THE COALITION FOR HEMOPHILIA B

SUMMER 2023

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

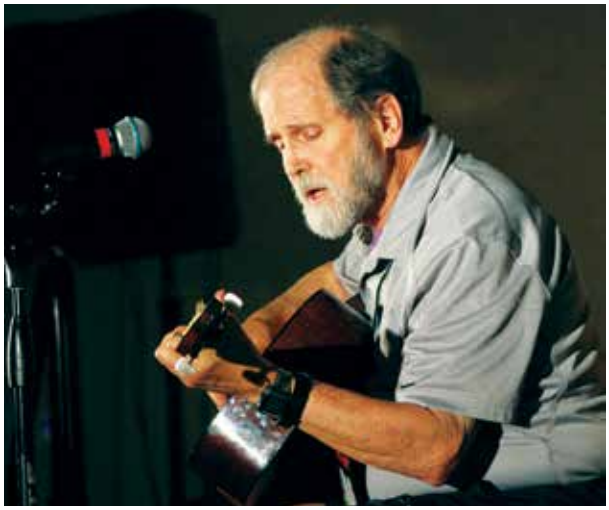
CONTENTS

THE COALITION FOR HEMOPHILIA

MISSION

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

Uncle Steve:
THE LUCKIEST GUY IN THE ROOM



BY RENAE BAKER

If you ever have some free time at a Coalition event, find Steve Long (aka "Uncle Steve") and ask him a question! He'll keep you fascinated, entertained, and impressed, and he'll leave you a little smarter. Steve tells me of his paternal grandmother - Lita Lillian Larkin Long - an Irish actress in the1880s married a Harvard Law School graduate who tried one case and said, "If that's what the law is, then screw it," and became a cartoonist for the New York World publication. He also speaks of his maternal great-great grandfather, John Henry Belter of Belter Furniture. "You've heard of the Lincoln bedroom? He made that furniture."

Steve's life falls in line with an impressive lineage of interesting careers, but perhaps more important to him is this: "Volunteerism is a Long family tradition that goes back to my parents. When we were kids, they told us, 'If you want to complain about something, go ahead and complain, but then do something about it.'"

"Something I picked up from my Jesuit education the French expression, 'noblesse oblige,' which means if you have a talent, you should develop it to help people. Steve took that attitude with him into the U.S. Air Force. Fun fact about Steve: He is the only person with hemophilia B who also has a military pension.

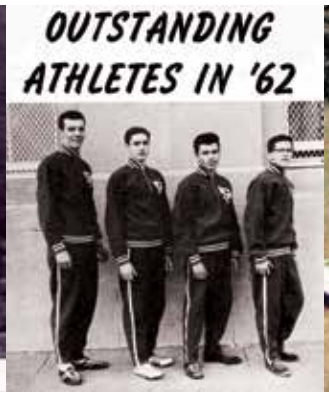
In 1972, seven years into his service in the Air Force, Steve had a wisdom tooth extracted. The surgeon slightly tore Steve's gum during the procedure. He bled for three weeks and drove from Langley Air Force Base in Virginia to Omaha, Nebraska for a three-month class. It had stopped bleeding, but when he arrived, pulled up to the gate and asked a question, it started up again for another week.

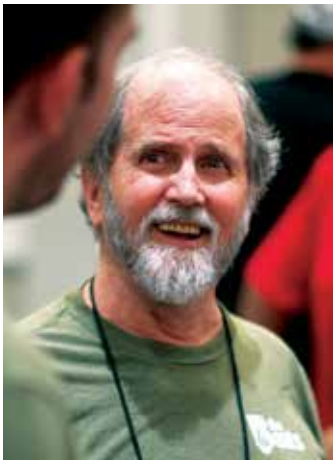
"They said, 'When you get back, you should have your blood tested.' I got back to Langley in September and they sent me to the Naval Hospital in Portsmouth, because, at the time, the VA hospital didn't have the capability. The Naval hospital screwed up the sample and it was taken again in February of '73. The Naval hospital doctor says, 'You have hemophilia B. Do you want to stay in or get out?' I said, 'I'd rather stay in!' He says, 'Fine! We won't make a big deal of it!'" Steve didn't argue. "If it had been an Air Force doctor, he probably would've said, 'You've got to be medically discharged.'"

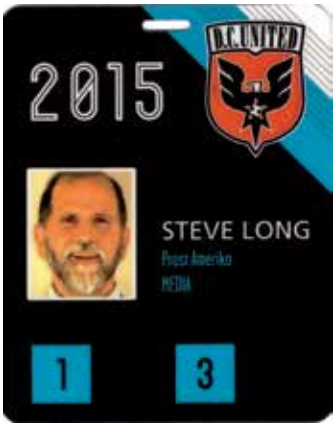
When I thank Steve for his service to our country, he says, "It was fun!" That "fun" included serving as an intelligence debriefer of a returning P.O.W. from Vietnam whose story would make a jaw-dropping movie now that (most of it) is declassified information.

"He was shot down in '67 and released in '73. He was very quiet. He told me, 'I'm only going to tell you what I personally saw.' I wound up doing 23.5 hours of tape with him. He's lived an extraordinary life. He knows it and loves to share his experiences, wisdom, and spirit.

Steve Long strains to overtake his Fordham opponent.







Adhering to Long tradition, Steve saw a need for a change, created the new position of USAF Intelligence Director, and filled it for over 27 years. You want to hear stories of harrowing escape attempts and heroism in the face of torture? Talk to Steve.

Steve's service in the United States Air Force spanned nine years as "active" and 18+ in the reserves. He started out as a Second Lieutenant, went to Intelschool in Denver, was stationed in the Canal Zone for a year and a half, and spent time in Duluth, Minnesota in the dead of winter. From there, he went to Thailand where he picked up his beloved sport of soccer, sat in front of a computer screen counting men on the Ho Chi Minh Trail, and recording sounds that were at times useful, and at others, humorous. He worked his way up to Major and might've continued to Third Colonel if he had taken the "career broadening" path of countless, boring hours working on an MBA in missile silos.

"I'm always the luckiest guy in the room." He starts to explain. "I was the base soccer coach in 1974, the year after being diagnosed. I don't have target joints," he explains. "I have target quads. So, after being hit in the thigh with a soccer ball, I'm in the hospital for three weeks with a bleed in my quadricep. In '74, they didn't know much about hemophilia, especially in the service, so they said, 'We're going try this clotting factor on you. It's called Konyne, and there is a 2% risk of hepatitis. Wrong! At that time, there was a 50% risk of hepatitis, and I got hepatitis C."

Steve explains that hepatitis C is a disease that is extremely slow-moving and debilitating. "I had VA disability because the Air Force gave me a disability. Throughout the 80s, I got free medical care through the VA."

"Back then, if you had hemophilia, it was really hard to get insurance. So, I had the VA to cover me. Not only that, but I had gone to the VA hospital here in DC any time I had a bleed. Either they were really cheap, or I had a really good hematologist. I was not given the really dangerous clotting factor at that time. They gave me fresh frozen plasma."

Steve ran the math on the number of units he was taking and determined that his risk of contracting HIV was 1/1,000. "Hep C was a blessing to me," Steve relates soberly. "It gave me free medical care. "My liver only hit cirrhosis in May of 2014. In June of 2014, I was in a special clinical trial that cured me. My liver is now back to normal."

"In my family, there are almost as many females with hemophilia as males." Steve proclaims. He is proud to be called "Uncle Steve" by his niece, Lori Long, who is very active and beloved in the community.

"I am one of the strong advocates for women bleeders," he states. To that end, he has served as Vice Chair, Secretary, and Member at Large of the Executive Committee of the HFA. He served as President of the Hemophilia Association of the Capitol Area, is currently an HACA Board Member and sits on the FAIR Time for Women Advisory Board. Among many of his talents, his has a passion for singing, playing guitar, and poetry.

About ten years ago, he started jamming with bluegrass folks who taught him that folk rhythms don't belong in bluegrass. From that experience, he met a group called King Street Bluegrass that was then playing charity jams once a month. They began playing for HACA every March, raising $500 each time and increasing awareness of hemophilia. I gave each member a red tie to reward their generosity!

He has also performed several times at The Coalition for Hemophilia B annual Beats Program. Kim Phelan said, "Steve has such a passion for all he does and he just gives his all. He had a lot of good ideas for the program, so we asked him to join the Beats Committee. He said, "Yes!" He is a blessing to us and all who know him."

Despite physical challenges from hemophilia B and just plain aging, he not only writes articles for Prost Amerika Soccer, but he continues to referee women's soccer games in the DC area.

Thank you for sharing yourself with us, Steve. We're the lucky ones!

GENERATION IX TEEN/ MENTOR PROJECT

BY BELLE GARDNER

Generation IX Project 2023, as many other Generation IX events, was a time to remember...

This year was so amazing because there were so many firsts for me and many others - first time mentoring, first time attending, and first-time infusing. The welcoming energy is always such a refreshing relief from the real world. Generation IX has always been a home away from home, a way to escape reality, and a way to make friends from all over the United States. It is always refreshing to go somewhere where there is no service because it helps you make more friends and helps you really connect with the community.

Taking place June 6-8, this event was filled with great activities including repelling down an extremely tall tree, walking along the treetops in a "challenge ropes course," being lifted 40 feet into the air by a rope and slowly brought down on the "flying squirrel," to rafting down a beautiful river. I actually got the chance to infuse while going up the "flying squirrel." It was amazing and I felt so accomplished because people infuse in such crazy and intricate places, so infusing on this was a new and thrilling experience for me.

Camp has always made me feel so welcomed and accepted because the one rule is to leave the outside world behind and accept the differences, embrace the community, and develop relationships. Generation IX has always felt like a family, and friendships that have been made at the camps continue to grow each year.

I always appreciate the effort and time the GutMonkey team puts into each event. The amount of self-reflection that they helped us with this year was so relieving. I can always rely on the fact that when I step into Gen IX, there is no judgment from any of the staff or attendees. I always appreciate The Coalition for Hemophilia B for putting on this event and Medexus for sponsoring such a wonderful program. It's safe to say that Gen IX is one of the best camp programs I have ever been to because it's so freeing and open.

Gen IX Teen/Mentorship is not only is it a time to enjoy the outdoors, but it is also a time to learn. Each activity and each discussion give the mentors and the teens ways to work together as a team to develop ways to reach the end goal. From activities like trying to get water from a bucket to a pool without moving the bucket or pool, to finding ways to simply step up and step out of our comfort zones and explore things we've never even imagined, Gen IX Teen/Mentorship is a way for teens to be mentored and train to become future mentors themselves.

COMMENTS:

"Gen IX Teen/Mentorship was a very wonderful experience. It is like getting to see the family again. I love the new ideas that we learned to take with us in our community that we are a part of, and I love the interaction between mentors and teens."

"Gen IX Teen/Mentorship is, as usual, absolutely amazing. I always look forward to going each year and seeing what GutMonkey has planned for us. Gen IX is important to me because it helps me meet people who understand what it's like to be a bleeder, and it's even more amazing that I get to meet women who are also bleeders. Gen IX means a lot to me because of the friendships and relationships I have built and continue to grow the more I attend the events. I love being an advocate for female bleeders, and Gen IX is just the place to do so. It helps you develop your sense of leadership, and with that, it allows you to grow into the mentor you know you can be."

"I really enjoyed Gen IX. It was great to connect with people that have gone through some of the same difficulties that I have. I also thought it was cool to see how far everyone had come from. I made some great connections and had an amazing time. Thank you!"

We are very thankful our generous sponsor, Medexus Pharma.

IXINITY®
coagulation factor IX
(recombinant)

DISCOVER MORE REAL PATIENT STORIES AT IXINITY.COM

IXINITY.com

MEDEXUS PHARMA

THE BEATS 2023

MORE MUSICALLY-EXHILARATING THAN EVER!

BY RENAE BAKER

"Such heart, such talent such determination, such courage, such a blast!"

To what is world class musician/singer-songwriter Joe Turley referring? The 5th Annual Coalition for Hemophilia B *The Beats* Music Program! Turley is not only a fan of the program, but also one of its instructors. He is the pianist, harmonica and sax player next to the stage for The Beats concerts.

This year's program took place from Tuesday, July 4th to Friday, July 7th at the Sheraton Music City Hotel in Nashville, and it was bigger and better than ever! "*The Beats* program has grown every year," returning participant and speaker Rick was delighted to point out. "To watch it grow and gain such momentum is, in itself, a tribute to the Coalition. So many youths today are looking for direction, yet sports are usually the only popular outlet. The Beats gives them a different opportunity." Rick tells how he has watched young, shy people come out of shells and learn to listen to, work, and grow with the community through music. "It has certainly been one of my greatest pleasures! Personally, I am just happy to be given the opportunity to get on stage with my brothers and share the love of music. What more could I ask for?"

The musical instruction offered this year has expanded from previous years. In addition to workshops in vocals (Baker and Turley), drums (Adams and Cook), guitar (Fitzhugh), and piano (Blizzard), this year's program offered instruction in brass (Mohnke), strings (Hamby), and woodwind (Biermann) instruments. The participants ranged from early teens who came in wide-eyed and unsure of what to expect, to septuagenarians who were ready, willing, and able to show the newbies the ropes. Returning teenage participants were also on hand to make them feel enthusiastic.

Previous participant Bryant marveled, "One of the great things I saw during the program was the way the younger performers banded together to support one another. Whenever I saw the teens and young adults practicing up on stage, I always got the sense that they were truly being helpful and kind with one

another when working out their songs. The result was some fantastic performances and a strengthened community!"

