B has been placing increased emphasis on educating our members about recognizing and effectively addressing mental health issues within our community. Our most recent efforts have included a series of half-day training sessions in mental health first aid. These latest trainings were held on January 29th (sponsored by Sanofi), February 26th (sponsored by Novo Nordisk), March 12th (sponsored by Medexus) and March 26th (sponsored by Takeda).

We all know that in general, first aid is about having the basic knowledge to help someone in distress when we encounter them and to get them to the next level of medical attention. However, there is also something called mental health first aid where individuals are given the basic knowledge to help someone in mental distress when we encounter them and get them to the appropriate professionals who can help address their situation. Our trainings were created by Debbie de la Riva LPC, founder of *Mental Health Matters Too*.

Debbie has a master's degree in counseling from the University of Houston and she became a licensed professional counselor in 1989. Her work experiences have included serving as a stress management therapist in a psychiatric hospital and managing a mental health resource department. When her son was born with hemophilia in 1994, the entire de la Riva family became involved in the bleeding disorders community at both the local and national levels.

Debbie has since become a major force in elevating the attention given to mental health issues in the bleeding disorders community, including many collaborations with The Coalition for Hemophilia B.

Training participants were given study materials and advance "homework" prior to their session. The agendas were then divided into interactive components



designed to help them understand how to identify potential mental health issues in family members, friends, community members, co-workers and others. Once a potential issue is identified, the goal is to provide the individual with referrals to professionals and other resources that can help them effectively address their problem. The goal is not to "fix" the problem as this does not fall within the scope of mental health first aid. Each training concludes with a test designed to assess what the individual has learned. Those who achieve a high enough level of knowledge, understanding and skills then receive certification as a mental health first aid provider.

Because of these sessions and others that went before them, the hemophilia B community now has a growing team of trained mental health first aid providers who can make a real impact on how we address mental health issues. If you are interested in joining a future training, please contact the Coalition and we will let you know when new trainings have been scheduled. Thank you to all the listed sponsors for making this vital program possible.



COMMENTS

"I just wanted to tell all of you Thank You so much for offering and allowing me to participate in the Mental Health First Responder course. The course was wonderful. I learned some great tools that I can use, not only to keep myself in check, but also to make sure

the people I work with and care about are ok. My family and I have had some real struggles over the last few years, not only with Covid but just in our lives. I honestly can not express enough how grateful I am for the Coalition. All the meetings and activities have come along at a time I needed them most and have kept me balanced. Thank You ALL again for everything you do.”

“The class was awesome and extremely informative. I enjoyed the teacher tremendously and was thankful she gave us the course work for it. I have actually been able to use my new skills in my work life to assist a co-worker so that was pretty rad. Thank you for offering it to the community.”

“I really loved the course! It teaches empathy at a whole new level. She also did a real good job of teaching us the warning signs to look out for and actionable steps to help others.”

“Not only did the Mental Health First Aid Course teach me how to recognize signs of severe stress / mental health issues in others, it also offered me insight into recognizing what is normal or in need of support in dealing with my own and family members’ levels of stress.”

“Debbie de la Riva offers kind, humble, and supportive information in dealing with mental health issues; she is approachable and truly in touch with her own feelings.”
“I really enjoyed the Mental Health First Aid class. I felt it was interesting and helpful. I learned many things including signs to look for in an individual experiencing a mental health issue and also how to guide them as to seeking help.”



“It was great! The course was very helpful in helping people who are going through a tough time. I learned that self care is very important in being a mental health first aider. I enjoyed every minute of it.”

“I learned quite a lot and I learned that this is a way long overdue topic of discussion. The need is vast specially during this COVID time. Not just adults are facing depression, anxiety and other mental health issues but children. Mental health should actually be discussed at every symposium and specially when newly diagnosed with hemophilia.”

“The course is well worth your time. You learn alot about how to effectively manage someone appropriately, in a mental health crisis without triggering them. You learn how to encourage someone to get help when they are struggling. There is a large amount of information in the manual, but all very helpful”

“Powerful”

“This training was eye opening for me. I am a 2+2=4 type person and learning soft skills like these are very helpful. After the training, I found myself thinking differently when communicating with my kids and with my friends when they were expressing difficult situations. The skills I learned, the questions to ask, the pauses to take were invaluable in the subsequent interactions I had and I am so thankful to the coalition for helping me become a more empathic human being, mother and friend.”

