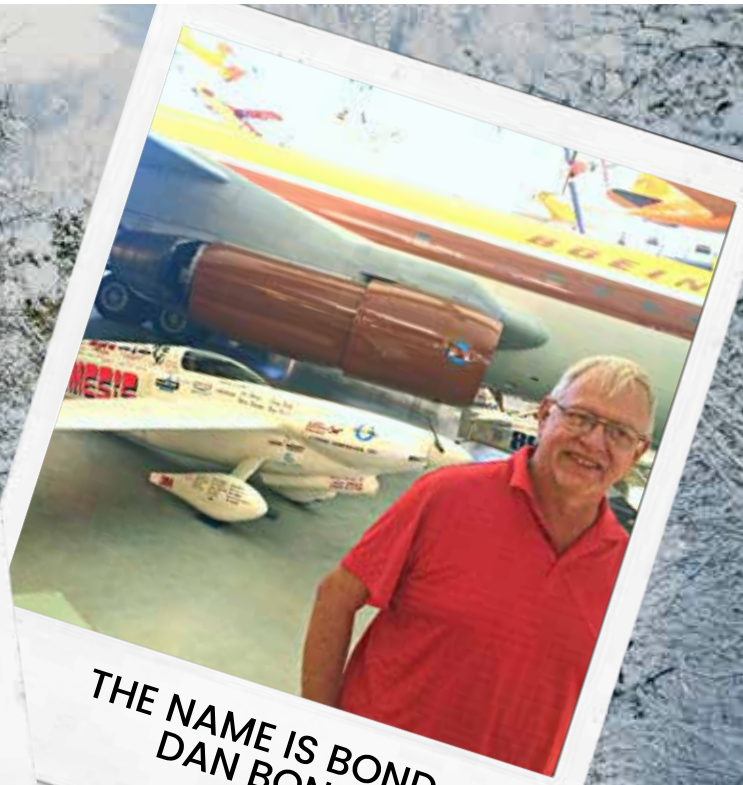
THE COALITION FOR HEMOPHILIA B

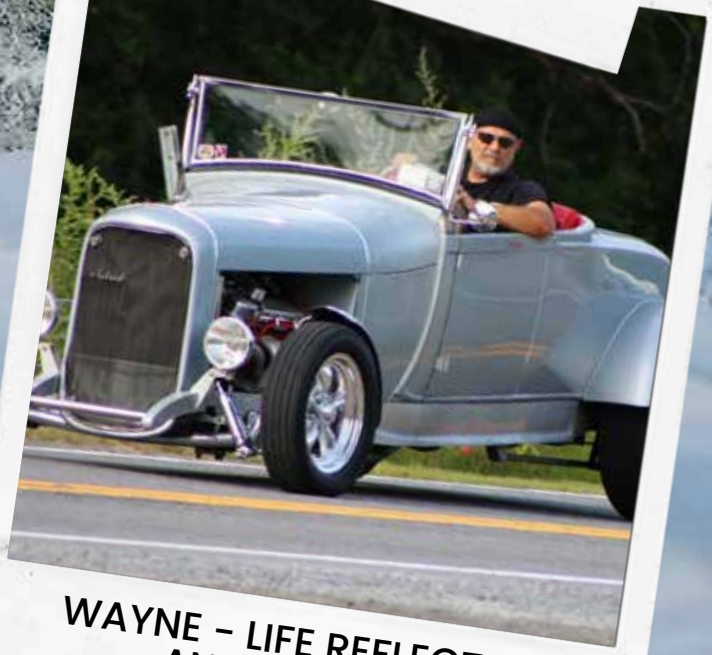
HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION WINTER 2022

ULTRAMARATHON MAN – RICARDO REACHING NEW MILESTONES



THE NAME IS BOND– DAN BOND



WAYNE – LIFE REFLECTIONS AND RESILIENCY

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MISSION

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

WAYNE COOK "BLEEDERSHIP" AT ITS BEST

BY RENAE BAKER

"People still can't believe I actually did this, but I called my entire hemophilia treatment staff, my orthopedic surgeons, and my heart doctors into one room to discuss how to manage my knee replacements."

He needs no introduction, but that's Wayne Cook, President of The Coalition for Hemophilia B. "I've always been a huge advocate when it comes to my health and making sure everybody is on the same page. My advocacy extends to community members as well.

Wayne, whose hemophilia B diagnosis is severe, has a life-long habit of looking upon his pain and challenges as "grist for the mill." The community members of The Coalition for Hemophilia B are the lucky recipients of the resulting "cakes."

A born leader, his life experience casts long shadows over the Coalition programming. Wayne has been president of the Coalition since 2006 but had been involved since 1992. "I was president of my local hemophilia chapter in Albany," he explains. "The Coalition had just started up and I was the only factor IX patient in the Albany area. My HTC nurse at the time said, 'Hey, I heard that this new organization called The Coalition for Hemophilia B is starting up and it's all about factor IX.'" Wayne connected with chief operating officer Kim Phelan, spoke at the Coalition meeting in New York City and, "have been involved ever since." Kim Phelan recalls, "The organization had just been founded. Wayne was the first person to volunteer his help and he gave us all the time he possibly could. We could not have done it without him! We became good friends - a friendship I have valued for over 30 years. He is someone I truly admire, he is such good person, a true inspiration. Every time Wayne was hit with a challenge such as hepatitis C, knee replacements, etc., he fought with a determination and served as a role model for so many in our close-knit community."

Wayne's impact is evident. Looking back on his early years with the Coalition, he remembers the heavy lifting of simply getting the organization up and running. "The development of our newsletter was greatly expanded. It was small, but it started reaching a lot of people. We



began getting our name out into the community by having a booth and introducing ourselves and holding small meetings at national events hosted by NHF and HFA."

He leans forward, his energy rising and his focus narrowing, "We started doing legislative work at Washington Days." Wayne points to the crucial work the Coalition did to get The Ricky Ray Hemophilia Relief Act of 1998 passed through the legislature. The Relief Act provided compensation and compassionate pay to people who had contracted deadly illnesses via bad blood products during the AIDS crisis. "It was a huge win," Wayne says with restless relief. "It should have been a bigger settlement, but at least it held the government accountable and made our blood supply safe."

Wayne pauses before he says, "If it wasn't for the hemophilia community, our blood supply would never have been made safe. It took the forces of the bleeding disorders community to stand up."