Rocky Williams, Coalition Community Relations Director, kept things humming. Rocky, knowing that there would be significantly more young people joining us this year, strategically planned teen icebreakers and a series of *Soundwaves and Vibes* regular check-in and bonding time to ensure that a safe, friendly and musically-optimal time was had by all. Judging by the connections made and musical collaborations, I'd say it was a success!

I believe one cannot give a moving performance without allowing oneself to be vulnerable in front of one's audience. To do that, it takes trust. It's not easy, but the concert performances of these teens and many of the adults are a testament to the strong community formed here, where hemophilia B patients bleed emotions and clot with music.

In addition to the musical clinics, The Beats hosted a medley of interesting presentations by a slate of guest speakers composed of Coalition members, outside professionals, and sponsored speakers. Returning favorite Elec Simon put us all, literally, on the same beat in his *Bucket Drum Jam*, and Yours Truly presented *Harness the Power of Stage Fright*.

There was Jeffrey Leblanc's *Physical Therapy for Musicians*, *Learning to Count* with Wayne, *Stop Waiting to Thrive* with Dan Bull, *Rhythm and Body Parts* with Rocky, *Tell Your Story* with Anna Moss and Ashley Smith, and *The Path to Resilience: Finding Confidence in Yourself and Managing Your Condition* with Chad Mitchell and Rick Starks. Max Feinstein challenged us in his *Critical Listening* workshop, and was then joined by Wayne Cook, Coalition President, leading us in a robust game of *Name That Tune*.

Max Feinstein feels the power of the program. A musician and patient with hemophilia who uses his music to advocate, Max states, "Nowhere else in the community have I felt more vital and effective as a contributor than at The Beats program. I have had the privilege of growing with the program as an educator, mentor, and friend to participants over the years, and for that, I am grateful."

Nashville singer-songwriter, rising country music star, and blood brother, Trevor Martin joined us again this year, and shared his music and stories behind his songs. Heard among the crowd, "Man! He's good!"

Happily, we were able to continue a couple of The Beats traditions this year. On night two, we could not believe our good fortune at the Grand Ole Opry to see legends Crystal Gayle, Lee Greenwood and Lorrie Morgan on stage in front of us! On day three, we

divided into two groups and went on the *Hands–On Home Recording Studio Experience* tour of Nashville record producers Jeremy Bose and Adam B. Smith.

Adam Smith is a Coalition member and is very generous with his time and talents. He not only opens his home recording studio to The Beats, but he and his wife, Tara, set up their A/V system in the main event room of The Beats. Adam makes us all sound and look good.

Throughout the program, the goal was to give everyone a chance to step out of their comfort zones and present what they had been honing during their musical workshops. The culmination of the blood, sweat, and tears was a joyful concert where the musicians delivered moving performances that highlighted their growth, talent, and hard work. The concert was live-streamed, so those in the room and community members from all over the world were able to witness the transformative power of music through this soul-feeding event.

I tried to write an article that included every act because every act was moving and beautiful, but it takes too many words, so I'll just have to pick out a few strands from the tapestry. As MC, I brought everybody together by singing *I Believe in Music* to open the show. Father-son duo Stuart and Matt played and sang a beautiful *Until I Found You*. Kristy Kiermann's woodwind class performed a *Minuet and Lullaby*.

First timer Mallory, who plays two instruments already, decided to try her voice out in the vocal class. The teen knocked our socks off with *There's a Song For Everything*. Muhammad and brother Ishmael performed with Owen in a brass trio covering jazz classics. Shardonnay gave true meaning to her solo, *Let It Go*. She gave herself to the song so completely that she took us with her, and it was an exciting moment. Bryant sang and played guitar to his mournful original *Bucketful of Stone*, which was very moving.

Newcomer Megan chose her Beats time to hone a song she was soon to sing at her sister's wedding,



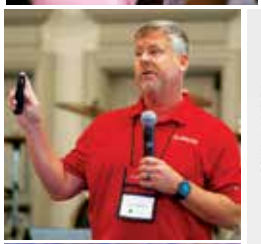




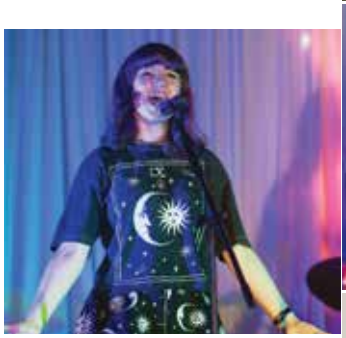









I Love the Way You Love Me. Beats Music Director Bill Gati coached her, and I'm so glad this program was here to give her the confidence to bless her sister in this way! No stranger to the Coalition but first-timer at The Beats, Lori sang a gorgeous *Blue Besides* with husband, Bryant, and band. Different configurations of bands took the stage throughout the night.

The act one closer was Izzy singing *Beautiful*. Her first year in The Beats, the teen took leaps of faith in class and trusted the fire in her belly and her increasingly close hemo family, and gave her all to this song telling us that we are beautiful, "no matter what they say." It was breathtaking and powerful. Later in the concert, she joined Nathan in *Creep* and bent notes up to the rafters. She promised that when she wins her Grammy, she will remember us at The Beats!

Act two began with Wayne and Rich leading a drum circle, which is such a great way to get people back in their seats and excited! We had cellos, duos, violin, more drums, more bands, more solos, a Max original, and we ended, arm in arm, singing *Count on Me*.

We are tremendously grateful to our sponsors: *Director Level* - CSL Behring; *Producer Level* - Novo Nordisk; *Conductor Level* - Sanofi; and *Performer Level* - CVS Specialty and Medexus Pharma. Special thanks to the sponsor volunteers who joined us and our Coalition volunteers and team: *Music Director* - William Gati; *Committee* - Wayne Cook, Adam Smith, Renae Baker, Shelby Smoak, Kim Phelan, Rick Starks; *Team and Volunteers* - Tara Smith, Farrah Muratovic and Rocky Williams.

HEMGENIX®
etranacogene dezaparvovec-drlb

FIRST AND ONLY FDA-APPROVED GENE THERAPY FOR HEMOPHILIA B

STEP INTO A WORLD OF
ELEVATED FACTOR IX LEVELS THAT LAST FOR YEARS

Patient portrayal; HEMGENIX not intended for women.

A ONE-TIME INFUSION DELIVERS GREATER BLEED PROTECTION*

37%
AVERAGE FACTOR IX ACTIVITY
SUSTAINED AT 2 YEARS

GREATER BLEED PROTECTION
VS. ROUTINE FACTOR IX PROPHY*

94%
OF PEOPLE DISCONTINUED FACTOR IX PROPHY AND
REMAINED PROPHY-FREE†

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

†51 out of 54 people remained free of continuous routine factor IX prophylaxis (prophy).

Step into a new world today at HEMGENIX.com

IMPORTANT SAFETY INFORMATION

What is HEMGENIX?

HEMGENIX®, etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

Please see full prescribing information for HEMGENIX.

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

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

HEMGENIX is manufactured by uniQure Inc. and distributed by CSL Behring LLC.
HEMGENIX® is a registered trademark of CSL Behring LLC.

www.CSLBehring.com **www.HEMGENIX.com** **USA-HGX-0296-DEC22**

CSL Behring

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use HEMGENIX safely and effectively. See full prescribing information for HEMGENIX.

HEMGENIX® (etranacogene dezaparvovec-drlb) suspension, for intravenous infusion
Initial U.S. Approval: 2022

----------INDICATIONS AND USAGE----------
HEMGENIX is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who:
- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or
- Have repeated, serious spontaneous bleeding episodes.

----------CONTRAINDICATIONS----------
None.

----------WARNINGS AND PRECAUTIONS----------
- Infusion reactions: Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or interrupt administration. Re-start administration at a slower infusion once resolved.
- Hepatotoxicity: Closely monitor transaminase levels once per week for 3 months after HEMGENIX administration to mitigate the risk of potential hepatotoxicity. Continue to monitor transaminases in all patients who developed liver enzyme elevations until liver enzymes return to baseline. Consider corticosteroid treatment should elevations occur.
- Hepatocellular carcinogenicity: For patients with preexisting risk factors (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age), perform regular (e.g., annual) liver ultrasound and alpha-fetoprotein testing following administration.
- Monitoring Laboratory tests: Monitor for Factor IX activity and Factor IX inhibitors.

----------ADVERSE REACTIONS----------
The most common adverse reactions (incidence ≥5%) were elevated ALT, headache, blood creatine kinase elevations, flu-like symptoms, infusion-related reactions, fatigue, malaise and elevated AST.

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring at 1-866-915-6958 or FDA at 1-800-FDA-1088 or *www.fda.gov/medwatch*.

----------USE IN SPECIFIC POPULATIONS----------
No dose adjustment is required in geriatric, hepatic, or renal impaired patients.

Based on November 2022 version

THE COALITION FOR HEMOPHILIA B

ETERNAL SPIRIT AWARD GALA HONORS DONALD D. AKERS JR, JD AND MARK W. SKINNER JD

BY RENAE BAKER

On August 10, 2023, community members and partners of The Coalition for Hemophilia B gathered at the Mansion at Oyster Bay to honor two extraordinary allies of the bleeding disorders community with the Eternal Spirit Award. The atmosphere was festive, fun, and heartwarming. The cocktail hour was enhanced by the beautiful singing and guitar playing of Emma Jude and delicious hors d'oeuvres. On the dining room stage, a cappella trio VocalsouL set a Motown groove as guests mingled before sitting for mouth-watering entrees.

The theme of this year's gala dinner was, "We Rise By Lifting Others," and our two honorees have exemplified this principle on multiple levels! Mark W. Skinner, JD and Donald Akers, Jr., JD, both attorneys, have harnessed their legal knowledge and deep commitment to the community to effect change on a national and international scale. Their profound impact on the world of hemophilia has immeasurably touched the lives of countless individuals and families.

The Eternal Spirit Award is a prestigious honor bestowed upon individuals recognized by the Coalition for their generous spirit toward the bleeding disorders community and their consistent efforts to enhance the well-being of this community. This year's honorees' resumes would take a small forest to print on paper!

Donald D. Akers, Jr. JD, affectionately known as "Donnie," has become an integral part of the community through his unwavering commitment. As a law partner and former District Attorney in Louisiana, Donnie dedicated his time and talents to the hemophilia community by providing pro-bono legal advice and presentations. He also played a pivotal role in founding the Hemophilia Alliance Foundation, driving advancements in research, awareness, and crucial resources for those affected by this condition. Through his tireless advocacy, he has given voice to the voiceless, championing the needs of patients and empowering them to lead fuller, more independent lives. Despite not having hemophilia himself, Donnie's involvement in the community began through his







friendships with those who did, and even earned a "Big Stick Award" by poking his own vein.

A longtime close friend and Coalition[1] member, Carl Weixler, paid tribute to Donnie via video at the awards dinner. Sitting in his easy chair, he referred to his friendship with Donnie as a "hilarious ride!"

"Donnie, I know you don't like getting awards, but tough - - - !" Carl laughed, "This one you get, and you deserve!" Referring to Donnie's introduction to the bleeding disorders community, Carl continued, "Little did you know, when you met Chuck back in college, that you would have such a turn in your life and dedicate so much of it to helping hemophilia people!"

Kimberly Haugstad also shared heartfelt words, highlighting Donnie's profound impact on the hemophilia community, his ability to truly listen and understand, and his unwavering commitment to helping others. She pointed out how he continues to "come back," and that he's helped many individuals in the community, including many who were in the audience that night. "Donnie is a perfect example of what The Coalition For Hemophilia B is all about." Kimberly said, "Heart, compassion, desire to teach and love."

Donnie was then given the beautiful Eternal Spirit Award, by Coalition President, Wayne Cook, and a very special, one-of-a-kind quilt featuring a fish created by Coalition member, Alma Jewel.

The next honoree was Mark W. Skinner, JD. Mark is fascinated by penguins and has traveled the world to see them. At last count, he had seen thirteen of the eighteen species of them! I wanted to lead with that humanizing, even "cute" aspect of him, because his accomplishments, stalwart leadership, and inspirational example he sets are quite enough to make anyone intimidated.

Mark is the President and CEO of the Institute for Policy Advancement Ltd in Washington, DC. He was born with hemophilia and is a liver recipient. Mark has made patient-centered outcomes research his life's work and has led the World Federation of Hemophilia and the National Hemophilia Foundation. He is part of the Scientific Advisory Council for MASAC, the Board of Directors for NORD, and is a member of ICER, to name just a few of the areas in which Mark is very active to improve the quality of life for patients with bleeding disorders and rare disorders.

Mark's story is one of the power of perseverance through adversity, and today is a shining example of how the human spirit can find a way to not only accomplish dreams and goals, but also to make life better for others along the way. Today, he is living his dream of working internationally.