HFA SYMPOSIUM

BY GLENN MONES

Wayne, Rocky, Farrah and many other CHB members had a great time April 20–23, 2022 at the HFA Symposium in San Antonio, Texas. It was great to see you! We were super excited as CHB Chairman Dr. David Clark was awarded *HFA Volunteer of the Year!*



EMERGING THERAPIES 101: AN EVENING WITH DR. DAVID CLARK

BY GLENN MONES

It was only several years ago that therapies for hemophilia B were limited to just a couple of products. Today, there are more treatments and even more coming.

One of the challenges is the need to understand how these new therapies are different and what questions to ask when considering a new treatment regimen in consultation with their HTC and healthcare provider.

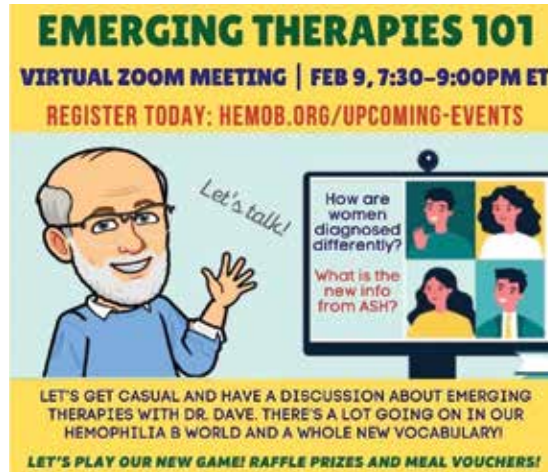
To address this need, The Coalition for Hemophilia B has hosted several programs designed to give community members an understanding of this exciting “new world” of treatment. Most recent of these was our **Emerging Therapies 101** held virtually on February 9th, 2022. The program featured Coalition Chairman Dr. David Clark.

Dr. David Clark, our featured speaker, has a Ph.D. in chemical engineering from Cornell University and more than 35 years of experience in the development and manufacture of plasma and tissue products, including factor IX concentrates. Much of that experience comes the time he spent working with the American Red Cross.

Dr. Clark has repeatedly used his expertise and verbal skills to help community members gain the understanding they need to navigate hemophilia B and its treatment. He has a gift of taking the complex and making it easy for us all to understand, empowering us to have conversations with our providers.

During the program, Dr. Clark offered a “deep dive” into the therapeutic modalities that have appeared on the scene. These included current products, new inhibitor treatments, gene therapy, subcutaneous delivery, rebalancing agents and others. The number of new therapies in development is astounding.

Dr. Clark explored many questions people have about gene therapy, including safety, how long will it last, and how will it be paid for. He also discussed the recent efforts by the International Society on Thrombosis and



Haemostasis (ISTH) and other groups to recategorize women affected by hemophilia. These efforts represent an important improvement in the attention paid to this important group of patients.

The event ended with more questions and answers with Dr. Clark followed by a fun, educational interactive quiz. Stay tuned for more virtual and in person events offering the most up to date information on the treatment of hemophilia B and other critical issues.

Emerging Therapies 101 was made possible by the generous sponsorship by CSL Behring.

CSL Behring

COMMENTS

“Dr. Dave has a great wealth of knowledge, and makes learning very easy! Thank you!”

“It was nice to hear Dr. Dave speak frankly/ casually about the new therapies in the works, and to learn about what is also going away. Really neat to see the list of all the different companies working on things in hemophilia right now too. It’s an exciting time. Thank you for hosting and connecting us, once again:)”

“There is so much going on and I do not understand all of it. Sometimes I am so confused. I appreciate Emerging Therapies because Dr. David Clark helps us understand things in an easier way and I don’t feel dumb when I have to ask a question. He makes us feel welcome.”

“Emerging Therapies helps me stay up to date on what is going on. A lot of the new information is hard to understand, and my doc is not going to sit with me for hours on end. I understand so much more now and I can actually talk to my doc and know what I am talking about. I look forward to seeing more of these. Thank you for all you do.”