He recommends the film, "Bad Blood," which exposes how clotting factor and other blood products were being manufactured in the early 1980s. "The pharmaceutical manufacturers would obtain plasma from the local blood banks that were offering money to drug users and the homeless for their blood donations. They were getting plasma from prisoners."

Wayne's understanding is that one of the largest sources of donated plasma was Angola Prison in Louisiana, which had a 95% HIV population at the time. Wayne continues, "So, these pharmaceutical companies were making factor concentrates knowing the plasma was contaminated with HIV. They knew there was something wrong with it and were still selling it. Parents were trusting the companies and administering the factor products to their children. It was like Russian Roulette - bleed to death or take a chance on contracting HIV/AIDS."

An anguished exhale caves in his countenance for a moment, "I've seen so many things over the many years I've been involved in the community. I've seen parents get divorced because of insurance issues. I've seen parents lose their children to the virus. I've seen husbands lose their spouses after unwittingly exposing them to HIV. Knowing they inadvertently caused their death caused extreme emotional grief."

Wayne began to realize through The Coalition for Hemophilia B he had a chance to make a difference in the lives of community members. The first few years were baby steps; however, 2006 was a pivotal year. The Coalition founder and president, John Taylor, was stepping down to venture into other areas of the hemophilia community. The Coalition Board decided it would be best to have the face of the Coalition be someone with hemophilia B. They asked Wayne if he would take the reins. He agreed. The team in those early days consisted of himself, Kim Phelan, Dr. Dave Clark, and dedicated volunteers who came to help.

"What's nice about our organization," he fights to keep his composure, "is that many of the programs we've hosted are rooted in the struggles I've experienced in my life."

Although Wayne was fortunate to have not been infected with HIV, he did contract hepatitis C. Having suffered the loss of his best friend to hep-C via AIDS, Wayne took treating his own case very seriously. The first doctor who treated him for his liver disease seemed ineffectual, so when a friend raved about another doctor and offered to connect them, Wayne, who was getting very sick, jumped in with both feet. Dr. Keller was a Florida hematologist and immunologist who had a reputation for working outside of the box.

"A lot of doctors weren't on board with Dr. Keller's way of thinking; however, but he had a good track record with patients who were surviving!" He was Dr. Keller's only patient who had Hep-C, but not HIV. It was Dr. Keller who instructed him to start taking factor three times a week. "I was doing prophylaxis before other doctors were even starting to think about it. Wayne relates that Dr. Keller's philosophy was to get you well first and then treat the hepatitis. To do that, he developed a line of vitamins and supplements - and they worked. It took a year, but he found himself

healthy and feeling good. That's when the targeted cirrhosis treatment began. Wayne saw Dr. Keller for three years and is thankful to him for saving his life.

"The treatment was like chemotherapy. Following treatment, I would get very sick. Just as I started to feel better, it would be time for another treatment, and I'd be sick again. This treatment broke down my immune system. My doctors started thinking I had contracted HIV because of all the infections I was getting. There were times that due to bad infections, I almost lost my right leg." He came out the other side with his health and limbs intact, but his perilous experience prompted him to work with Kim Phelan on programs to teach the community how to be healthy while dealing with the treatments for hep-C.

Another program was conceived when he made a gargantuan discovery during his knee replacements. "I had both knees replaced; the first in 2006 and the second in 2007. We did a cost analysis of the price to have my right knee done compared to my left.

For the first knee, we went with the traditional bolus infusion the day of surgery, and then every 12 hours infused to 100% for about 8 weeks. After that time, the dose was cut to 50% every 12 hours, and then it was cut down again. After4 months, I was back on my regular regimen of factor." The final tally came to a whopping 2.5 million dollars just for factor replacement.

Floored by that number, Wayne researched a process called continuous infusion before getting his left knee replaced. Explaining the difference, he shared that instead of intermittent large doses of factor for 16 weeks, he received continuous dosing of factor via a pump for 22 days. After that, he returned to his normal prophy dosing. Factor replacement for his second knee was just $750,000. A huge cost savings!

Wayne wanted everyone to know about this money-saving and quality-of-life-improvement information! Through The Coalition, he began telling his story and discussing his results with bleeding disorder organizations, pharmaceutical companies, doctors, and researchers. He became such a champion for the cause that organizations that had nothing to do with hemophilia invited him to speak to their groups about advocacy.

When Wayne talked to the pharmaceutical companies, he affected substantial changes in the way things were being done; changes that have enriched the hemophilia B community in measurable and immeasurable ways.

He looks back at the inception and trajectory of a key advisory group on which he sits to this day. Genetics Institute was the first company to market a recombinant factor product and, in 1995, formed an advisory group consisting of 4 community members. When Wyeth Pharmaceuticals acquired Genetics Institute, the advisory board remained intact, meeting annually.

At these meetings, in Wayne's opinion - slow progress was frustrating, "We'd present something to them and it seemed they wanted to run with it. We'd ask what the status was and they would reply, 'We're not going to address that now. We want to talk about another topic.'"

Not content to sit on his hands any longer, he decided to lay it on the line with the pharmaceutical giant. In 2006, just after he'd become president, he met with executives at Wyeth and said, "Look – you're the only company that has a recombinant product on the market for hemophilia B. You have an advisory board that you don't utilize. I'm suggesting if you want to be the leader in this hemophilia

community, you need an active hemophilia patient advisory board that will work as a board of directors for you."

Wyeth listened. Board members were selected, guidelines and bylaws were drafted, and the working board of directors became official. Wayne has served on the board since 1996 and has been Chair since 2006. He is proud of the Board and considers it a great organization. He not only helped create it, but he pounced on a critical opportunity to save it when it was at risk of being eliminated.

"When Pfizer acquired Wyeth," Wayne began, "there was talk of eliminating the Board. We were at an event in New York City, and I happened to be introduced to Pfizer's VP of Global Marketing. I told him straight out - if you get rid of this advisory board, you're not going to be in touch with the patient needs. They agreed and that's why the Board is still around. There are a few new people, but some have served on the Board since 2006."

When Wayne went through periods of dire depression, he told Kim Phelan he wanted to get the word out about the beast that often plagues his fellow bleeders. The result of those conversations is The Beats, a music program, which had its maiden voyage in 2019, and is an annual favorite for Coalition members who find creating music a great tonic for their depression. "We can host this program and teach people how to be healthy," Wayne states. Cognizant of the fact that the Coalition wasn't meeting the needs of people who don't find sports outings appealing, he is gratified to see people who weren't participating in other Coalition events, joining in to create music together.