Dr. Glenn Pierce, Vice President, Medical, at the World Federation of Hemophilia, paid tribute to Mark via video. "I first met Mark Skinner twenty-eight years ago, shortly after he joined the NHF board and I finished my second term as president," Dr. Pierce began. "I was

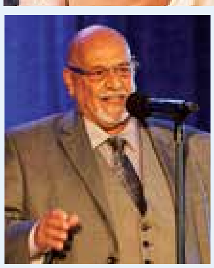


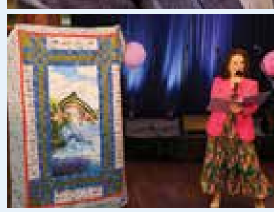
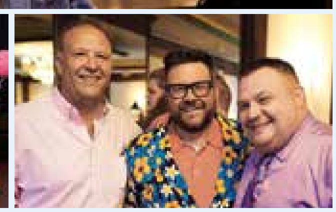


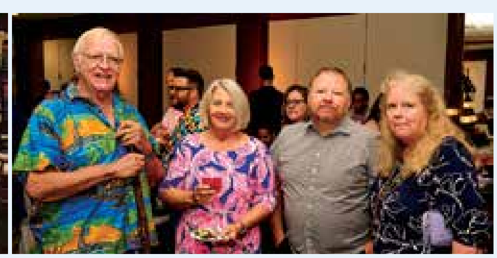
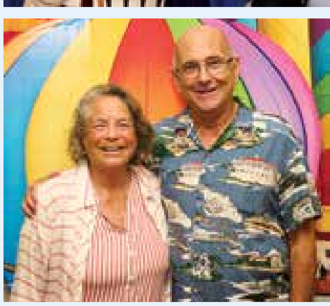









immediately struck by his grasp of blood safety issues. During his time with NHF, he has continued to play a significant role in advocating for a safe product supply around the world."

Dr. Pierce went on to speak about Mark taking on the role of president of WFH and being instrumental in getting better care to bleeding communities around the world, as well as making advancements here in the United States. "He has been the best strategic-thinking partner, and his impact endures through WFH today." Dr. Pierce then cited Mark's continuing energetic advocacy for women and girls, patient-relevant outcome measures, and shared decision-making in health equity within the bleeding disorders community. During a recent surgery, Dr. Pierce recalled Mark's mantra "nothing about me without me," and realized that, although the work is far from done, Mark has helped create the tools that can facilitate the conversations around shared decision-making.

Wayne Cook followed the powerful video with some words of his own, heralding a few of Mark's many accomplishments and recalling his time serving with him on the board at NHF. "Mark was a great resource that I was able to reach out to who could help me understand our community more. I remember a part of one particular conversation where he gave me some advice that has always stuck with me throughout the years. That was to always stay strong and speak from the heart, and if you always remember this, you will become a stronger leader." Then Wayne presented Mark with the Eternal Spirit award, saying, "Mark has always been one of the most admired individuals that I have ever met. All he has done for this community, nationally and globally, is a remarkable testament to his passion for our community and improving our lives."

Mark was also given a blanket, also hand quilted by Alma Jewel. He was thrilled to learn that it featured penguins! The appreciative audience sat back down, after their standing ovation for both recipients, and Craig Drohan took the stage to shake the hands of the student awardees of the Dr. William N. Drohan Scholarship, which was founded in honor of his late father. This year's recipients are:

James Hensley: James will be attending Virginia Tech in the fall, majoring in pre-med Sociology with a Psychology minor in the honors program. His goal is to become a doctor focusing on rare diseases and healthcare disparities.

Emily Marlatt: Emily is a Nursing major attending Towson University.

Seth Pastakos: Seth is a senior at St. John's University, studying Business Healthcare Finance. He has been on the Dean's List and actively volunteers in the hemophilia B community. Seth's passion lies in helping people achieve their potential and honoring his country.

Steven Sclafani: Steven is attending Utica College, where he is studying Physical Therapy with minors in Entrepreneurship and Aging Studies.

Ella Wood: Ella is majoring in Psychology at the University of Iowa, aspiring to become a social worker and positively impact the lives of others.

It is heartening to think that these young scholars with aspirations to alleviate pain in various healthcare fields, have been inspired by the exceptional individuals they have seen around them. Honoring extraordinary hearts and minds like Donald D. Akers, Jr. JD, and Mark W. Skinner, JD, is a double pleasure, as they undoubtedly serve as role models shaping the futures of these aspiring young individuals.

Following these inspiring moments, a heartfelt testimonial was shared by a mother and her young adult son who has hemophilia B. They highlighted the vital role played by The Coalition for Hemophilia B in rectifying a dire situation and praised the relentless dedication of Kim Phelan in resolving it, sharing "she never gives up until it's resolved. For that, we are thankful."

We extend our gratitude to all who joined us in celebrating our honorees and contributed to the William N. Drohan Scholarship Fund and our Patient Assistance Fund. Your belief in our work, which positively impacts so many, is deeply appreciated.

Special thanks are also extended to our sponsors, whose support makes a significant difference!

Diamond

CSL Behring · MEDEXUS PHARMA · sanofi

Gold

CVS specialty · The Alliance Pharmacy · novo nordisk · Hemophilia Alliance

Friends

accredo · Bruce A. Gordon, CPA PC

Good Gumming:
Floss and Communication

INTERVIEW WITH ALICIA UNGER AND DOUG HUTCHINSON BY RENAE BAKER

Have you ever asked your dentist or hygienist if you floss correctly? Me neither! But, Coalition for Hemophilia B member Doug Hutchinson, DDS, says it can be an insightful and extremely helpful question. "It's a great conversation starter!" For the hemophilia community, communication is very important. And not just with your health providers.

Kismet brought Doug to Alicia's salon chair nine years ago. They became instant friends as she cut his hair and they talked. One of the things they had in common was an awareness of the value of communication. Alicia would have benefitted from more communication from her family about their history of hemophilia B. As a child, she lived in the country and spent a lot of time outdoors. "We just ran wild all the time. I can't tell you how many times I cracked my head on something, but you just pick yourself up, dust yourself off…" she laughs. "Unfortunately, I wasn't aware that I might have hemophilia."

She says, "My mother's brother was the first in the family to have hemophilia. We lost him to bad blood in the eighties." She cites the Ryan White story about the young boy with hemophilia who contracted AIDS via a tainted blood infusion, was subjected to unfathomable hate, and who fought for a year and a half to be allowed to go to school in the mid 1980s. "Because of that, my family took the approach of not talking about it." Alicia did get tested to see if she was a carrier.

"Again, unfortunately, because we didn't talk about it, there was not much education around it and I was tested for the wrong hemophilia." She was only tested for hemophilia A and subsequently told that she was not a carrier. Years later, when Alicia was in labor for her first baby, Landon, she had trouble progressing and ended up having an emergency C-section.

"It was January, RSV season, so they wanted to keep him for five days of antibiotics as a preventative for any possible lung issues." Alicia was looking forward to bringing her baby home with her on day six. "They did his circumcision, and he never quit bleeding. That's how we figured out he has hemophilia."

They were immediately transported to Children's Hospital where Landon was treated with factor. Landon, now 12 years old, was diagnosed with severe hemophilia B. And Alicia? "They still like to classify me as a 'symptomatic carrier,' but I do require factor for any surgery trauma because my bleeding is prolonged." She also describes her bruises as much larger, more frequent and longer lasting than Landon's. "My goodness!" She exclaims, "They hang on forever!" Grateful for the improvements in the medications, Alicia is happy to report that, these days, Landon is doing "amazing" and treating every two weeks on a prophylactic basis.

Landon's birth took place before Alicia met Doug. That fateful day when he sat down in her salon chair, the conversation turned to Landon, who was about three years old at that time. Alicia was surprised to learn that Doug knew about hemophilia and other bleeding disorders. Doug had studied at the School of Dental Medicine at the University of Pittsburgh and was already a practicing dentist in a private practice he shared with one partner. Eventually, they married and expanded their family of three to four with the birth of Kylie, now seven years old.

"If you have a bleeding disorder, communication with your dentist is paramount," Doug stresses. With Dental groups (DSO's) being so common today, some people become weary of filling out forms for what feels like every visit and – in some cases – the patient withholds

information. Doug finds this disheartening and assures us that your dentists want to know about your bleeding disorder.

"Your dentist wants to know the severity of diagnosis, your inflammation and bleeding risk, and to make sure things are coordinated properly with your hematologist." For your comfort and the comfort of the doctor, you may want to consider not only filling out the forms but asking for a consultation with the dentist, either via telemedicine (Zoom) or face-to-face. When seeing your dentist and hygienist, don't be afraid to ask questions. "Am I flossing correctly?" is a good one. Dental professionals are happy to watch how you do it and demonstrate correct methods. "Tell-show-do" is a method used to demonstrate to kids and adults alike.

Doug and his partner have been co-owners of their practice in Northwest Pennsylvania since 2011. His business model is "If you are coming to my office, you will be seeing me." It gives his patients comfort to have that personal relationship with him, but not everybody is able to see a dentist in a solo practice. Doug advises:

"Whether you're talking about solo or group practice, the most important thing is that the patient and the doctor have a level of comfort. You need to find a provider who feels comfortable treating and understanding your conditions. If you have physical or mental special needs, you want to make sure you go to a doctor who feels comfortable with your need set. The patient should feel that they are in a good, comfortable setting and they are with a doctor who is knowledgeable and understanding of their condition." Doug points out he isn't comfortable extracting impacted wisdom teeth, so he refers patients who need extractions to an oral surgeon. Likewise, if a doctor isn't knowledgeable about your special needs and is uncomfortable treating you, it is best for all concerned to find another. Dentists don't always invite consultations to determine these comfort levels.

"It's a good idea to seek that out," Doug points out. "That way, the relationship can start out on the right note. You've got to be your own advocate. The Zoom appointments are more available than ever since the pandemic, and there are a lot of dentists who have adopted that telehealth model."

Doug was motivated to go into general dentistry in large part because of the generational and familial relationships that get nurtured through the profession. He finds it gratifying to have patients whose baby teeth he tended grow up and bring their children in. "Sometimes you'll have four and five generations of one family. The rapport you can have is very rewarding. That extends to taking care of my own family– parents, aunts, uncles, and yes, he is Alicia's, Landon's and Kylie's dentist.

"If we want to help out our overall situation," Doug says with fatherly wisdom, "oral hygiene is key. Limit soft, sticky food, sweet teas and energy drinks." He explains we don't want to keep our mouths constantly in an acidic state. Add fermentable sugars to acid and, "that's when things go wildfire!" Coffee with cream and sugar is an example.

"Many people don't realize how extremely acidic Gatorade is." Alicia adds. "And it's not so much about total consumption as it is about frequency," Doug continues. "It's better to drink acidic drinks in one sitting than to nurse it all day long, keeping your mouth in a state of acidity." To sum it up, "Stay on top of your oral hygiene so bigger procedures are not necessary." Which leads us back to flossing. "You don't want to shy away from those areas. The bleeding occurs where there is a buildup of bacteria." Doug explains. Regular flossing will help prevent that. "The best advice I can give is to be serious about your preventative care so you don't need the more serious procedures. You want to keep trying to promote good oral health."

Alicia admits to having bleeding gums. "I don't know exactly what Doug would say about this," Alicia confesses, "but I've adopted the Water Pik instead of floss. I find it less invasive and I have fewer bleeds."

To which Doug responds, "I would say the Water Pik is an adjunct. I've been asked many times, what's better, traditional floss, floss-on-a-stick or Water Pik? Regular floss works the best because you can contour it, wrap it around the tooth, get underneath the gums and get friction. But if I know someone isn't going to use traditional floss but they will use floss-on-a-stick, well then floss-on-a-stick it is!" Doug believes that flossing and adding the Water Pik to the routine is the best way to go. "It's a great adjunct. A healthy adjunct."

Alicia has a word of advice to share, too: "Keep an open line of communication and do not be afraid to ask questions. It's a good rule for life in general, but it definitely applies to dental health!"


Connected to milestones.
Life with hemophilia shouldn't be defined by limits. Through personalized education and empowering resources, we're focused on making more possible for you and the people you love.
Let's connect.
rarebloodddisorders.com
@HemophiliaCoRes
1-855-SGZHEME
© 2022 Genzyme Corporation. All rights reserved
Sanofi is a registered trademark of Sanofi or an affiliate.
MAT-US-2019890-v3.0-07/2022
sanofi

HEMOPHILIA NEWS

BY DR. DAVID CLARK

NHF IS NOW NBDF

8/17/23 At its 2023 Bleeding Disorders Conference, the National Hemophilia Foundation announced that they are changing their name to the National Bleeding Disorders Foundation. NHF was founded 75 years ago to help people living with hemophilia. Over time, it has evolved to work with other bleeding disorders such as von Willebrand Disease, rare factor deficiencies and platelet disorders. Therefore, this is not a new mission but a name change to reflect what the organization is actually doing. As Dr. Len Valentino, the current CEO, said, "No matter what [bleeding] disorder you have, you will find a home in the National Bleeding Disorders Foundation." They also have a new logo and a new tagline: Innovate | Educate | Advocate. [NBDF press release 8/17/23]

CLINICAL COURSE OF LIVER CANCER IN HEMOPHILIA

8/14/23 Hemophilia patients are known to develop liver cancer (hepatocellular carcinoma) at a younger age than is typical for those without hemophilia. However, not much else is known about the clinical course of liver cancer in people with hemophilia. Most liver cancer in hemophilia results from hepatitis infections that were caught from plasma-derived factor products before they became virologically safe in the late 1980s.