COMMUNITY VALENTINE LADYBUG CUPCAKE DECORATING PROGRAM

BY GLENN MONES



Throughout the pandemic, the Coalition has hosted a wide variety of events to bring members of the community together virtually, even when they could not come together in person. These events have been geared to a variety of age groups and occasions, with community members participating from throughout the country.

On February 12th, just prior to Valentine's Day, community members joined us for

virtual **Love Bug Cupcake Decorating** assisted by expert cake creator Daniela Delgado of *Daniela's Little Wish*.

All pre-registered participants were mailed a box in advance with everything needed to create these fanciful holiday treats. With Daniela's guidance, it was easy and fun for everyone to become instant pastry chefs! The event wrapped up with a couple of rounds of the popular quiz game Kahoot! Based on the feedback we received, everyone had fun and appreciated the opportunity to spend time with the community. Stay tuned for more events soon!

Special thanks to our sponsors Medexus Pharma and Paragon Hemophilia.



COMMENTS

"This was such a fantastic way to spend time with other families in the hemo B community! We really had a great time making cupcakes, playing games, and just seeing everyone one. So much fun! Thank you!"

"We are "flying" our little cutie ladybug cupcakes to friends and family tomorrow!! Thanks for a such a fun time decorating You've given us a great memory and gifts to give! Love ya for that!"

We had so much fun making these adorable lady bugs! I did not think I could create something so nice! It was so nice seeing all the families virtually. This is what community is all about!

TO VIEW MORE PHOTOS VISIT OUR FACEBOOK PAGE UNDER "ALBUMS" OR OUR WEBSITE: HEMOB.ORG



LET'S PLAY IX GOLF FUNDRAISER

Wednesday, May 18th was a beautiful day for golf! Sponsors gathered along with caregivers and people with hemophilia B gathered for a good cause. A big thank you to Perry Parker for holding clinic and to Hope Woodcock-Ross both who give so generously of their time and make this program extra special each year.

Special Thanks to the generosity of our Sponsors, we are able to provide golf access and education to children within our hemophilia community so they can experience this fun social activity and its many health benefits. Funds raised also benefit the BCares Patient Assistance Program.



LET'S PLAY NINE
 HELP MAKE THE GAME OF GOLF ACCESSIBLE TO YOUNG PEOPLE IN THE HEMOPHILIA COMMUNITY.
 YOUR DONATIONS HELP SUPPORT OUR EFFORTS

- OUTFIT BEGINNING GOLFERS WITH THE EQUIPMENT, LESSONS AND SUPPORT NEEDED TO ENJOY THE GAME OF GOLF
- PROVIDE SUPPORT TO SEASONED GOLFERS TO HAVE ACCESS TO THE GAME
- SUPPORTS OUR BCARES PATIENT ASSISTANCE PROGRAM

THANK YOU FOR YOUR SUPPORT!

LET'S PLAY NINE

THANK YOU TO OUR SPONSORS!

GOLD LEVEL

CSL Behring **MEDEXUS PHARMA**

novo nordisk

SILVER LEVEL

CVS specialty

BRONZE LEVEL

WINGMEN





Calling all young people with hemophilia, ages 7-19! Did you know we offer a scholarship so you can explore golf as your sport of choice! This scholarship may be used toward golf lessons in your hometown and golf equipment/gear. Learn more about eligibility and apply now: hemob.org/scholarships. The deadline is October 15, 2022, so act quickly!



LET'S PLAY IX: GOLF SCHOLARSHIP

AGES 7-19

DEADLINE: OCTOBER 15, 2022

APPLY TO USE FUNDS TOWARDS GOLF LESSONS IN YOUR HOMETOWN AND GOLFING GEAR!

HEMOB.ORG/SCHOLARSHIPS



Congratulations to the 2022 William N. Drohan Scholarship awardees!
Seth Patsakos, Emily Marlatt, Cecilia VanSant, Luke Luckey, Michael Joshua, Steven Sclafani, Ryan Van Ekelenburg, Jordan Howard and Blake Skipworth.
We can't wait to see the amazing things you will do!