Coalition member and Beats participant, Zach, credits Wayne's inspiration, "The Beats program is a fantastic experience that should be a keystone program for years to come. Allowing bleeders to display their musical abilities and their general love for music is beneficial to the health and wellness of this community."

Wayne, a talented drummer in his own right, would like to see The Beats program broaden. "I'd like to see this expand to include acting, drama, and other artistry. It's going to take time for that. We're still in our beginning stages here, but there was a time when we were in the beginning stages for our Symposium too." Wayne remembers the first Symposium had about 40 to 50 attendees. Fifteen years later, the attendance is 700 to 800!

If Wayne's name is mentioned to any Coalition member, you will likely hear the terms father figure, blood brother, and friend. Members call him kind, compassionate, caring, and a welcoming brother. They credit him for being a great leader and for his ability to get people to open up and connect. Perhaps one of the ways he does this is by being so open about his challenges. He makes no secret of his difficult childhood, which he describes as "ugly." People admire him for his strength and perseverance through it all and see him as an inspiration during their own "ugly" times.

Coalition member, Milinda, has gratefully benefited from his honest sharing. "Life wasn't easy," she begins. "Wayne fell many times. Some people were awful to him, but he made it through because of his strength and perseverance. This man saved me by giving me this community he grew for all of us so we can learn, educate ourselves, advocate, understand different therapies, and most of all, giving us to each other. It changed my life."

Zach relates, "In getting to know Wayne, I'm humbled by the hurdles he has overcome in life and the amount of dedication he has put into a lifetime of work within the hemophilia community." Coalition Patient Liaison Farrah Muratovc credits Wayne with making all Coalition members feel welcomed and comfortable. She says people view him as a person everyone can talk to when they see him. Indeed, Coalition Community Relations Director, Rocky Williams says, "For me, spending time with Wayne is a highlight of any event." Coalition member, Christopher, raves, "He's like a big brother everyone one wants to connect with."

Wayne's eyes well up as he hears these touching tributes. As he considers how he has come to have such a place in the hearts of the Coalition, he offers, "I've always been very honest and upfront. I don't pull punches or mince words with anyone. I tell it how it is. I'm not afraid to open up about my life because I don't want to see anyone go through what I did. If I'm as honest as I can be, it might help them understand that if I can go through these struggles and successfully deal with them, then they can too."

After enduring an unhappy childhood, Wayne has achieved a life in which family is everything to him and brings him the ultimate happiness. Losing his first wife of 29 years, Maureen, Wayne remarried 11 years ago to Kelly. He practically glows as he states emphatically, "I cherish every moment with Kelly, my kids, and grandkids. Kelly and I have a special bond. We not only love and respect each other, but we like each other, and that's key!"

Wayne recently shared the news with the community that he is facing some serious health challenges. When he and his wife found out that his cirrhosis had returned, he cried. Thoughts of the end were suddenly very real to him. "But," Wayne promises, "I'm not ready to check out yet. I just turned 60. I've got too many things I want to do."

At the top of his list are to see his grandkids grow up and to spend as many quality years with his wife as possible. Wayne is looking at this current challenge as just another phase he must go through. "I've always been strong. I've always fought through everything I've ever struggled with. You have to take time to feel the pain and pity of it, but then you have to stop and say, 'OK, this is what I have to do. Stand up, put your big-boy pants on and do it.'"

In his letter to the community, he reassured with, "I don't want anyone to feel sorry for me. Everyone who knows me knows I have never backed down from a fight when it comes to my health. So, I'm going to win this fight."

Will there be an educational program springing from his current set of challenges? "I think we do need to cover more topics for older adults," he begins. "We have a lot of programs for parents and younger adults, but there are issues with our aging population that need to be discussed." Because older people with hemophilia B are pioneers, Wayne offers, "I would like to offer a presentation to give them an 'A-ha' moment where they say to themselves, 'Geez! I never thought I was going to be able to retire. I never thought I'd need to see a doctor about heart disease or being able to take an aspirin every day.'" He wants to prepare this contingent of the Coalition for the challenges that – it could be said – are blessings to achieve. "I'd like to do something like that and then add speakers to give the older generation a steppingstone. We had some speakers who have addressed retirement and such, but we need to explore more."

In the same manner he gathered his medical team in a room to discuss his knee replacements, Wayne has already gotten his doctors on the same page for his liver treatment. He illustrates, "I had a doctor who had never worked on a hemophilia patient before, a cardiologist who needed to take care of my heart during the surgery, and my hematologist. They and the nursing staff are all going to work together to take care of me. We're going to work as a team because that's the way it needs to be. I want everybody communicating."

Looking back through the years, Wayne says with an air of serenity about him, "I don't look at my hemophilia as a disability. As I've gotten older, I've looked at it more as an opportunity. If I hadn't had hemophilia, I wouldn't have been able to travel and I wouldn't have made such fabulous friends!" As Wayne shares his intentions to spend more time on himself and with his family during this time, he is continuing his mission to help the hemophilia B community by being open and honest in sharing his own experiences.

The Coalition extends its best wishes and tremendous gratitude for the many ways in which Wayne Cook has enriched our lives through his life of service to this community. Thank you, Wayne, for your indomitable "Bleedership" of The Coalition for Hemophilia B. Your tremendous contributions have surely lengthened countless lives and immeasurably improved the quality of more lives than you could know.

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GO THE DISTANCE

Get lasting high trough levels for continuous bleed protection

ONLY IDELVION DELIVERS

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

+

0 SPONTANEOUS **BLEEDS**†

+

7- AND 14-DAY DOSING FDA APPROVED‡

* The average dose for people receiving prophylaxis every 7 days was 37 IU/kg.

† The median AsBR for people who started and stayed on 7- or 14-day prophylaxis was 0. For people who switched to prophylaxis from on-demand, the median AsBR was 0.7. AsBR=annualized spontaneous bleed rate.

‡ Once well controlled (1 month without spontaneous bleeding or requiring dose adjustments on a weekly dose of ≤40 IU/kg), people 12 years and older can be transitioned to 14-day dosing.

§ Hemophilia FIX Market Assessment. Third-Party Market Research.

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IMPORTANT SAFETY INFORMATION

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

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

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

Please see additional Important Safety Information and brief summary of prescribing information on adjacent page and full prescribing information including patient product information at IDELVION.com.