A new study by a group in Japan suggests that the clinical course of the cancer and its prognosis do not differ much from those in non-hemophilia patients. They looked at 22 patients (all male; 20 As, 2 Bs) with hemophilia and liver cancer who were diagnosed between 2003 and 2021. Compared with data from a nationwide survey of liver cancer patients, people with hemophilia were diagnosed at an earlier average age, 63 years vs. 72 years for those without hemophilia. All of the patients received standard treatments. The median survival after treatment for the patients with hemophilia was 6.4 years (range 0.9 – 18.7), compared to a median survival of 5.8 years for patients without hemophilia. [Matsuda N et al., Eur. J. Gastroenterol. Hepatol., online ahead of print 8/14/23]

FRAGILITY FRACTURES IN HEMOPHILIA

5/28/23 Now that people with hemophilia are living longer, they are experiencing more diseases of old age, such as osteoporosis and fragility fractures. Osteoporosis is decreased bone mineral density. The bone loses calcium and actually can become porous. A fragility fracture is just what it sounds like, breaking a bone because it is too weak. People with hemophilia have an increased risk of low bone mineral density (BMD), for unknown reasons. Just as the body is constantly making new proteins and removing the old ones, new bone is constantly being made and old bone is being broken down. The cells involved are osteoblasts, which make bone, and osteoclasts, which break down bone.

Osteoporosis is multifactorial, that is, it has a number of causes. Some studies suggest that both factors VIII and IX may be essential for bone health, probably through their activation of thrombin during clotting. Thrombin can activate osteopontin, a protein that is important for osteoblasts that build bone. Other studies have shown that the factor VIII-von Willebrand protein complex is important in inhibiting a protein called RANK, which inhibits a pathway that osteoclasts use to break down bone. People with hemophilia have also been shown to have lower levels of sclerostin, a protein that promotes bone formation. Joint bleeding and damage may also play a role.

The researchers found that the risk of fracture in hemophilia increased with disease severity and age (with the risk doubling after the age of 31), with an increase of about 1.3% per year of age. They also found that the incidence of fragility fractures in hemophilia patients was significantly higher than in the general population. Bone loss in hemophilia patients starts in childhood. This is one reason that people emphasize the importance of physical activity and exercise, which can help build bone. Bone grows in response to stress.

Another aspect is that most hemophilia patients don't know the condition of their bones. Osteoporosis testing usually occurs only at the time of the first fragility fracture. Studies also suggest that factor infusions may help reduce osteoporosis. So if you're an older person who isn't on prophylaxis because you don't bleed much, you might want to rethink that. You might not

need factor as much for clotting as to keep your bones healthy.

The authors have this advice: "Management of bone disease in PWH [persons with hemophilia] should begin in childhood to achieve the best possible PBM [peak bone mass], encouraging low-impact resistance training throughout life, regular assessment of BMD [bone mineral density] and fracture risk, and avoidance of smoking, alcohol consumption and obesity. It is, therefore, important to remember that PWH are at increased risk of falling due to the combined effects of arthropathy, reduced muscle strength and balance problems, so, an exercise program focusing on strength, balance and motor coordination should be encouraged at all ages." [Alito A et al., Int. J. Mol. Sci., 24:9395, 2023]

EFFECT OF MOVEMENT ON FACTOR LEVELS IN JOINTS

6/27/23 Hemophilia A, a deficiency of factor VIII, and hemophilia B, a deficiency of factor IX, have similar symptoms because factors VIII and IX work together to activate factor X in the clotting system. If you have a deficiency of either factor, factor X doesn't get activated as much, and your blood doesn't clot well. Bleeding into the joints is a consequence of both conditions, but we still don't understand why that leads to joint damage. A group of Israeli researchers has recently explored the fate of factors VIII and IX in the joints.

When a joint is moved, it has a tendency to bleed. In a person without hemophilia, the clotting system responds immediately to stop the bleeding so only a micro-clot is formed. In people with hemophilia, bleeding occurs for a longer period of time, which introduces blood into the joint. Substances in the blood probably start reactions that lead to joint damage. We'd like to know why there is bleeding at all – especially in the joints - and what causes it. Therefore, the researchers looked at what happens to the clotting factors in the joint space.

One of the first things they found is that both factors tend to break down in the joint, but high levels of both tend to protect each other. Thus, if you have high levels of both VIII and IX in the joint, both factors tend to be stable, but if you have a low level of either one, both tend to degrade.

Factor VIII appears to be degraded in the joint by thrombin, the final enzyme generated in the clotting system. An enzyme is a protein that causes a chemical reaction. Thrombin converts the protein fibrinogen to fibrin, which sticks to other fibrin molecules to form a clot. Thrombin also affects many of the other clotting factors. It activates factor VIII but also degrades the activated factor VIII. Thrombin probably is the cause of decreased factor VIII levels in the joint. Joint bleeding triggers the clotting cascade to produce thrombin, then degrades the activated factor VIII to produce lower levels in the joint space.

Factor IX, on the other hand, is degraded in the joint, but not by thrombin. It might be degraded by other enzymes in the joint space, mainly by loss of the factor VIII that was protecting it. The study also showed that both factors are degraded in the skeletal muscles associated with the joints.

One of the unanswered questions in this study is why joints and muscles that are only intermittently in movement tend to bleed while muscles that undergo constant movement, like the heart, don't bleed. All of this is to better understand what's really going on in hemophilic joints. [Cohen H et al., Int. J. Mol. Sci., 24:10731, 2023]

CURRENT PRODUCTS FOR HEMOPHILIA B TREATMENT

BY DR. DAVID CLARK

The large number of products available for treatment of hemophilia B increases the chances that every patient can find a product that works well for them. Selecting the best product may be a process of trial and error, but working with an experienced hemophilia treater can shortcut the process. If you think you could be getting better results, don't hesitate to ask your physician.

Many new families may not be aware of the large number of products available for treatment of hemophilia B. Other patients and families also may want an update on the many newer products available. This is a brief survey of the products currently available in the U.S.

One of the most important principles in medicine is that every patient is different. Although we all share many similarities, we also have unique genetic and medical backgrounds. An advantage of the large number of products available is that a patient who does poorly on one product might have better results with another. Patients are encouraged to work with their physician to find the best product for their needs.

Currently, most hemophilia B patients, except those with inhibitors, are treated with factor replacement products. These products contain the normal factor IX protein to replace the defective factor IX molecules produced by their own bodies. They all require periodic intravenous infusions to maintain the amount of factor IX in the patient's blood at a level required for good hemostasis (adequate clotting). There are improved factor IX products currently under development, as well as a number of non-factor products.

The current products fall into five general categories: standard half-life (SHL) factor IX products, extended half-life (EHL) factor IX products, inhibitor treatment products, gene therapy and ancillary products. Note that all of these products are only available by prescription.

Standard Half-Life (SHL) Products

The SHL factor IX products currently available in the U.S. are listed below in Table 1.

The four current SHL products all consist of normal human factor IX. They are all descendants of the original plasma-derived concentrates that were developed in the 1960s. The original products were called Factor IX Complex or Prothrombin Complex. They were mixtures of several clotting factors including factors II, VII, IX, and X plus the anticoagulants protein C and protein S. These proteins all have similar chemical structures, which makes them difficult to separate from each other.

The complexes were a huge leap forward in treatment of bleeds, but it was soon apparent that they could not be used in large amounts or for prolonged periods of time because they would cause thrombosis, dangerous unwanted clotting. This prevented their use for prophylaxis or surgery. Factor IX complex products are still on the market but should not be used for hemophilia B treatment because of their safety risks. They are currently used mainly for treatment of liver disease.

AlphaNine, the sole remaining plasma-derived product, was one of the first products to contain highly purified factor IX without the other factors. It has proved to be safe from thrombotic complications and is still used by a number of patients. The other big change in factor IX products was the introduction of methods for viral inactivation and removal, which happened in the mid-to-late 1980s.

Prior to the introduction of those methods, plasma-derived products often were contaminated with infectious agents like hepatitis B and C and HIV, the AIDS virus. Plasma-derived products are now considered completely safe. There have been no incidences of viral transmission from clotting factor

Table 1: Standard Half-Life (SHL) Factor IX Products Currently Available in the U.S.

Brand Name	Generic Name	Manufacturer	Type
AlphaNine SD	Coagulation Factor IX (Human)	Grifols Biologicals	Plasma-derived
BeneFix	Coagulation Factor IX (Recombinant)	Pfizer/Wyeth	Recombinant – CHO cells
Ixinity	Coagulation Factor IX (Recombinant)	Medexus/Aptevo	Recombinant – CHO cells
RIXUBIS	Coagulation Factor IX (Recombinant)	Takeda/Baxalta	Recombinant – CHO cells

products since the late 1980s, or from any other plasma-derived products since the early 1990s.

One of the main reasons for introduction of recombinant products was viral safety – to eliminate the dependence on human plasma for these products. Another was the ability to produce unlimited amounts of a product without dependence on the limited supply of plasma. Recombinant products are made in animal cells that have been genetically engineered to produce the desired protein. All three recombinant SHL factor IX products are made in Chinese hamster ovary (CHO) cells that are grown in large tanks in a process called cell culture.

A little-appreciated fact is that all recombinant products are also treated for viral inactivation and removal. Although the cells used for cell culture are thoroughly screened to make sure they are safe, it was discovered early on that some of these cells may contain hidden virus genes in their DNA. These viral genes could under some production conditions be "turned on" and introduce infectious viruses into the products. Now, in addition to having manufacturing steps to inactivate and remove any viruses, every batch of product, whether plasma-derived or recombinant, is also tested to make sure there are no known infectious agents present in the final product.

Most hemophilia B patients use recombinant products, but there are some patients who still use **AlphaNine** because it works better for them. The reason for this is unknown, but there are two important possibilities. One is that the recombinant products only contain a single version of factor IX, the most common variant, which is considered "normal" factor IX. However, plasma, which is collected from thousands of donors, contains a whole range of factor IX variants. Many people have small mutations in their genes and produce a factor IX that isn't modified enough to produce hemophilia, but still has some changes that may make it work better or worse in hemophilia patients.

Another possibility is that the animal cells used in cell culture glycosylate the factor IX product differently than human cells do. Many of the clotting factors, including factor IX, are glycosylated after the protein is made. That means that they have carbohydrate chains attached to various parts of the molecule. The carbohydrate chains are strings of sugar molecules linked together (glyco comes from the Greek word for sweet or sugar). There are many different types of sugars beyond what we think of as "table sugar." We don't completely understand the reasons for these sugar chains, but we know that human cells add on different combinations of sugars than CHO cells do, for instance. These differences may cause variations in how well the products work in some patients.

Extended Half-Life (EHL) Factor IX Products

The EHL factor IX products currently available in the U.S. are shown below in Table 2. All of the EHL products are recombinant.

The body is constantly removing existing copies of proteins from the bloodstream and replacing them with new copies. This is part of the process for keeping the body in good working order. The factor IX proteins introduced by the various products are subject to the same removal process. The half-life is the amount of time it takes for half of the protein to be removed.

The typical half-life of normal factor IX is 23–25 hours, although that can vary significantly from person to person. The SHL products all have half-lives similar to that of normal factor IX derived from plasma. That means that a patient using an SHL product has to infuse new factor IX every three days or so. (Note that some of the SHL products, like **BeneFix**, have developed alternate dosing schemes using higher doses to keep factor IX levels in the needed range for a week or more.)

The EHL products use various methods to keep their factor IX in circulation for longer periods of time. These products can be dosed at intervals of one to two weeks, again depending on the patient's individual response.

Alprolix contains factor IX molecules attached to the Fc region of an antibody molecule. The body has a special mechanism to keep antibodies in circulation longer than most other proteins. Antibody molecules are shaped like a Y. The two arms of the Y are the Fab regions of the molecule that bind to viruses, bacteria and foreign proteins to remove them from circulation. The base of the Y is the Fc region that attracts immune

Table 2 – Extended Half-Life (EHL) Products Currently Available in the U.S.

Brand Name	Generic Name	Manufacturer	Type
Alprolix	Coagulation Factor IX (Recombinant), Fc Fusion Protein	Sanofi	Recombinant – HEK cells
Idelvion	Coagulation Factor IX (Recombinant), Albumin Fusion Protein	CSL Behring	Recombinant – CHO cells
Rebinyn	Coagulation Factor IX (Recombinant), GlycoPEGylated	Novo Nordisk	Recombinant – CHO cells

cells to destroy anything that the arms bind to. The Fc region is also the part of the molecule that interacts with the system that keeps antibodies in circulation longer. It turns out that linking factor IX to an Fc molecule also keeps the factor IX in circulation longer.

Another aspect of **Alprolix** is that it is made in cell culture in human embryonic kidney (HEK) cells. Using human cells to produce the product potentially produces a factor IX that is glycosylated (has carbohydrate chains attached) more similarly to the factor IX molecules made naturally in the human body. Whether that actually improves the performance of **Alprolix** is unknown.

Idelvion uses a similar method. Its factor IX is linked to an albumin molecule. Albumin is the most prevalent protein in plasma. It thickens the plasma and also carries many other molecules around in the circulation. There is also a special mechanism in the body to keep albumin in circulation longer. Linking factor IX to albumin also improves its half-life.

Rebinyn uses a different method to keep its factor IX in circulation longer. Polyethylene glycol (PEG) is a long water-soluble polymer that has found many uses in medicine including improving the half-lives of drugs.

Rebinyn uses factor IX with PEG chains attached to the ends of the carbohydrate chains described above in the SHL section. These long PEG chains wave around and coil up randomly around the factor IX molecule. They form a loose shell that tends to hide the factor IX molecules from the liver cells that normally remove factor IX from circulation.