Congratulations

2022 WILLIAM N. DROHAN SCHOLARSHIP AWARDEES

THE COALITION FOR
HEMOPHILIA 

NAT LATHROP MUSIC
SCHOLARSHIP
[HEMOB.ORG/SCHOLARSHIPS](https://hemob.org/scholarships)



We are honored to offer the Nat Lathrop Music Scholarship for the 2nd year. This scholarship is for students who are pursuing an interest in music because Nat's family appreciates the connection between music and mental health. Learn more about eligibility and apply now: hemob.org/scholarships. The deadline is October 15, 2022, so act quickly!



MEETINGS ON THE ROAD

IN-PERSON, OCTOBER-NOVEMBER 2022

WE'LL BE COMING TO THESE STATES:

- Missouri
- Georgia
- Texas
- Minnesota
- Virginia
- Michigan
- North Carolina
- Colorado
- Ohio
- Pennsylvania
- California
- Illinois
- New York



THE COALITION FOR
HEMOPHILIA 

DEADLINE
TO RECEIVE
BOX:
NOVEMBER 4

WOMEN'S WINTER EDUCATION & EMPOWERMENT RETREAT

DECEMBER 2-4

register: hemob.org/programs-events

ALL VIRTUAL

A SPECIAL THANKS TO OUR SPONSOR:

sanofi



B TOGETHER

**CHB 2023 SYMPOSIUM
HEMOB.ORG**

**We can't wait to see you at our
Annual Hybrid Symposium 2023 in Orlando, Florida!**

The Coalition for Hemophilia B's Annual Hybrid Symposium is the largest regular gathering exclusively for families and individuals affected by hemophilia B. We hope you can join us from Thursday, March 16, 2023 to Sunday, March 19, 2023 at the Renaissance Orlando OR *virtually!*

Learn more about applications, travel scholarships, and scholarships for first-time attendees at <https://www.hemob.org/annual-symposium>.

All applications are due December 3, 2022!

The Coalition for Hemophilia B understands there are families within our bleeding disorder community who feel the effects of the current economic situation. While the Coalition will also contribute to this fund, we ask our more fortunate Factor Nine Families to help us by making a financial donation to the Factor Nine "Holiday Fund" to help buy gifts for children with hemophilia.



To make a donation, please send a check payable to:

The Coalition for Hemophilia B "Holiday Fund"
757 Third Avenue, 20th Floor; New York, NY 10017

Please respond by **November 23, 2022**
 so Factor Nine Santa can load his sleigh with holiday gifts for all
 good girls and boys!

For families in our community in need of a little holiday cheer, we would like to help put something under the tree for your children! Fill out this form and send it to Santa's special elf, Kim, at the "East" Pole. Factor Nine Santa has a busy schedule, so please send this form no later than **November 23, 2022**. Your name and information will be kept strictly confidential. Send mail this form to:

The Coalition for Hemophilia B Holiday Cheer
757 Third Avenue, 20th Floor; New York, NY, 10017



We wish you all a beautiful holiday season filled with love, happiness and good health!

Name: _____

Street Address: _____

City, State, Zipcode: _____

Phone: _____ Email: _____

Please give an exact description of your child's wish item. Gifts will be purchased and sent to your home. **Please note which child is affected by hemophilia B.**

Child's Name and Age:

Child's Name and Age:

Child's Name and Age:

Wish List:

Wish List:

Wish List:



757 Third Avenue, 20th Floor; New York, New York 10017
 Phone: 212-520-8272 Fax: 212-520-8501 contact@hemob.org

VISIT OUR SOCIAL MEDIA SITES:

Website: www.hemob.org

Facebook: www.facebook.com/HemophiliaB/

Twitter: <https://twitter.com/coalitionhemob>

Instagram: www.instagram.com/coalitionforhemophiliab

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



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



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.

Sanofi

to be able to talk about things with the other teens and the game was pretty awesome! He also told me a few things that blew my mind! He actually learned new information and words he normally does not know related to hemophilia. Now I think that's pretty cool too! Thank you!"

"The experience pushed me out of my comfort zone. I became more comfortable hosting a meeting and it gives me more confidence in my ability to do it again."

"The best part of the entire experience was once we were on call. Getting to talk and interact with people you've never met before but have something really unique in common with."

"I really liked this event. I think it was fun and it was good to go to a hemophilia event but not talk about hemophilia the whole time. It was fun to do the activity and I would like to do something like that again."