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

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

CSL Behring

CSL Behring

IMPORTANT SAFETY INFORMATION (cont'd)

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

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

In clinical trials for IDELVION, headache and dizziness were the only side effects occurring in more than 1% of patients (1.8%), but are not the only side effects possible. Tell your healthcare provider about any side effect that bothers you or does not go away, or if bleeding is not controlled with IDELVION.

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

Initial U.S. Approval: 2016

BRIEF SUMMARY OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use IDELVION safely and effectively. Please see full prescribing information for IDELVION, which has a section with information directed specifically to patients.

What is IDELVION?

IDELVION is an injectable medicine used to replace clotting Factor IX that is absent or insufficient in people with hemophilia B. Hemophilia B, also called congenital Factor IX deficiency or Christmas disease, is an inherited bleeding disorder that prevents blood from clotting normally.

IDELVION is used to control and prevent bleeding episodes. Your healthcare provider may give you IDELVION when you have surgery. IDELVION can reduce the number of bleeding episodes when used regularly (prophylaxis).

Who should not use IDELVION?

You should not use IDELVION if you have had life-threatening hypersensitivity reactions to IDELVION, or are allergic to:

- hamster proteins
- any ingredient of IDELVION

Tell your healthcare provider if you have had an allergic reaction to any Factor IX product prior to using IDELVION.

What should I tell my healthcare provider before using IDELVION?

Discuss the following with your healthcare provider:

- Your general health, including any medical condition you have or have had, including pregnancy, and any medical problems you may be having
- Any medicines you are taking, both prescription and non-prescription, and including any vitamins, supplements, or herbal remedies
- Allergies you might have, including allergies to hamster proteins
- Known inhibitors to Factor IX that you've experienced or been told you have (because IDELVION might not work for you)

What must I know about administering IDELVION?

- IDELVION is administered intravenously, directly into the bloodstream.
- IDELVION can be self-administered or administered by a caregiver with training and approval from your healthcare provider or hemophilia treatment center. **(For directions on reconstituting and administering IDELVION, see the Instructions for Use in the FDA-Approved Patient Labeling section of the full prescribing information.)**
- Your healthcare provider will tell you how much IDELVION to use based on your weight, the severity of your hemophilia B, your age, and other factors. Call your healthcare provider right away if your bleeding does not stop after taking IDELVION.
- Blood tests may be needed after you start IDELVION to ensure that your blood level of Factor IX is high enough to properly clot your blood.

What are the possible side effects of IDELVION?

Allergic reactions can occur with IDELVION. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the chest or throat, difficulty breathing, light-headedness, dizziness, nausea, or decrease in blood pressure.

Your body can make antibodies, called inhibitors, against Factor IX, which could stop IDELVION from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

IDELVION might increase the risk of abnormal blood clots forming in your body, especially if you have risk factors for such clots. Call your healthcare provider if you experience chest pain, difficulty breathing, or leg tenderness or swelling while being treated with IDELVION.

The most common side effects of IDELVION are headache and dizziness. These are not the only side effects possible. Tell your healthcare provider about any side effect that bothers you or does not go away.

Based on July 2021 revision

Please see full prescribing information, including FDA-approved patient labeling.

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 Departmentat 1-866-915-6958.

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

THE NAME IS BOND, *DAN BOND*.

BY RENAE BAKER

What's in a name? One might hazard a guess that the intriguing, thrill-seeking life Dan Bond has been living was psychologically summoned by the lexical potency packed into the surname he has in common with 007. But Dan's determination to overcome the challenges of his childhood deserves more of the credit.

Dan has an appreciation for well-placed words. His email sign-off quotes never fail to provoke thought, like "Unless life also gives you water and sugar, that lemonade is going to suck" - R. K. Lilley. He could easily write an article about himself, but his humility might get in the way of some details. He acknowledges that he has been living an exciting life. Among other elations, he has been to dinner with the US Air Force Thunderbirds, lunch with racecar driver Al Under Jr.'s crew chief and flown upside down over a 1,000-foot-high mountain ridge.

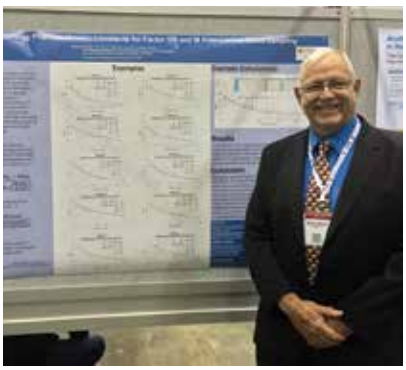
"It's not about me being smart or exceptional. It's more about my being lucky and persistent," he says. "The truth is, I have spent a lot of time understanding my disease and complications. I have good relationships with all my doctors, and I understand the importance of staying on top of my medications. There may be limits to what we can do, but not to what we can accomplish."

How easy it would be for Dan to withhold from readers the astonishing adventures of his life. Dan's maternal grandmother lost five infant sons to bleeding. This gave his parents the foresight to have a relative in the delivery room, ready to give Dan a direct transfusion on the day he was born, in 1953. No transfusion was needed that day. When Ted Bond, a "rough neck" oil field worker, learned that his infant son had severe hemophilia B, he moved his family 1,500 miles, from La Mesa to Galveston, in order to be close to a major medical center. Then he went to medical school and became a research physiologist-hematologist. This was an exciting time, in the world of hematology. "Researchers discovered plasma could stop, or at least control bleeding." Dan relates, "I was in uncharted waters. Nobody knew my life expectancy, but everyone doubted it would be long, because major bleeds were still life-threatening."

When fresh, frozen plasma (FFP) came along in the 1970s, it was found to be even more effective at bleed control. "Using FFP for prophylaxis gave me a decent approximation of a normal life and another step up the life expectancy ladder." But, just as his hopes for a longer life were setting in, the same FFP concentrates which brought such hopes and expanded freedoms, became a nemesis to Dan and thousands of others dependent on blood products in the 1970s and 80s. Through contaminated FFP, Dan contracted HIV and hepatitis.

The "blood scandal" is one of the reasons Dan is one of only a few hundred men over the age of 60 living with hemophilia B. He has also had his share of bleeds, arthritis, joint replacements, and re-replacements. One of Dan's favorite email sign-off quotes is, "Our scars are permanent reminders of our victories over things that tried to kill us." If ever there was an example of victory over difficulty, it is Dan. He took his life from grounded in hospital beds to – literally – soaring!