The SHL products have small differences from between other, but even those small differences can cause one product to behave differently from another in a given patient. Some patients even do better on plasma-derived products than on recombinant products. Meanwhile, the EHL products use very different methods to extend their half-lives as well as having small differences in the underlying factor IX molecule like the SHL products. Patients should have access to all of the available products so they can find the one that works best for them. There is no one size fits all. Each patient is unique.

INHIBITOR TREATMENT PRODUCTS

The products used for treatment of patients with inhibitors are listed below in Table 3.

Inhibitors are antibodies that the immune system produces because it thinks that an infused factor IX product is a foreign protein that could be dangerous. Some of these, known as non-neutralizing antibodies, bind to factor IX but don't interfere with its function. Inhibitors are neutralizing antibodies that bind to factor IX in locations on the molecule that prevent it from working. Inhibitors also occur against factor VIII in hemophilia A where they are a major problem. Inhibitors occur much less frequently in hemophilia B. Only about 3–5% (the numbers are hard to pin down) of hemophilia B patients develop inhibitors, but when they do, it can be a very serious problem.

Factor VIII inhibitors can often be eliminated by a process called immune tolerance induction (ITI). However, ITI works poorly in many hemophilia B patients with inhibitors. In addition, many hemophilia B inhibitor patients also develop allergic reactions to factor IX including anaphylaxis, a severe reaction that can be life-threatening. Hemophilia B inhibitor patients are also prone to a kidney disorder called nephrotic syndrome. Most hemophilia B inhibitor patients end up just living with their inhibitor and using bypassing agents to treat bleeds.

Inhibitor treatment products are called bypassing agents because they trigger other parts of the clotting system, bypassing the factor VIII/factor IX step. They work for both hemophilia A and B inhibitor patients, but they don't work as well as a regular factor product would work in a hemophilia patient without inhibitors. They have fairly short half-lives, requiring frequent infusions to treat bleeds, and are expensive. They can be used prophylactically, but most patients just use them for on-demand treatment of bleeds.

FEIBA is a plasma-derived version of factor IX complex in which the clotting factors have been activated using a proprietary method. It is used by some hemophilia B inhibitor patients, but because it contains factor IX, it carries a risk of allergic/anaphylactic reactions. The way **FEIBA** works is not fully understood, but it contains activated factor VII like the other two bypassing agents. The other activated factors in **FEIBA** probably also trigger other parts of the clotting system.

Table 3 – Inhibitor Treatment Products Currently Available in the U.S.

Brand Name	Generic Name	Manufacturer	Type
FEIBA	Anti-inhibitor Coagulant Complex	Takeda/Baxalta	Plasma-derived
NovoSeven RT	Coagulation Factor VIIa (Recombinant)	Novo Nordisk	Recombinant – BHK cells
Sevenfact	Coagulation Factor VIIa (Recombinant)-jncw	HEMA Biologics	Recombinant – transgenic rabbits

NovoSeven is a recombinant activated factor VII product. The overall clotting system consists of two pathways, one that depends on factors VIII and IX, and the other that depends on factor VII. Adding activated factor VII triggers that alternative pathway to eventually form a clot. Note that **NovoSeven** is produced in cell culture using a different microorganism, baby hamster kidney (BHK) cells. The choice of cell type is usually determined by which type works best to produce a particular product.

Sevenfact is a new recombinant activated factor VII product. It is similar to **NovoSeven** but made by a completely different process. For **Sevenfact**, rabbits have been genetically engineered to produce factor VII in their milk. The rabbits are milked and the milk purified to capture the factor VII, which is then activated to produce the final product. Producing a protein in a genetically-engineered animal is called transgenic production. It has also been used for other pharmaceutical products approved by FDA. Its advantage is that very large amounts of protein can be produced at relatively low cost. It was originally seen as a way to produce high-quality but lower-cost products for developing countries, but that aspect has yet to be realized.

GENE THERAPY

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

The single gene therapy product currently available in the U.S. for treatment of hemophilia B is listed below in Table 4.

Hemgenix uses an adeno-associated virus, type 5 (AAV5) to deliver the high-activity Padua factor IX gene to cells in the liver. The AAV5 viral genes are replaced with the factor IX gene, so there is no risk of infection. AAV5 targets the liver to introduce the new factor IX gene into liver cells, where factor IX is normally made. The results are somewhat variable. Most patients will obtain factor IX levels in the mild hemophilia range (5 – 50% of normal), but a few will see higher levels in the normal range (50 – 150%). The duration of the effect, the length of time that factor IX production will last, is currently unknown, but an earlier experimental version has persisted for up to five years with no signs of decreased production.

There are a number of limitations to this treatment. The product is not indicated for patients under 18 years of age or for women with hemophilia. Patients with pre-existing antibodies against AAV5 are not excluded, but the treatment may not work for those with extremely high anti-AAV5 levels. There is also a risk of liver inflammation after receiving the infusion, and patients with pre-existing liver damage should only be treated under the supervision of a hepatologist (liver doctor). If inflammation occurs, patients are treated with corticosteroids. Untreated inflammation can result in decreased factor IX production, as well as other health effects.

Ancillary Treatments

The ancillary products currently available in the U.S. for treatment of patients with hemophilia B are listed below in Table 5.

These three products can be used to treat minor bleeds in patients with mild (factor level 5 - 50% of normal) or moderate (1 - 5%) hemophilia. All three are fibrinolytics, which inhibit the breakdown of clots. As soon as the clotting system is activated, the fibrinolytic system, which is part of the healing process, is also activated to start breaking down the clot. In a patient without hemophilia, the clotting process is very rapid compared to the fibrinolytic process, so a good clot is produced that only gradually breaks down over time. In a patient with hemophilia, however, the clotting process is much slower, so inhibiting the fibrinolytic process can make a difference in whether or not a stable clot is formed.

The large number of products available for treatment of hemophilia B increases the chances that every patient can find a product that works well for them. Selecting the best product may be a process of trial and error, but working with an experienced hemophilia treater can shortcut the process. If you think you could be getting better results, don't hesitate to ask your physician.

Table 4 – Gene Therapy Product Currently Available in the U.S.

Brand Name	Generic Name	Manufacturer	Type
Hemgenix	Etranacogene dezaparvovec	CSL Behring	AAV5 vector with Padua FIX gene

Table 5 – Ancillary Products Currently Available in the U.S.

Brand Name	Generic Name	Manufacturer	Type
Amicar	Aminocaproic acid	Akorn	Fibrinolytic
Cyclokapron	Tranexamic acid	Pfizer	Fibrinolytic
Lysteda	Tranexamic acid	Ferring	Fibrinolytic

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.

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 be separated into three categories, 1) improved factor products, 2) rebalancing agents and 3) gene therapy. These updates are divided into those three categories. Within each category, the entries are generally listed in order of the names of the organizations developing the product.

IMPROVED FACTOR PRODUCTS

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

Idelvion May Allow 21-Day Dosing Intervals

5/23 CSL Behring markets Idelvion, an extended half-life recombinant factor IX concentrate. In a report on their extension (longer-term) study of the patients in their original Phase III clinical study, they explored the possibility of switching to a longer dosing interval (the time between infusions). Adults, 18 or older, in the original study were permitted to increase their dosing interval from the initial 7-day interval to a 14-day interval if they were doing well and had had no bleeds in the past six months. In the extension study, those on a 14-day interval were similarly permitted to switch to a 21-day interval after six months if they had no bleeds.

Eleven of the 59 patients in the extension study were able to switch to 21-day dosing at a 100 IU/kg dose. They had a median annualized bleeding rate (ABR) of zero (range 0 – 4.7), and their median factor IX level was greater than 5% up to the 21st day. The study shows that patients who are well-controlled on a once-weekly dose of Idelvion could possibly extend their dosing interval to every two weeks, or in adults, possibly every three weeks. [Pabinger I et al., Res. Pract. Thromb. Haemost., 7(4), 100195, 2023]

New Studies on Alprolix

Sanofi markets Alprolix, a recombinant factor IX product that is fused to the Fc region of an antibody molecule to give it a longer half-life in circulation. Several new studies on Alprolix have recently been published.

5/29/23 A long-term analysis of the data from the three Phase III studies of Alprolix showed reduced pain, increased physical activity and improvements in Quality of Life in Phase III patients who have been followed for 4 - 5 years. The study followed 92 adults and adolescents 12 years or older as well as 30 children younger than 12. [Astermark J et al., Ther. Adv. Hematol., 14:1-13, 2023]

7/18/23 Sanofi performed a biodistribution imaging study of Alprolix to see which tissues take up the protein. The results were compared to those for normal recombinant factor IX and Novo's Rebinyn. As expected, they found that Rebinyn stays mainly in the bloodstream while normal factor IX and Alprolix were found in a number of different tissues, including joint and muscle tissue. The conclusion is that fusion to an Fc fragment does not impact tissue distribution. There was no conclusion whether the difference in biodistribution between Alprolix and Rebinyn affects efficacy in patients. [van der Flier A et al., Blood Coagul. Fibrinolysis, online ahead of print 7/18/23]

7/27/23 Another study looked at differences in efficacy between Alprolix and Idelvion using the data from both products' Phase III studies. They found no significant difference between the products. [Mancuso ME et al., J. Blood Med., 14:427-434, 2023]

8/11/23 The final study looked at the pharmacokinetics (PK) of Alprolix. PK measures the lifetime (half-life) and clearance (removal from the body) of a product. In this study a previously published PK model for Alprolix in adults was extended to cover children under 12 years of age. They found that the previous PK model significantly underpredicted the factor IX levels in children, that is, their levels after an infusion were higher than predicted by the model. The new model gives much better predictions for children receiving Alprolix. [Koopman SF et al., Br. J. Clin. Pharmacol., online ahead of print 8/11/23]

Takeda Halts GO Study of FEIBA for Inhibitors

8/23/23 Takeda markets FEIBA, an activated prothrombin complex concentrate for treatment of hemophilia A and B patients with inhibitors. Some hemophilia B inhibitor patients use FEIBA but many cannot because it contains factor IX to which they have developed allergies. Takeda's GO clinical study was a long-term real-world effectiveness and safety study. The study started in 2014, but after Hemlibra was approved for treatment of hemophilia A inhibitor patients in 2018, many of the study subjects switched to Hemlibra, making it difficult to complete the study. Hemlibra is not effective in hemophilia B. [Hagen T, Managed Healthcare Executive article 8/23/23]
7/24/23 Takeda has published the results of their truncated GO study. [Ettingshausen CE et al., Ther. Adv. Hematol., 14:1-17, 2023]

Intranasal Delivery of Factor IX

8/24/23 A group of French researchers is exploring the possibility of treating hemophilia B patients with intranasal delivery (nose drops or sprays) of factor IX. In studies in mice, both normal recombinant factor IX and Alprolix were taken up after intranasal delivery and delivered to the bloodstream. This "proof-of-concept" study demonstrated that the nasal route might be a possible alternative to i.v. infusion. [Fieux M et al., J. Thromb. Haemost., online ahead of print 8/24/23]

REBALANCING AGENTS

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

Centessa Doses First Patient in Phase IIb Study of SerpinPC

7/10/23 Centessa Pharmaceuticals is developing SerpinPC, an inhibitor of the anticoagulant activated protein C (APC) to control bleeding in patients with hemophilia A and B, with or without inhibitors. SerpinPC is a subcutaneous injection, once every two weeks. They have now dosed the first subject without inhibitors in their Phase IIb study. They plan to start treating patients with inhibitors later this year. [Centessa press release 7/10/23]

Updates on Novo's Concizumab

Novo Nordisk is developing concizumab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) to control bleeding in patients with hemophilia A and B, with or without inhibitors. Concizumab is a once-daily subcutaneous injection.

8/9/23 On 3/10/23 concizumab, brand name Alhemo, was approved in Canada for hemophilia B patients with inhibitors over 12 years of age. Now Canada has also approved the product for treatment of hemophilia A patients with inhibitors. Approval in the U.S. has been delayed after FDA requested additional information from Novo. [Novo Nordisk press release 8/9/23]

8/31/23 The Phase III clinical study data for concizumab for patients with inhibitors has now been published in the New England Journal of Medicine. [Matsushita T et al., N. Engl. J. Med., 389:783-794, 2023]

GENE THERAPY

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

AstraZeneca Buys Pfizer's Early-Stage Gene Therapy Programs

7/28/23 AstraZeneca, a British pharmaceutical company, has been slowly increasing its investments in gene therapy. In the last few years they've acquired Alexion, a rare disease company, and LogicBio, a pioneer in gene therapy. Now they've acquired Pfizer's early-stage (pre-clinical) programs in gene therapy. Pfizer had announced earlier that they wanted to sell off those assets. Pfizer will still continue their current efforts to license their late-stage gene therapies for hemophilia A and B. It is not known whether AstraZeneca will work on hemophilia. [Biopharma Dive article 7/28/23]

Lilly to Acquire Sigilon Therapeutics

6/29/23 Sigilon has been developing a cell therapy treatment for hemophilia. Unlike gene therapy, in which a new gene is introduced into the existing cells in the body, cell therapy introduces completely new cells into the body. The challenge is keeping the immune system from rejecting the new cells.

Sigilon's product consists of genetically-modified cells that express a protein of interest. In the case of hemophilia, the cells would produce clotting factors.

In Lilly's case they would be beta-cells that produce insulin. The cells are encapsulated in a polymer material called Afibromer that prevents the immune system from seeing them as a foreign body. The idea is to place the encapsulated cells in the lesser sac (omental bursa), which is a small cavity between the stomach and liver where they will generate factor.