Stay tuned for upcoming events beginning September through February! Contact Rocky at RockyW@hemob.org if you are interested in helping create the next teen adventures!

These amazing teen events were made possible by the generous sponsorships of Sanofi.

One teen exclaimed, "I loved the Gartic Phone game and just having chill time to talk."

The teen events are in full swing! Here are a few more comments we received after the events:

"I think this meeting was great because many of the people who joined participated and joined in on the conversations we were having. I think we had some good conversations with Matt, and it was a lot of fun talking and hanging out with all the teens. The escape room was fun, and it made it much easier when everyone helped to solve the riddles. The process of creating the teen event was very interesting, and it was a lot of fun to be able to plan the whole event. These events are super cool because I get to connect with many other teens and people in the hemophilia community. If I had the opportunity, I would hundred percent be interested in creating another event."

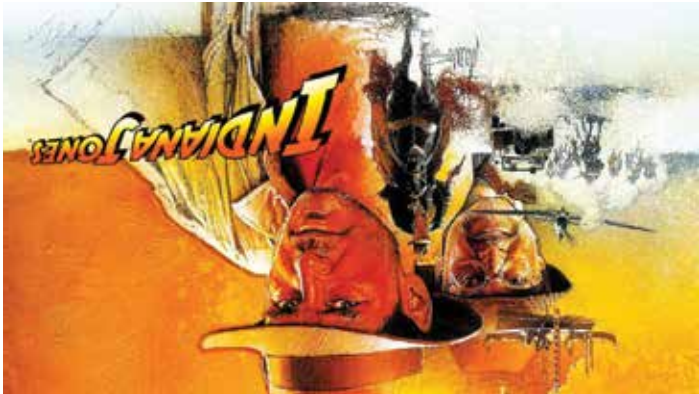
"My daughter is enjoying the teen events. She is on the quieter side, so she is a hard read. However, she looks forward to the events and has shared with us that they are fun. Additionally, she likes getting to know the other teens and has mentioned how nice it would be to meet in person. Thank you for allowing her to take part. She often feels overlooked, so these events help her feel part of the community."

"My son said it's super cool to connect with other teens just like him. He also said it was fun and that he liked

TEEN PROGRAMS THRIVING!

BY ROCKY WILLIAMS

The Teen Program for the hemophilia B community has been thriving! Working with teens, we have successfully created outstanding programs that have the teens engineer and co-host the events that they want to see. From mental health check-ins facilitated by family therapist Matt Barkull to icebreakers, games, raffles, and trivia, the teens have the opportunity to interact and hang out with each other in a fun and safe environment.



You know it's going to be a wild ride when you start off with an epic Indiana Jones car and airplane chase! Our teen event on March 16 was a smash hit!

Created with the help of community teen, Daniel, teens were provided the opportunity to experience their own riveting Indiana Jones-style scavenger hunt. Searching high and low, teens found many of their sacred possessions and shared how they related to each item. Teens learned more about each other and their hemophilia and discovered they have much in common. "The scavenger hunt was fun because it was fast-paced," said Tristan.

Nick, said, "I thought the Indiana Jones teen event was a great opportunity to meet and network with other kids my age. The games were fun and engaging!"



Daniel expressed, "We spent time learning about hemophilia and time learning about ourselves."

The parent of one of the teens mentioned, "I know my son enjoyed it! I could hear him in his room having a great time!"



Gartic Blast

Another exciting teen event was Gartic Blast! Community teen, Muhammad, shared his vision for a teen event with the community. The event was filled with interactive games that educated teens on hemophilia and current news.

After both events, several parents had the following to say about the experience for their teens:

"Thank you so much. He had lots of fun. He really enjoyed getting to know everyone and was impressed with how a lot of the teens were either in the band or choir. He said we have lots in common on top of having hemophilia."

"I wanted to share with you that he had a really good time and was glad he attended! That means everything! Thank you!"

"I just wanted to thank you for organizing the teen event. He thoroughly enjoyed the event and learned a lot from your individual meetings. I appreciate the opportunity and guidance. Our boys need positive male role models. He told me this morning that he cannot wait for next month now."