By the time he was 12, Dan had spent 1/3 of his life in the hospital. "I calculated that I had spent more time in the hospital than any of the doctors treating me," he shared. Spending so much time away from his home,



inside institutional walls, without the benefit of hospital staff that took more than a clinical interest in him, had a penetrating impact on Dan. "Growing up in the hospital made me unusual; 'raised by wolves,' I guess. It had a huge effect on my personality and social skills." Dan found it difficult to make friends. Looking back, Dan remembers, "My most serious childhood bleed was when a classmate threw a brick and hit me in the back. I spent two months in the hospital. First day back in school, I got another bleed when my knuckles hit his nose. My knuckles are fine. His nose is still bent."

On the positive side, the lack of control he felt over his bleeding disorder, and needing to roll with the flow, has created a flexible, easy-going side to his personality. "People who insist on planning sort of mystify me," he muses. Another side of his personality is a competitive nature. "I really enjoy that," he says with a deceptively quiet smile.

Perhaps that competitive streak was born of his determination to overcome his physical limitations. To that end, it seems Dan's father has had a profound influence on his life. A paternal perk of being on the leading edge of blood clotting and coagulation research was that Ted was able to involve Dan in clinical pretrials, including one which studied the effect of Rattlesnake venom on blood. Outside of the laboratory, Ted helped Dan set his sights high. Literally.

When Dan was 14, Ted bought an airplane. Convincing the FAA medical examiner that a person with hemophilia could fly took over a year, but Dan persevered and soloed (piloted a plane by himself) at 15 years old, before he even had a license to drive a car. Dan reflects, "Like most teenagers obsessed with something, I collected every magazine about flying that I could find. About that time, Daryl Greenamyer modified a WWII fighter and named it the Conquest I. The headline in the magazine read: 'WORLD SPEED RECORD RETURNED TO THE US.' I knew right then that's what I wanted to do. I collected magazine pictures, clipped quotes, and made a collage of his exploits that's still in my office today."

Dan took his fascination and declared it his focus in college, graduating with a degree in aerodynamic engineering from the University of Texas at Austin. His concentration was in engine cooling and drag reduction.

After college, he dabbled in the world of racecars, and looked for work with airplanes. "Quite coincidentally, the top three airplane racers were in Texas, all within about three hours of where I lived. So, I called the top guy and offered my services. He declined. The second guy never called me back, but the third guy said, 'Sure! Come on down!' So, I went to work for him."

His name was Jon Sharp. Sharp was attempting to build a racing plane that would use cutting-edge aerospace engineering to (in Sharp's words,) "kick everybody's butt" in the field of air racing. Sharp credits having a strong team working with him on the creation of the Sharp DR 90 Nemesis. The Nemesis is still considered the most successful air racing plane in history. It won 47 out of 50 races entered, 9 consecutive Reno Gold National Championships, 16-plus World Speed Records, it was the 6 IF1 Points Champion from 1994-1999, and was retired to the Smithsonian Air & Space Museum in 2000.

Dan is credited with being the member of the team to convince Sharp to use an airfoil with extensive laminar flow, which was key to the success of the plane. Dan didn't get to fly the Nemesis, he notes with a hint of regret, but he has flown plenty of others. If you're wondering how the two air racers who blew Dan off fared, Dan tells me, with that competitive glint in his eye, "They never won another race."

Asked about his most memorable experiences, he mentions aerobatics. Yes, that is just what it sounds like: "feats of spectacular flying performed in aircraft to entertain an audience on the ground."

"It's exhilarating; pulling enough Gs to pass out is an experience everyone should have at least once," he says. "When you pull back on the stick real hard, the centrifugal force pushes you down in the seat, and you essentially weigh six times more than you do. Then all the blood rushes out of your head and you pass out."

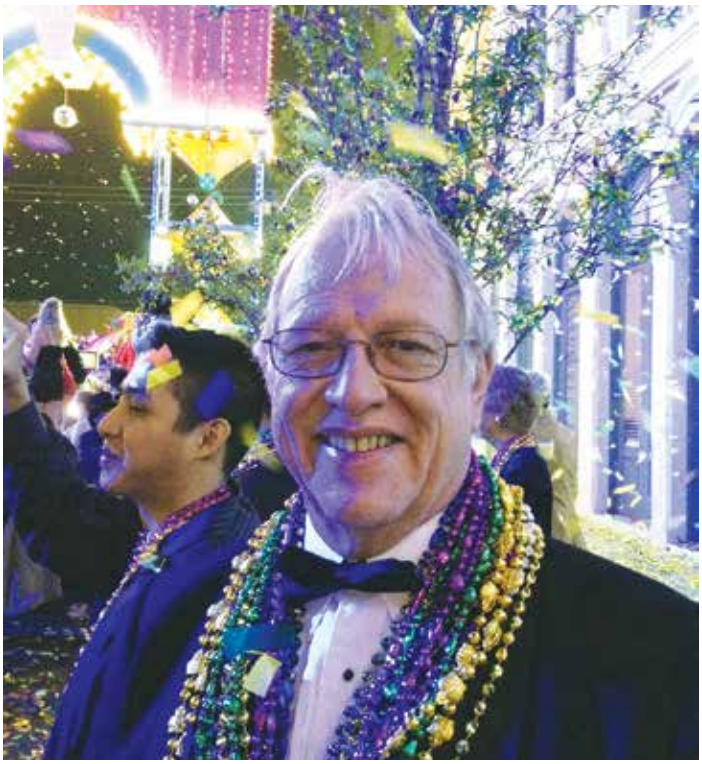
Dan tells one of his "near death experience" stories. "I was crewing for a cross country race plane. I got to fly as co-pilot in the support plane, a Riley Rocket; a hot rod version of the Cessna 310 made famous by Sky King of my childhood. Somewhere along the way to Kitty Hawk, the racer radioed that he had a fuel leak, and that we had to land immediately. Unfortunately, we were on top of 20,000 feet of solid cloud cover."

After the mayday call, while trying to ascertain which airport at which they were cleared to land, they fought severe turbulence. "The G meter later showed +6 and -3 Gs. Every negative G sent the charts through the roof, along with cookies, open sodas, and tools. The positive Gs turned these things into missiles. While all this was going on, the racer was holding formation off our left wingtip. Since he was depending on us for navigation, he had no choice but to follow us down. The turbulence would bounce the racer so severely that it would roll almost 90 degrees in each direction. When we finally got down, we found a defective sensor. I told them that they could stop trying to scare me. Mission accomplished!" Next time you see Dan at a Coalition event, be sure to ask him about the other three near death experiences!