Although they started with an interest in factor IX for hemophilia B, their first clinical study in 2020 chose factor VIII and hemophilia A as their target. The trial unfortunately was a failure. One of the three subjects developed an inhibitor to factor VIII, and all three saw significant decreases in factor production. After surgery to remove the encapsulated cells, they were found to have fibrosed, that is, the body had covered them with fibrous tissue to wall them off from the rest of the body. The cells inside were dead.

Although Sigilon has been working to solve the problem of fibrosis of the encapsulated cells, it is not clear whether they will still focus on hemophilia. With the acquisition by Lilly, their lead program is now production of insulin by the implanted cells. However, if they are able to solve the problem of cell rejection for insulin, it shouldn't be difficult to substitute cells making other proteins. [Sigilon press release 6/29/23]

Improved Joint Health with Gene Therapy

6/25/23 One of the considerations for whether gene therapy could be called a "cure" for hemophilia is whether it can improve the joint health for patients who already have joint damage. In the past, we have usually considered joint damage to be irreversible, but a recent report from Roche/Spark for their hemophilia A gene therapy, SPK-8011, suggests that idea might be mistaken.

In a small subset of subjects in their Phase I/II study they saw significant improvements in joint health over a three-year period. Interestingly, knees showed the most improvement, trailed by ankles, which showed modest improvement. Elbows showed only modest to no improvement. [ISTH Congress 2023, abstract OC 20.3]

Hydrodynamic, Non-Viral, Gene Delivery

6/23 Although it works, delivery of gene therapy using viruses is sub-optimal, and many researchers are working on better ways to deliver new genes to the body. A collaboration between scientists in Japan and at the University of Georgia is looking at hydrodynamic injection to the liver.

Basically, a catheter was used to blast a high-pressure solution of factor IX genes into the liver. In experiments in mice, dogs, pigs and baboons, they showed that the liver cells take up the new genes. One baboon showed a factor IX level of 53% of normal that persisted for the 210 days of the study. The injections produced no safety concerns except for a slight increase in liver enzymes, which is to be expected. This could be another way to perform gene therapy in the future. [Kamimura K et al., Mol. Ther, Nucleic Acids, 32:903-913, 2023]

Liver Cancer Not Due to Gene Therapy

6/23/23 One subject in the clinical studies for Hemgenix developed hepatocellular carcinoma (liver cancer) after the treatment. A group of scientists from Europe, as well as from CSL and uniQure investigated that development thoroughly and found that there was no evidence that the patient's liver cancer was caused by the gene therapy treatment. Many older patients with hemophilia have been previously exposed to various viruses through plasma-derived factor concentrates, and thus have significant risk factors for hepatitis, which often leads to cirrhosis and cancer. This patient was a 69-year-old male who had had both hepatitis B and C. The scientists found no relationship between his AAV gene therapy treatment and his cancer. [Schmidt M et al., Blood Adv., online ahead of print 6/23/23]

Outcomes–Based Therapy ("No Cure; No Pay") for Gene Therapy

8/17/23 The high cost of gene therapy is a huge issue for patients and payers. Only the wealthiest hemophilia patients would be able to pay $3.5 million for Hemgenix, and even they might be reluctant. Therefore, we are left with only the payers, mainly insurance companies and governments, to pick up the tab. As expected, they are very nervous about laying out these large sums for a product that might not work in all patients and/or might have a limited durability (the length of time the product functions).

Both CSL Behring (Hemgenix for hemophilia B) and BioMarin (Roctavian for hemophilia A) are offering "warranties" for their products. These warranties offer full or partial reimbursement to the payers if patients fail to respond to the therapies. [Managed Healthcare Executive article, interview with Joe Pugliese of Hemophilia Alliance, 8/17/23]

women & girls with hemophilia

articles to support, educate, and empower

Messages From the Eye of the Hurricane

INTERVIEW WITH JENNIFER LYNNE BY RENAE BAKER

They say it's calm in the eye of a hurricane. You might even be able to see the sunshine from within it. However, weather experts strongly advise that one continue to shelter in place and prepare for the worst because, circling that eye is the strongest part of the hurricane, and within seconds those devastating winds may be upon you.

Jennifer Lynn lives in a condo that has been in the eye of two hurricanes! The last one was Ian, the massive destruction from which the region is still recovering. Originally from Brookfield, Wisconsin, Jennifer has lived for many years in beautiful Punta Gorda, Florida. Having graduated with a BA in marketing and journalism from the University of Wisconsin, Madison, she describes herself as a computer nerd. "I make a living being a web developer. I've been self-employed forever," she states. "My clients are all over the world and range from non-profits to law firms."

Jennifer, who has mild hemophilia B and von Willebrand's disease, is also a columnist in health newsletters. Every week she writes about the struggles of women with bleeding disorders. She knew that she was making a difference in the lives of women who had been diagnosed and were being treated as a result of her articles, but she was determined to take it further.

"As a woman affected by hemophilia B and von Willebrand's disease, I realize that increasing awareness about bleeding disorders in undiagnosed women and girls is essential. All women should be aware of the symptoms of a bleeding disorder and be tested if warranted. Bleeding disorders, even mild ones, can cause life-threatening problems at all stages of life."

Jennifer shared that she is curious about how we spread the word about women and bleeding disorders to people outside of the bleeding disorder community. "We do a fine job with people who have been identified and who already know they're a part of the community, but what about people living with iron deficiencies, anemia, and heavy periods who have no clue what's wrong with them? How do we reach those people before they have a life-threatening event?"

To that end, Jennifer has created her own website and is the chief content researcher and author for it:

www.GirlsBleedToo.com. On this beautiful, easy-to-navigate site, you will find links to resources she has found helpful, patient assistance programs, available scholarships for people with bleeding disorders, and the fruits of her thoughtful research via her approachable style of communicating. "I'm trying to make it a one-stop shop for women with bleeding disorders, so they can learn more and find helpful resources. There is nothing else like this out there right now." You may contact her through the website to suggest a topic for her column or feature for her website. She invites you to tell her your story.

Jennifer's observations have led her to believe that the first place to start raising awareness about women with hemophilia is with the medical training our future doctors are receiving. "There are still a lot of doctors who believe that hemophilia just exists in men," Jennifer explains. "When they pick their specialties, the part about hematology is focused on cancers and anemias, not bleeding disorders. There is very little education given towards that. That's not the specialty where they make money. There's more money in oncology, whereas bleeding disorder treatment centers are federally funded. I think a good place to start is with healthcare providers having more knowledge about what to look for and what to do when they think they've identified a bleeding disorder in a person."

Jennifer's bleeding disorders were identified in 1975 when she experienced life-threatening bleeding from a tonsillectomy when she was ten. "I was identified before I had my period, but that's a huge symptom for women at that age. Having menstrual bleeding is an important part of being diagnosed, and if you can't talk about it, it doesn't help." Jennifer would like to see screening of young girls when they begin menstruating and having open discussions about it.

Jennifer believes that we have to do better, as a community, messaging the issues when it comes to women with hemophilia or women who are carriers. "We need to be clearer with our message. There can't be all of this 'You're a carrier, so you're not affected.' We've come a long way since I was a kid, but we're not there yet. We need women who are affected by hemophilia to speak out and step forward so that people understand that it's not something that just affects men. Women deal with different issues than men, and there is a need for more clinical trials that include women."

Clinical trials for hemophilia are essential to advancing medical knowledge and developing treatments. Jennifer looks through the clinical trials and provides links to them under "Resources" on her website. She has noticed a woeful lack of women in these trials. "Most of the clinical trials are limited to men and to people with moderate to severe hemophilia. Consequently, women with the disorder and the mild population are denied the opportunity to participate in trials that could

potentially enhance their quality of life. I think we can do better."

Jennifer realizes that this neglect stems from a lack of awareness and misconceptions about the prevalence and severity of hemophilia in women. "As a result, women have struggled to access treatment, even though they face unique bleeding complications during menstruation and childbirth. Including women with hemophilia in clinical trials will allow researchers to better understand hemophilia's specific impact on women's bodies while providing participants access to cutting-edge therapies in development and aiding them in their own healthcare advocacy. In this way, we can ensure that women's unique needs are met, contributing to a more inclusive and equitable system.

Yet there is hope on the horizon. Quoting one of Jennifer's articles, "Thankfully, the medical community and researchers have made a slow but significant shift in recent years toward recognizing and addressing the needs of women with hemophilia, including specialized care and more tailored treatments, by gathering data on them in trials they have conducted. Including women with hemophilia in clinical trials marks a significant step toward addressing the historical neglect we've faced. It's time to break down the barriers and ensure that no one is left behind in the pursuit of better health for all."

Jennifer wasn't always this outspoken. The stigma visited upon the community because of the bad blood caused her to feel unsafe talking about her bleeding disorder. "I grew up during the tainted factor era. It was horrible. You didn't speak out. You didn't want people to know you had a bleeding disorder, because that was associated with HIV and AIDS. I used to receive notices in the mail that I had received a (compromised) blood product. I was checked every six months for AIDS and

Jennifer with her nephews, George and Bernard

hepatitis. The young people today don't know what it was like."

"The sacrifices of a lot of our people were integral in our having a clean blood supply and safe factor products today. It was definitely a different time in the bleeding disorders community, and we need to not forget."

Jennifer encourages everyone to stay connected to the community. "There are a lot of advancements happening right now within the hemophilia community. If you are a woman with hemophilia B, be sure you are seen at a hemophilia treatment center where the hematologists are very up to date on hemophilia. In my opinion, the treatment that you receive at hemophilia treatment center is better than what you get in a cancer clinic with a hematologist who doesn't specialize in bleeding disorders."

"Sometimes I think, 'How would my life be different if I didn't have this diagnosis," Jennifer muses. "But because I did, I became very active with Camp Heartland, which is a summer camp for children who have AIDS. I became a foster parent to some of the kids, and I learned so much through that! You always have to try to see the positive side, and – for me – there's been a lot of positives."

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.

Takeda

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

Important facts about RIXUBIS®:

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

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

What is RIXUBIS used for?

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

Who should not use RIXUBIS?

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

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

What should I tell my healthcare provider before using RIXUBIS?

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

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

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

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

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

What are the possible side effects of RIXUBIS?

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

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

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

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

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

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

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

Issued 05/2018
S46187 02/19

Takeda

ADVOCACY NEWS

THE WORLD OF WORK

BY ELLEN KACHALSKY, LMSW, ACSW, CCM

If you have been working but are finding it increasingly hard to do your job, especially if your physical condition changes, you may wish to consider a number of tools which may help.

ADA Accommodations under the Americans with Disabilities Act: If you are working but having difficulty due to issues with your joints, pain, and mobility, you may want to consider asking for an accommodation at work. Please think about this if you have a visible disability, i.e. you have trouble walking and use a cane or walker. Asking for help with specific tasks can include having someone bring you supplies or take supplies to someone else. It can include positioning you closer to an elevator and parking closer to an entrance (make sure to have a Handicap Parking placard or license. Your healthcare provider will need to complete a form). It could also mean you have access to a private room in case you need to infuse your factor medication. An ADA accommodation provides some protections when you make that request.

FMLA: If ADA Accommodations are not enough to help with your situation, you may wish to request FMLA (Family & Medical Leave Act) time off. FMLA allows you up to 12 weeks off in a 12-month period, which can be used intermittently or continuously, and also provides some protections for you. You could use this time for periodically returning home for treatment and recuperation after a bleeding episode, or going to your health care provider for exams, tests, procedures, and treatment. You could use this continuously if you were to have surgery or a procedure which requires recuperation. If you need a family member to help you during these times, they can also request an FMLA from their employer so they can have the time off to help you.

You might be wondering, "What happens if I can no longer do my job?" If this might be temporary because you are having surgery and recuperating, then consider:

Short-Term Disability (STD): If or when it becomes extremely difficult to continue doing your job, you will need to find out if you can apply for short-term disability (if you have that as a benefit through your employer, or you purchased your own policy). STD is generally covered up to six months and provides limited income replacement.

Long-Term Disability (LTD): If your condition warrants being off longer than six months, long-term disability would start as long as you have that benefit through an employer or you purchased your own policy. Most LTD insurance companies, which continue to provide income replacement, now require you to apply for Social Security Disability also (see below).

If you've been off work and on short-term disability and you have long-term disability, paperwork will be required for your doctors to complete and justify continuing that leave for medical reasons. Even if you do not have LTD, and your condition is a) expected to last a year or more OR b) prevents you from doing your job, you may apply for SSDI. The Social Security Administration has some other criteria you will need to meet, including being unable to adjust to other work because of your condition. For bleeding disorders, they will review if you've had at least three hospitalizations for your condition or complications from that condition

within the year before applying. They will also look at other complications, including limitation of joint movement.

So, ask for advice from your HTC staff Social Worker or a disability attorney (usually initial consultation is free) before calling 1-800-772-1213 to start an application over the phone so you apply under the correct category. You also must have worked five of the last ten years and paid your FICA and Medicare taxes from your wages. If you have no source of income, then at the same time, tell them you want to apply for SSI (Supplemental Security Income).

In either case, if you are approved for SSI, payments will start from the month you applied and will allow you to have Medicaid through your state until your SSDI starts. Even if you do not have enough credits to get Social Security Disability (SSDI), you can usually get SSI through your state. IMPORTANT: Note the amount you get from SSI is usually an amount set by each state, and when your SSDI starts, that amount may then disqualify you for the state SSI and Medicaid, because SSDI is usually a higher amount.