SIBLING BOND

BY FELIX

My name is Bethany, I go by Beth, Felix, and many other monikers! I live in Forney, Texas, and I am currently 15 years old.

While I do not have hemophilia B, I am the daughter of a symptomatic carrier and the younger sister of two brothers with severe hemophilia B. Safe to say, my life has been far from the "norm" of a lot of my peers given that my two siblings have this blood disorder. Sometimes, it is easy to forget how scary things can get.

I remember this one time my second eldest brother had to be rushed to the hospital because we thought he had a really bad bleed and worried that it would get worse. I was not able to go and had to stay home with my older brother and my mom while my dad took him to the hospital. We were all very scared and wondered if he would be okay. Hours later, we got a call from my dad saying that he was going to be fine but needed some rest. When my brother returned home, I was so happy to see him! I was greatly relieved to see him healthy again. Let's just say on that day, I was extra nice to him.

My brothers have proven time and time again life is meant to be lived! Living with the complications of hemophilia can be challenging, that is a fact. However, with treatment, people with hemophilia can manage their bleeding symptoms and continue to live fulfilling lives, just like my brothers. While a lot of my identity is tied to

being the sister of two brothers with severe hemophilia B, I still have a lot of passions that are unique to me as an individual. One thing I'm very passionate about is painting. Painting has always been a stress reliever for me. Whenever I am stressed, sad, or mad I know that I could take out some paint and a canvas and everything will be okay! I've been painting ever since I was little but only got "good" at the artform when I was about 10 or 11. My family is very supportive of my painting and are always giving me new ideas when I need them, especially my mom. My biggest challenge when it comes to painting for me is giving up. Sometimes I will start a project and it will become too hard or something goes wrong and I get frustrated but that's when I remember "there are no mistakes, just happy little accidents." I hope someday people will recognize my paintings and think to themselves, "Wow, that kid doesn't paint too bad!" I hope someone gets inspired or even feels comforted by my art. A lot of times in my life, I've felt different - and not always in a good way. I would like to remind everyone, especially those with siblings with hemophilia B, that it is okay to feel and be "different." Our differences are what make us unique individuals. While I am sure it is easy to love the people in your life, please remember to love yourself too and never give up on your dreams! In wise words of the musician Pitbull: "Live life, don't let it live you."



THANK YOU,

LET'S PLAY IX

BY JAMES

The Coalition for Hemophilia B's **Let's Play IX Scholarship** introduced me to golf. Before Let's Play IX, my only exposure to golf was on vacation. My parents asked me over the years if I wanted to learn golf and I always told them I wasn't interested. At the time, volleyball was my favorite sport.

In the two years leading up to the scholarship, I had had knee surgery on my right knee and had a tumor in my left femur. I couldn't walk for more than 45 minutes without having to stop for the pain, which made playing volleyball impossible. To qualify for the *Let's Play Nine Scholarship*, you must have hemophilia B and be between the ages 7 and 19. When I learned I had won the golf scholarship, my first thought was, "Okay, cool. I guess it could be fun." Since I was fortunate enough to get a scholarship from the Coalition, I thought I should at least give it a try. My mother offered to pay for one or two lessons and if I liked it, we would use the scholarship for clubs.

Soon enough, the scholarship did go toward clubs.



I started off with lessons and played for the high school team this past fall during their season that was reduced due to the pandemic. The season started about six weeks after my tumor was treated. Because of this, I wasn't sure if I would be able to make it through a practice much less a match. There were only four of us on the team which meant if any of us became injured or sick we would have to forfeit the match. We made it through the entire season!

This year is my second year playing for the high school. I hit my lowest score our first match! I have seen overall improvements in my life thanks to the sport. For example, I have lost 30 pounds and started playing recreational beach volleyball again because golf helped my knees recover.

I also have an amazing set of teammates that have turned into great friends. If it wasn't for the Let's Play Nine Scholarship, I never would have made these friends, earned a varsity letter, or found another sport to love.



inspired!

Stories and artwork from teens in the Hemophilia B Community

SPRING 2022

IN THIS ISSUE:

- Thank You, Let's Play IX
- Sibling Bond
- Teen Programs Thriving!



FELIX:
SIBLING BOND



JAMES:
THANK YOU, LET'S
PLAY IX

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!