It has been many years since Dan has flown airplanes. These days, he gets his thrills by traveling, snorkeling, scuba diving, and watching Broadway shows with his childhood sweetheart. For the past 15 or so years, he has also enjoyed attending The Coalition for Hemophilia B events. In short order, he realized that the community welcomes and values him. He appreciates the fun, familial aspect of the Coalition and has been an active member ever since his first meeting.

Looking back on his many adventures, Dan offers, "While I'll be the first to admit that hemophilia helped form who I am today, none of these things happened because of it. Hemophilia shapes our lives, but it should not dominate or define them." The biggest hurdle this adventurer has faced might surprise you. Then again, it might not: "Insurance. I've had to change insurance carriers three times, because I hit the lifetime cap." He ruminates on the community of people with blood disorders. "We are here today because of a vast network of people who have done things they didn't have to do: strangers donating blood have kept our community alive for generations, doctors getting up in the middle of the night to treat us have saved us countless hours of pain, researchers developing things that just a few years ago were the stuff of science fiction, grandmothers falling asleep rubbing our sore knees, society banding together to pay for our treatment – none of these people had to do these things. They do these things, because we need them done, and it leaves us with a lasting debt that must be repaid."

Dan continues with a call to action: "What can you do? Get involved. Be a camp counselor. Volunteer with your organization or chapter. Join a clinical trial. Talk to your legislators. Let your history benefit the next generation."



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ULTRAMARATHON MAN

AN INTERVIEW OF RICARDO AND ANNETTE RAMIREZ BY RENAE BAKER WITH JANINE ACHURY

"I want to show the hemophilia community that with the right treatment, you can do whatever you want. You can run 100 miles, ride a bike or whatever. That's why I want to do this."

If someone were to make a feature film about Ricardo Ramirez, (and they should!) it would span the genres of action, adventure, drama, and romance. Knowing that I would be interviewing Ricardo, whose first language is Spanish, we arranged to have Annette and Janine Achury with us on the Zoom to help translate. I was impressed to see that Ricardo didn't need much help expressing himself in English, but it was interesting to get Annette's point of view of her experience loving a person who is living an "extreme" life while managing hemophilia B.

Ricardo is a 50-year-old, strong, determined, self-effacing champion of his cause; encouraging others with blood, sweat and an impish grin. He is a version of a Mexican American Harrison Ford.

We met with Ricardo and Annette on a Sunday. Sunday is Ricardo's "long run" day. "I ran 24 miles this morning," Ricardo tells us. "Yeah, last week Sunday, I ran 20 miles and the next day, 17."

Ricardo works repairing autos in a body shop, and Annette works for the City of Los Angeles Department of Animal Services. They both have grown children. They are both runners. That's how they met. They tell us that they've been together for two years. When I ask how much of that time they've been married, they start laughing and reveal that they have been married for most of the time they've known each other.

"We got married very quickly," Annette admits. "We have a lot in common," starts Ricardo. "We have the same birthday and even had the same last name already!" Annette picks up the story:

"It was just strange meeting each other. We gravitated toward one other and in a very short time, became best friends. We were running together five days a week and spending all our spare time with each other. It became clear that we just worked!"

"We just didn't want to spend time apart from each other," Annette explains. "Our lives just totally merged."

"And then COVID-19 happened," Ricardo drops.

"Yes, it's totally a COVID marriage," Annette picks up. "The world shut down the month after we moved in together."

As many did during this time, Ricardo was laid off from his job and Annette worked from home. Suddenly they were together 24 hours a day! The wedding was made easier by the fact that Annette's daughter had become an ordained minister and stood ready to officiate at a small ceremony in the Olympic Valley at Lake Tahoe.

The Coalition For Hemophilia B leadership became aware of Ricardo who is an Ixinity Ambassador with Medexus. Last summer he spoke about his experience completing the Rio Del Lago Ultramarathon, which is billed as a "100 mile endurance marathon."

What is an ultra marathon? The standard definition of an ultramarathon is anything past the marathon, or 26.2 miles. However, the shortest standard distance considered an ultra is 31.07 miles. According to a study conducted by Run Repeat director, Paul Ronto, and mathematical analyst, Vania Nikolova, participation in ultra running events is on the rise. There has been a 1676% increase in participation since 1996. But how many of them have Hemophilia B? Only one that we know of - Ricardo Ramirez is an ultramarathon champion.

Sparing having him toot his own horn, Annette touts a few of his accomplishments. "Because he's an ultramarathon runner, his miles start at 32-mile races, and go up from there. He has actually run a 200-mile race and won. The big race that he's training for now is a 135-mile race across the Death Valley Basin."

Ricardo grew up in Fresnillo, Zacatecas in Mexico. His

family were not aware that he and his younger brother have hemophilia B. As a schoolboy, he played soccer, basketball, volleyball and baseball. He had nose bleeds but took them in stride. The first noteworthy incident he remembers is hurting his arm and having it swell so remarkably that he was taken to a doctor. The doctor decided Ricardo's muscles had torn and set out to perform surgery. When he made the incision, he found the muscles were fine, but there was a lot of internal bleeding. Assuring Ricardo and his parents that nothing was wrong, the doctor sutured the arm, leaving an impressive scar which serves as a reminder that there are still doctors in the world who are who don't know how to diagnose or treat patients with hemophilia B.

"In my birthplace," he recalls, "We didn't have a hematologist. It was unfortunate because I went to the hospital almost every month due I had a stomach ulcer. The doctor presumed he had hemophilia A but had no idea how to treat with plasma, so nothing would stop the bleeding." Around the age of twenty, Ricardo says, "It got to be too much for my body." He was hospitalized and lost so much blood that he fell into a coma. He doesn't remember for how long he was out, but when he awoke, he was blind in his left eye. "Little by little, the sight came back," he says with relief.

Ricardo now lives in Los Angeles where he has been properly diagnosed with severe hemophilia B and is on a prophylactic treatment regimen. His younger brother is still in Mexico and continues to struggle to get adequate treatment and sufficient clotting factor. Ricardo is plugged into the Spanish speaking hemophilia communities and does what he can to help his brother, but it is clear to him that there is a lot of work to do to educate doctors about hemophilia B.

Ricardo has four children and five grandchildren. His one-year-old grandson has been diagnosed with severe hemophilia B. Ricardo's daughter, is very concerned that her son won't be able to live a normal life. Ricardo tries to reassure her by pointing out that he is a living example of how full and active life can be with the bleeding disorder.