If you are approved, SSDI payments start after five months of the "onset" of the disability. After you have been on SSDI for 24 months, you will then be eligible for Medicare (what most people get at age 65 upon retiring).

Because insurance coverage, as well as Medicare coverage is so complicated, please get advice from your HTC Social Worker, financial counselor, and other organizations that can help review your needs for coverage.

WHAT THE DATA SHOWS ON COPAY ACCUMULATOR ADJUSTMENT PROGRAMS

BY KOLLET KOULIANOS, MBA, HEMOPHILIA ALLIANCE PAYER CONSULTANT

By now, there is no doubt that our HTC's are not only well versed on what Copay Accumulator Adjustment Programs (CAAP's) are, but likely most, if not all, of you have had patients reach out seeking help, given the most recent data published by Avalere[i] suggests that 83% of commercial enrollees are on a plan that has copay accumulator language. If you consider that the first CAAP was implemented by a self-funded employer in January of 2017, the uptake has been unprecedented.

From the beginning, we have expressed concern for the unintended consequences that could result for the approx. 55% of chronic disease patients on a high deductible health plan (HDHP)[ii] given that enrollees must satisfy his/her entire deductible before the plan contributes the first penny; and the fact that deductibles can be as high as $9,100 individual / $18,200 family in 2023. Manufacturers state that they offer patient copay assistance programs to commercially insured patients to reduce a patient's out-of-pocket cost burden, because frankly they are untenable.

Research conducted by the National Pharmaceutical Council and Xcenda[iii] found that increased patient cost-sharing was associated with worse medication adherence (84% of studies), persistence[iv] (79%) and discontinuation of medicine use (58%). In addition, six of nine (67%) studies found that increased cost-sharing was associated with decreased medication initiation.

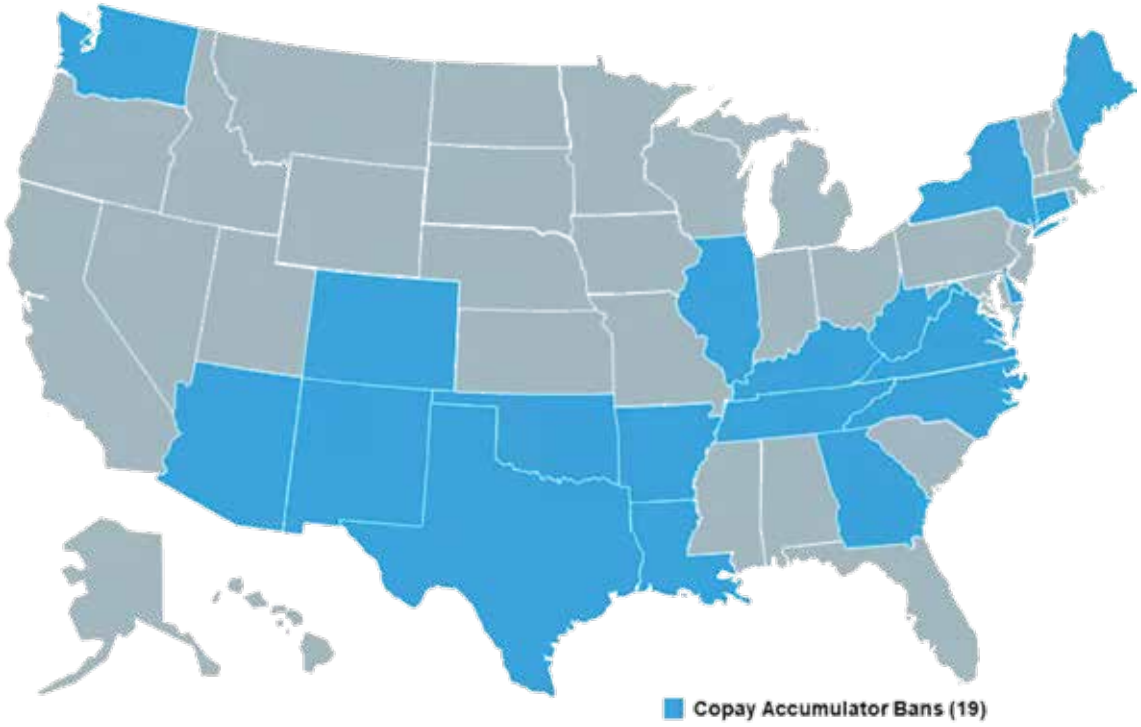
The greater the magnitude of patient cost-sharing, the worse the adherence outcomes regardless of the condition, patient population, or type of cost-sharing. This evidence indicates that patients fail to start and continue needed medicines because of the cost burden.

Some evidence also suggests that higher patient cost-sharing for medications was associated with increased hospitalizations and decreased use of outpatient services. Four of six studies showed a significant increase in inpatient hospital utilization associated with higher cost-sharing.

As of today, 19 states (map below) have banned payer and pharmacy benefit manager (PBM) copay accumulator programs.[v] These laws apply to state-regulated health plans, including the individual, fully insured large group, and small-group markets. It is possible additional states could join the growing number to pass accumulator laws as several states have yet to wrap up their 2023 legislative sessions.

A poster presented at the end of June 2023 at the Academy Health ARM2023 conference, suggests that states that have banned the use of copay accumulators saw a decrease in patient out-of-pocket liability and an increase in treatment adherence and persistence.[vi] For those of you who may still be working to pass legislation in your state, these findings should be beneficial to share with your state legislators looking to reign in PBM games.

CURRENT STATES WITH ACCUMULATOR BANS



Because states do not have authority over ERISA plans, federal work is also being done in parallel to the state efforts. H.R. 830 The HELP Copays Act has been introduced by Representative Buddy Carter (R-GA) and currently has 61 bi-partisan cosponsors; and the Senate companion bill S.1375, led by Senator Roger Marshall, MD (R-KS) has also been introduced and currently has 13 bi-partisan co-sponsors.

This article focuses on copay accumulators, not copay maximizers. We will dive into copay maximizers in the coming months. Any questions can be directed to the payer team at the Hemophilia Alliance and for additional resources focused on both accumulators and maximizers visit www.allcopayscount.org

REFERENCES

i. Copay Accumulator and Maximizers: Evolving Policy Landscape | Avalere Health

ii. National Patient and Caregiver Survey lead by the National Hemophilia Foundation and included the Arthritis Foundation, Kidney Fund, MS Society, Cancer Support and the Aids Institute w results found here: COVID-19 Exacerbates Treatment Affordability Challenges & Health Inequities | National Hemophilia Foundation

iii. High Patient Out-of-Pocket Costs Lead to Worse Medication Adherence Without Overall Health Care Savings | National Pharmaceutical Council (npcnow.org)

iv. Persistence refers to the period of time from initiation of treatment to discontinuation, whereas adherence refers to the extent in which the patient acts in accordance to the prescribed interval and dosing regimin.

v. State Copay Accumulator Bans Will Affect 19% of US Commercial Lives | Avalere Health

vi. https://www.linkedin.com/posts/drjmob_academy-health-poster-activity-7080319268872966145-_9mH/?utm_source=share&utm_medium=member_desktop&lipi=urn%3Ali%3Apage%3Ad_flagship3_messaging_conversation_detail%3Bvcd1c9FYSxythnRaROzFAQ%3D%3D

2023 TEXAS BLEEDING DISORDERS CONFERENCE

Jibin and Farrah had a great time at the Texas Bleeding Disorders Conference in The Woodlands, Texas, June 16–18.

It was great working with the Lone Star Chapter and the Texas Central Bleeding Disorders for such an incredible event! Thank you to everyone that stopped by our booth. It was great to see you!

2023 NATIONAL BLEEDING DISORDERS FOUNDATION CONFERENCE

This year's Bleeding Disorders Conference, held by the National Bleeding Disorders Foundation, took place from August 17-19, 2023 in Washington, DC!

Wayne, Rocky, Erica, Farrah, April, and Fel had an incredible time representing The Coalition for Hemophilia B at the 75th Annual Conference. Thank you to everyone who visited our booth. It was so great to see everyone and to participate in this amazing event!

THE COALITION FOR HEMOPHILIA

2023 MEETINGS ON THE ROAD!

FUN - ENGAGING - INTERACTIVE - NEW SPEAKERS

DATES & LOCATIONS - CAN'T WAIT TO SEE YOU!

- **SEPTEMBER 16:** AUSTIN, TX
- **SEPTEMBER 23:** CLEVELAND, OH; CONCORD, CA; & ALBUQUERQUE, NM
- **SEPTEMBER 30:** INDIANAPOLIS, IN
- **OCTOBER 7:** PHILADELPHIA, PA
- **OCTOBER 14:** NEW ORLEANS, LA
- **OCTOBER 21:** BOSTON, MA
- **NOVEMBER 4:** PHOENIX, AZ; MIAMI, FL; & MILWAUKEE, WI

TOGETHER WITH NEIGHBORING STATES!

Questions? Email Erica at contact@hemob.org or call 212-520-8272

THE MEETINGS ARE FREE, BUT YOU MUST REGISTER NOW!

HEMOB.ORG/UPCOMING-EVENTS/

GAS & TOLLS REIMBURSED | CHILDCARE | KIDS/TEEN TRIP | APPLY FOR A HOTEL NIGHT 3+ HOUR DRIVE

THE HEMOPHILIA B PRODUCT LANDSCAPE

VIRTUAL ZOOM MEETINGS
NOVEMBER 8 & JANUARY 25 FROM 7:00PM– 8:30PM

REGISTER TODAY: HEMOB.ORG/UPCOMING-EVENTS

LET'S GET CASUAL AND HAVE A DISCUSSION ABOUT EMERGING THERAPIES WITH DR. DAVE. THERE'S A LOT GOING ON IN OUR HEMOPHILIA B WORLD AND A WHOLE NEW VOCABULARY!

RAFFLE PRIZES AND MEAL VOUCHERS!

Partnership with Purpose

THE COALITION FOR HEMOPHILIA | CSL Behring Biotherapies for Life™

Survive & Thrive

VIRTUAL MENTAL HEALTH SERIES

TAKE IT TO THE NEXT LEVEL AND THRIVE!

Learn the tools to get you there!
There is a light so bright within all of us, but sometimes it goes dim.

TAKE BACK YOUR POWER AND THRIVE

September 20,
October 18,
November 29, &
December 7

7:00PM-8:30PM
EXPERT LIFE COACHES, RAFFLES & FOOD VOUCHERS!

For more info & to register:
hemob.org/upcoming-events
questions? contact@hemob.org

Partnership with Purpose

THE COALITION FOR HEMOPHILIA | nova nordisk

AGING WITH HEMOPHILIA

50 AND OVER VIRTUAL MEETING SERIES

Three More To Go!

- 26 October
- 05 October
- 09 November

Topics Include:

- Health issues for those over 50, including spouses needs
- Medicare and insurance
- Staying young, staying fit, and healthy exercise options
- Retirement preparation
- Mental health, stress relief, and mindfulness
- Caring for caregivers and relationship advice

Expert speakers, community games, raffles, rap sessions, and social time

7:00-9:00 PM EST

Register Today:
hemob.org/upcoming-events

HOSTED BY The Coalition for Hemophilia

SPONSORED BY sanofi

NEW PARENTS & HEMOPHILIA

PARENTING SUPPORT VIRTUAL MEETING SERIES
OCT 12 | NOV 16

YOU'RE INVITED TO SET TIME ASIDE FOR YOU!

As a parent of a young child, newborn to age 4, we invite you to grab a cup of tea or coffee and join new parents, connect with experienced parents who have been there, and build your strong village of support.

Includes rap session, hot topics, raffles, food voucher, and a really cool game!

Register Today: hemob.org/upcoming-events

Partnership with Purpose

The Coalition for Hemophilia

sanofi

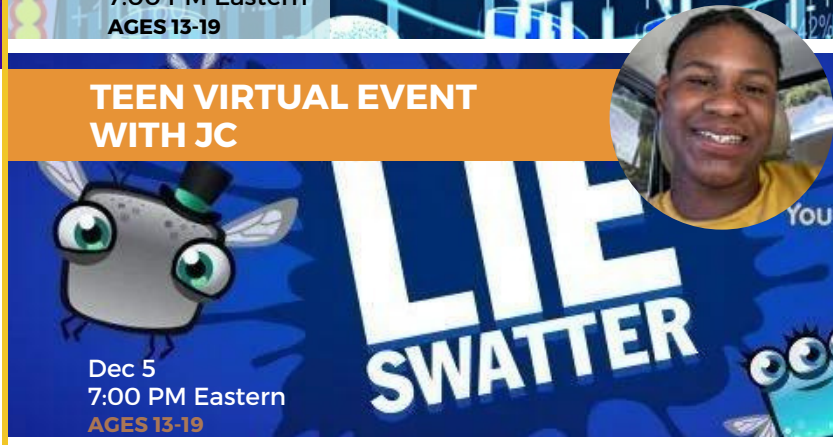
Oct 10 & Nov 14 , 7 pm Eastern

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

RAFFLES AND FOOD VOUCHERS!

REGISTER TODAY:
hemob.org/upcoming-events

The Coalition for Hemophilia



RAFFLES AND FOOD VOUCHERS!

REGISTER TODAY: HEMOB.ORG/UPCOMING-EVENTS

Partnership with Purpose

The Coalition for Hemophilia | MEDEXUS PHARMA

You're Invited

GINGERBREAD HOUSE DECORATING + TRIVIA NIGHT
CONTEST OPTIONAL

Saturday, December 16, 4pm Eastern

FIRST 50 TO REGISTER BY NOVEMBER 4
WILL RECEIVE A GINGERBREAD KIT

REGISTER TODAY: HEMOB.ORG/UPCOMING-EVENTS

Submit your gingerbread house photos to contact@hemob.org
Prizes for Trivia + Winning Gingerbread House
questions? contact@hemob.org

Partnership with Purpose

MEDEXUS PHARMA | The Coalition for Hemophilia | PARAGON HEMOPHILIA

VIRTUAL WOMEN'S EDUCATION & EMPOWERMENT RETREAT

EDUCATION | EMPOWERMENT | CONNECTIONS

Register Today: hemob.org/upcoming-events

DECEMBER 1-3

Partnership with Purpose

The Coalition for Hemophilia | sanofi

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 22, 2023**
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 22, 2023**. Your name and information will be kept strictly confidential.

Mail this form to the address below or scan the QR code to apply.

The Coalition for Hemophilia B Holiday Cheer
757 Third Ave, 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, Zip code: _____

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:**
_____	_____	_____
_____	_____	_____
_____	_____	_____
_____	_____	_____


THE COALITION FOR
HEMOPHILIA B
ANNUAL SYMPOSIUM
DALLAS, TX: APRIL 25-28, 2024
APPLICATIONS FOR TRAVEL AND
HOTEL GRANTS NOW OPEN:
HEMOB.ORG

BCares

THE COALITION FOR HEMOPHILIA B PATIENT ASSISTANCE PROGRAM

"ONE OF THE MOST IMPORTANT THINGS YOU CAN DO ON THE EARTH IS TO LET PEOPLE KNOW THEY ARE NOT ALONE."

SHANNON L. ALDER

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

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

LEARN MORE hemob.org/bcares

PLEASE DONATE hemob.org/donate

THE COALITION FOR HEMOPHILIA
Email: contact@hemob.org
Phone: 212-520-8272

THE COALITION FOR HEMOPHILIA

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/

SCAN ME

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

LANDON: A TEEN OF MANY TALENTS!

BY SHELLY FISHER

Landon took time from packing for his trip to the Coalition for Hemophilia B's The Beats Music Program in Nashville, TN, to talk with me about a few of the things he loves: the electric guitar, cooking, and car shows.

It was clear just a few minutes into our visit that he knew his way around the six strings of his Charvel guitar, the kitchen and a racetrack! While this eighth grader had only been playing the electric guitar for a few months, he was already laying down licks from songs like "Enter Sandman," by Metallica, and "Smells Like Teen Spirit," by Nirvana. He's even written a few songs and recalled a specific composition he wrote for his presentation in science class to promote awareness about styrofoam's effect on the environment. With a laugh and ready smile, Landon shared that he had tried the trumpet, but it just wasn't his thing. He confided that his musical talent also extended to singing in the choir, where he joined the altos last year at school. It was no surprise at all when I found out that he would be playing at The Beats Music Program in Nashville, and both he and his mom were eagerly looking forward to what the next few days would bring.

What does an 8th grade electric guitarist like to cook? When most teens are making grilled cheeses and the occasional scrambled egg, Landon's not kidding around in the kitchen. "Filet mignon medium rare and pasta," were Landon's answers with no hesitation when asked what his house specialties were this past summer. It's no wonder he had aspirations to be a chef someday, and I could easily see him working in a restaurant by day and rocking the house at night with his band.

When I talked with Landon, he was making the most of his summer but also looking forward to the new school year. He was enjoying hanging out with friends and swimming and had just started playing on a summer soccer team for the first time, but the thing he seemed most excited about was driving his go-kart around the neighborhood. He told me, "It's really fast!" When asked if he was able to drift in his go-kart, his response was immediate, "Definitely!" He then added with a grin, "I destroyed the tires on it." Like most teens nearing the end of summer, Landon was also excited to meet his new teachers and start new classes, with a specific interest in consumer science.

As our conversation turned to his diagnosis of hemophilia B, Landon's mom joined us to help with

the sequence of events. After all, Landon was only six days old when he continued to bleed after a routine procedure. Though his mom recalled a distant uncle who had died after receiving contaminated blood, and a cousin who was known as a "bleeder," no one in her family discussed either diagnosis openly. At seven pounds, Landon received four blood transfusions and two rounds of FFP. Because of their experience, Landon's mom is passionate about communication and transparency among families with histories of hemophilia B. "Embrace it and do what you have to. No one wants to have it, but you have to have conversations about it with your family."

It was Landon's love of car shows and a slightly delayed "prophy" schedule that led him to one of his more serious episodes. He had been at his aunt's house wearing crocs and it was one day past his prophylaxis therapy when he walked on a hilly golf course. What might have resulted in just a blister for some caused a bleed and a great deal of pain for Landon the next morning. Once at the hospital, his mom was shocked by the lack of knowledge she encountered, but a quick call to their hematologist got Landon the factor IX that he needed. He was unable to walk for two days and used a walker for a week afterwards.

When I asked him what advice he might have for someone who was recently diagnosed, the rockstar-chef-car enthusiast smiled broadly and said, "Make sure you're safe, make good choices, but still have fun."

Rock on Landon!

CYBERBULLYING AND SOCIAL MEDIA: A TROUBLING TREND

BY MATTHEW D. BARKDULL, MS, MBA, LMFT, MEDFT

With the Internet at our fingertips, finding people without headphones in their ears and devices in their hands is becoming ever more difficult.

As our dependence on technology continues to increase, and the virtual world becomes more rooted into our culture, there is greater opportunity for people to bully and abuse while remaining anonymous. There may be no greater time than the present to become more guarded from those who use the Internet to be unkind. Anytime people become targets of deliberate and repeated emotional harm by means of electronic devices, they are experiencing what social scientists call "cyberbullying." Where face-to-face intimidation and aggression once ruled, bullies are finding social media to be an effective tool to accomplish the same thing.

Teenagers are especially at risk. A 2018 Pew Research Center study found 97% of teens between the ages of 13-17 use a social media platform such as YouTube, Facebook, Instagram, Snapchat, etc. Forty-five percent of these teens are online regularly. A 2023 research study now shows that 85% of teens are not only frequently online but are extensively using social media.

Of concern, we are beginning to learn that the rates of cyberbullying attacks increased significantly during and even after COVID.

Unfortunately, teen cyberbullying is not a fleeting trend. Recent statistics suggest that this behavior is now at jaw-dropping levels. Consider the following 2023 statistics:

- More teens are cyberbullied in the United States than any other country in the world.
- About 60% of US teens say they have experienced some form of cyberbullying.
- About 70% of US teens report that someone has spread rumors about them online.
- When school students were asked to indicate which social networking sites they had experienced cyberbullying, 42% said Instagram, 37% said Facebook, 31% on Snapchat, 12% on WhatsApp, 10% on YouTube, and 9% on Twitter.
- US school teachers report that cyberbullying is their #1 safety concern in the classroom.
- Girls are more likely than boys to be both perpetrators and victims of cyberbullying.
- Around 50% of LGBTQ+ youth experience harassment online, which is over two times higher than the average rate.
- Interestingly, only 7% of US parents are concerned about cyberbullying at school although 65% of parents know that their teens could be experiencing cyberbullying over social media.

REPERCUSSIONS OF CYBERBULLYING

As outlined above, cyberbullying is NOT a victimless behavior and not a harmless prank. In fact, recent documentaries, studies, news articles, and other media have been highlighting examples of ruined lives caused by cyberbullying. The following trends are especially troubling:

- When teens experience cyberbullying, they are at greater risk of suicidal thoughts and self-harm. In fact, teens are two times more likely to attempt suicide if cyberbullying is actively taking place.
- About 64% of cyberbullying victims say it has affected their ability to feel safe at school and interferes with learning, resulting in poor self-esteem.
- Cyberbullying impacts sleeping habits and depression.
- Teens are beginning to avoid school altogether because of cyberbullying.
- Practical Tips for Protecting our Youth
- Given the prevalence of cyberbullying, social scientists have studied how we can better protect our youth from becoming a target or, if they are a target, how to stop cyberbullying and get the help they need.
- Make the subject of safe online activity a common subject of discussion. Despite research showing that teens are more likely NOT to divulge troubling problems, being persistent in building a culture of trust and support tends to reverse this trend of silence. Trust and support are critical as having teens immediately inform a friend, teacher, advocate, parent, family members, etc. of cyberbullying is one of the most effective ways to prevent and/or stop the bullying.
- Reinforce to teens that they should NEVER post personal information online.
- Restrict teens' access to their online profile to trusted friends and family only.
- Teach the importance of NEVER opening messages from unknown or suspicious accounts.
- Practice securing passwords and enabling two-factor authentication on all social media accounts.
- Encourage teens to log out of their online accounts when they leave their device or computer.
- Teach that teens should never argue with a cyberbully. Ignoring and reporting bullying behavior to a trusted adult(s) is always the best option.
- Promote pausing before posting a message or a photograph. Reinforce the question, "Do I really want to post this? Is it worth it?"
- Reinforce to youth that if their friends are suggesting cyberbullying, promote and emphasize abstaining. It is also helpful for teens to suggest to peers that not only is it hurtful, but cyberbullying can lead to criminal prosecution. Please note that in a growing number of states, legal action can be taken against cyberbullies.
- Encourage teens to advocate for those they learn are being cyberbullied. Promote being brave and standing up for victims.

For more information on how teens, caregivers, treatment teams, and others can protect against cyberbullying, I encourage you to visit the following websites:

https://www.stopbullying.gov/

https://www.schoolsafety.gov/bullying-and-cyberbullying

https://staysafeonline.org/online-safety-privacy-basics/cyberbullying/

https://www.internetmatters.org/issues/cyberbullying/protect-your-child/

https://kidshealth.org/en/teens/cyberbullying.html

Flying High With Chase

BY SHELLY FISHER

Chase is flying high, but don't tell him the sky's the limit! His advice? "Don't let anything hold you back from what you want to do."

With his high school graduation behind him, Chase took time from his busy work schedule as a mechanic at a local airport and an impending tee time to visit with me. Five minutes into our visit, it became clear the planes he described were more than just the focus of his work as a mechanic in the summer. Chase shared that two to three times a week, he actually jumps in the cockpit and takes flight during flying lessons. As a student halfway to getting his pilot license, he confided that he hopes to work full time next year as a crop duster. It seemed to me at first that the "crop-dusting" sky was an adequate limit for this graduating senior, but he soon proved me wrong! Chase announced that he plans to eventually land a career as a pilot on a commercial airline. "It makes you feel free and one hour we're here and two hours later we're in Kentucky, or Tennessee."

With a background in competitive travel baseball since he was 10, it was no surprise that this soon-to-be pilot told me he likes to see different places and has particularly enjoyed participating in the Coalition's annual symposium each year. He also enjoys taking his "pavement princess," a F-250 diesel truck with a 15-inch lift and 800 horses under the hood, to truck shows. What's his dream destination? "I'd like to land a plane on Mackinac Island in Michigan."

Chase counted playing freshman baseball as one of the best and most memorable times of his high school career. He contributed to his team's success as a first and third baseman. When asked if he had any advice for the incoming 9th graders, he offered, "Stay on top of your work. Looking back, I could have made it easier on myself by just doing my work when it was assigned."

The youngest to have hemophilia B in his family, Chase was diagnosed at birth, but it didn't slow him down. To offset the odds of injury during baseball season, he opted to take prophy treatments three times a week. After an injury during weightlifting, he had to have surgery, and later dislocated a knee as well, but he described these events as nothing major. He attributes the minimal effect of these injuries to a "healthy regime of prophy." When prompted for advice for those who are early in their diagnosis, he encouraged them to "not let it keep you from doing what you want." He added, "I never let it hold me back. If I could do it, I was gonna."

When pressed for something his family and friends might say

about him, he said, “I think they would say I am a caring person.” He expressed gratitude to his mom and dad for seeing him through having hemophilia B and his uncle Shad for keeping him in check. “He was always on me a little bit because I didn’t always watch what I did.”

Chase commented that he spends his free time with family and friends, and most weekends he can be found wakeboarding, skiing, and tubing on the lake. He also told me he has fond memories of traveling for baseball with his mother, who was also a team mom, and enjoys restoring an old pontoon with his dad. Golfing, an early favorite before it interfered with his baseball swing, has become his latest athletic pursuit and fills in the gaps for some much-needed down time.

When I encouraged him to tell me his favorite quote or motto, Chase barely paused before supplying some lyrics from one of artist Paul Brandt’s western songs:

“Don’t tell me the sky’s the limit when there are footprints on the moon.”

The song’s title, “There’s a World Out There,” and the first chorus do an amazing job of describing one of the most inspiring seniors I have ever met. Here’s to you, Chase, and the class of 2023!

“There’s a world out there and I wanna be in it.
I got a life and I’m gonna live it.
Don’t tell me the sky’s the limit,
There’s footprints on the moon.
I wanna do my walkin’ down the road less traveled,
Sew my dreams where they won’t unravel.
If you play it safe you won’t get nowhere,
I can’t stay in here when there’s a world out there.”

B inspired!

Stories and artwork from teens in the Hemophilia B Community

SUMMER 2023

IN THIS ISSUE:

FLYING HIGH WITH CHASE

LANDON:
A TEEN OF MANY TALENTS

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!