Ricardo puts his money, time, effort and sweat equity where his mouth is by running ultramarathon races. How does he do it? He didn't jump right in. He didn't start running until the age of 36. "I was overweight," he remembers. "I started walking in the park and ran into a friend who invited me to a running group, so I started running."

He ran and won so many marathons, he can't even tell us the number. About ten years ago, after five years of running marathons, a friend invited Ricardo to join him in running an ultramarathon. "So, I went for my first 50k, which is around 31 miles. I finished in first place."

The ultramarathon to which he has aspired to for years is the Badwater race across Death Valley. Badwater is considered the world's toughest footrace. Only 100 participants are selected to compete, and people come from all over the world to test their physical and mental endurance capacity in the 135-mile race. Ricardo was accepted for this race. I ask what is behind his aspiration to tackle Badwater.

I need something to complete my running career, so I choose Badwater. After that, I want to go back to marathons."

"This is your Mount Everest," I grasp.

"This is my Mount Everest. My goal is to complete the ultramarathon in 35 hours."

Regard treatment, Ricardo explains he will infuse 1-3 hours before the race and again 20-24 hours into the race.

"Do you get to take breaks?" Janine asks.

"Not really," he explains. "Just to eat and such, but no more than 20 minutes. No naps," Ricardo states definitively. "If I fall asleep, I won't get up."

When asked if this worried her, a burst of laughter and then, almost comically Annette answered in a low, steady voice, "Yes. Yes, it does. We met because of running. Ricardo helped me reach some of my goals." She explains that she is new to ultrarunning.

"After the very first 50-mile race I ran, we rushed Ricardo to the emergency room. He had a condition called rhabdomyolysis, where your muscles start to break down, due to overexertion and your liver tries to filter out the muscle tissue, causing liver damage. That was a real scary incident. We had taken him to urgent care because he thought he was urinating blood. The urgent care doctors said they didn't know how to treat someone with hemophilia. They advised him to go right to the emergency room. Annette rushed him to the hospital where, again, they were told, "We've never

treated anyone with hemophilia, so we don't know what steps to take."

"Luckily," she continues, "Ricardo's hematologist was very accessible, and we were able to immediately get ahold of him. He told the treating doctors what tests to run and how to treat him, staying in constant communication while Ricardo was hospitalized. After lots of IV fluids and determining there were no internal bleeds, Ricardo was released. It took him all of two to three days to recover."

"And then I was back to running!" Ricardo laughs.

"He did not complete the next fifty-mile race he competed in," Annette shared. Since then," Annette picks up, catching her breath from laughing at the insanity of it all, "I've seen him finish a 100-mile race, but it still worries me to see him run a 135-miles across Death Valley in the middle of the summer where the temperature is between 110 and 130 degrees." The laughter stops. "Yeah, it's a scary thought. I'm gonna be there the whole time. I'm going to be his crew chief. I'll be there to take care of him, but he's gotta do the work."

That means that while Ricardo is running, Annette will travel in a vehicle and will be at checkpoints where they can give him fuel and water and check to see how he's doing and send him on his way.

I ask Ricardo if he can go into the vehicle to cool off. He tells me that he can but must resume running in the same spot where he stopped.

I asked Ricardo if his grandson's diagnosis has intensified his passion to reach the hemophilia B community through his ultramarathons.

"Yes!" he says, emphatically. "Especially with this marathon. I want to show the hemophilia community that, with the right treatment, you can do whatever you want. You can run 100 miles, ride a bike or whatever. That's why I want to do this!

Many of his family and friends don't understand Ricardo's passion to run. Even his doctor, who is a runner, doesn't understand how or why Ricardo is running these ultramarathons, but he clears him and gives him his okay for each race.

"Have you ever become discouraged and thought you might stop running," I ask.

"Yup," he offers, without hesitation. "Often, I have said, 'I don't want to run anymore,' then the next day, I sign up for another race!"

"There have been races he hasn't been able to start and races he hasn't been able to finish," Annette illustrates. She explains he has sometimes gotten injuries at his job which resulted in large hematomas. "I've seen pictures of him where his entire leg is swollen purple, and he still went to race, thinking he was going to finish. He didn't of course!"

"I try my best," Ricardo says with a shrug. "If I finish, okay. If I don't, I don't."

The Rio Del Lago race he completed last summer was his third attempt.

"Thinking back to those times when you nearly threw in the towel and quit, what made you change your mind and sign up for the next race," I ask.

"Each time I run any race, I push myself. I put a lot of effort into my races. I'm tired when I finish the races."

When you feel that fatigue and maybe even sickness at the end of a race, where is the exhilaration?" I probe. "Do you feel it the next day?"

"Sometimes, the next day, I sign up for another one," he is poking fun at himself now, "and sometimes I say, "I am too old for this!"

But I really do want to know, because I have never understood the joy of pushing one's body that hard.

Annette offers her insight, "Yeah, the following day when you have that sense of extreme accomplishment. I haven't done a race of that length, but in the one's I've done, you have so many ups and downs - emotionally, physically, mentally - and then to push yourself through it give you a sense of accomplishment.

It's not just what your body can achieve, because you trained for that, but it's pushing your mind to go past the pain, past your comfort levels to do something most people wouldn't try or even think to do, and you're out there doing it!

This is what he is passionately sharing with the hemophilia B community. "If you get proper treatment, you can do anything. You can live a normal life."

"A normal life?" Annette cries out, "But you don't live a normal life! You live an extreme life!"

"An extreme life, then," he laughs triumphantly!

And as we close our conversation we see Fernando with his sparkling, brown eyes, gleaming at the satisfying purpose his ultramarathon mission is achieving.

Life expectancy for those with bleeding disorders has increased with the development of new and improved treatments. The impact of living longer with a bleeding disorder may mean modifications and adjustments to lifestyle. This can impact people at any age. Humans are social creatures–we get support from family and friends, community, work colleagues, and spiritual and social groups. The ultimate goal is maintaining your best quality of life which requires recognizing barriers to being able to do what you want to do.

GETTING HELP TO LIVE YOUR BEST LIFE:

PHONE A FRIEND, ASK THE AUDIENCE, OR CONTACT YOUR HTC

BY SHERRY HERMAN-HILKER, PT AND ELLEN KACHALSKY, LMSW, ACSW, CCM

Solving any problem starts with figuring out what the problem is. Once you know what you need, it is much easier to find resources to help you.

- Maybe you are struggling with being socially isolated from others
- Maybe you have physical limitations
- Maybe you need to plan for a surgical procedure
- Maybe you don't have computer/device knowledge or access and feel that you are "disconnected"
- Maybe you are confused by the medical language that is used about your health

We all face many challenges through our lives, and we can't always manage them alone.

We need to learn to identify our limits and then challenge ourselves to get help to move beyond them. This may be uncomfortable, but you are resilient, you can adapt, and it's never too late to learn something new. It could be something little – for example:

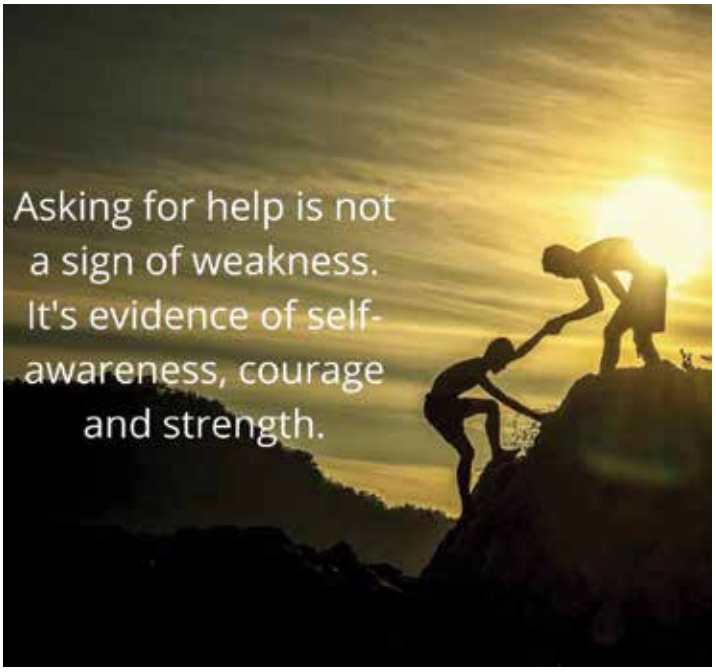
- Asking your grandchild to help you get connected to a video appointment
- Reaching out to your treatment center and asking them to explain your insurance
- Asking your physical therapist what to do if you can't put on your own socks
- Or it could be something bigger – you need help because:
- You are no longer feeling safe in your own home
- You don't have friends or family that can help you after a surgery
- You don't feel comfortable telling your providers what your "real" struggle is
- You don't feel comfortable asking for accommodations from your employer
- You may not be comfortable sharing what is going on in your home or your life
- You may be concerned about stigma or being judged.

Are you too afraid or embarrassed to ask for help with any of these things? Living your best life may require you to strep out of your comfort zone and ask someone to help you or advocate for you if you don't feel you can do it yourself.

Ask yourself, "What are my barriers to asking for help?" Do you know who to ask? Do you feel comfortable asking? This is the second most important step in actually solving the problem.

If you feel uncomfortable asking for help, can you figure out why? Do you or did you:

- Feel anxious?
- Get denied help in the past?
- Feel less independent?
- Not have money to pay for help?



Your treatment center team has expertise and is happy to help you, but those who have lived with hemophilia and the associated challenges are also excellent resources. If you take the opportunity to benefit from both the expertise and the experience you will likely be able to make some positive changes .

Who else can you ask for help? Depending upon the problem, you may find help in a community group such as:

- A church or synagogue or temple or mosque
- Local non-profits
- Your primary care physician
- Your treatment center team
- Your local library
- Local city services
- The Area Agency on Aging
- Local clubs or social organizations (knitting groups, social clubs, rotary & lions clubs, etc.)
- Local chapters and foundations of bleeding disorder organizations
- National organizations including HFA, NHF, Coalition for Hemophilia B, etc.

This is not a comprehensive list by any means, but may give you an idea of where to start. If you need help identifying resources, your HTC social worker and other team members may be able to help you. Don't hesitate to ask!

In the following section, you will find some questions that have been asked about challenges related to aging with a bleeding disorder. The response to each question is in a problem-solving format. These questions are just examples with strategies for working through the process of finding solutions.

PHYSICAL BODY & HEALTH ISSUES

I am having a hard time getting around at work.

What is your work environment like? Consider specifically what is hard for you. Make a list of the problems you are having. Are they due to the environment? Are they due to the demands of the job or due to your health/physical body? Think about all the details of what you are experiencing. This will make it easier to focus on possible solutions.

With the list made, ask yourself who can be helpful in this case. Your boss or supervisor or co-workers? Your HTC staff, social worker, nurse, physical therapist, physician? Who to ask may depend on what your barriers are.

I need my teeth pulled. I am in constant pain. My dentist says he won't do it. How do I get help?

Call your treatment center. They can guide you through this process, but it is important that you give them enough time in advance of any procedure. They will offer points to consider. Your hematologists, your HTC hygienist, your nurses, and your social worker all have a part in this.

- What is your dentist recommending, when and why?
- Why won't your dentist do it? Is the dentist referring you to an oral surgeon or other specialist?
- Will you need factor infusions or other medications before and after a procedure?
- Do you have dental insurance? If so, what portion of it does it cover?
- Do you have funds to cover the balance? Do you need funding help?
- How are you managing your pain?

I am having trouble concentrating at work and it is hard for me to complete my job. Now what?

Consider more specifically what you mean. Do you have any other symptoms? This may or may not be related to your bleeding disorder. This may be a good time to reach out to your primary care doctor to ask questions about your unique situation. A medical work up may be needed to understand what is happening. Your hematologist and HTC may also have a role. It is always good to reach out to the HTC since they generally know you well.

Think about things that are going on in your life and try making a list of what might be impacting your concentration. Consider your work environment. Would it be helpful to talk with your supervisor? Many things can impact concentration- physical, emotional or situational. Who you need help from depends on the reason for your difficulty.

I am feeling emotionally drained, angry, and sad. Where do I go for help?

You may benefit from seeing a behavioral therapist, such as a social worker, psychologist, or psychiatrist who can assess you and may recommend medication or other non-medical techniques to help improve your mood, sleep, and appetite. This is also a good time to alert your primary care provider who can take a look at your overall health and your HTC staff who can help you consider your emotions in the context of your bleeding disorder.

Do NOT be self-conscious about seeking help for cognitive and emotional issues, just as you should NOT be embarrassed to seek medical help! Your physical health can affect your mental health, and your mental health can affect your physical health. Remember, your

body and mind are connected, and we are learning more about these connections every day.

Who amongst your family and friends can you ask for help? Consider family, friends, churches, community organizations, the treatment center, the chapters, foundations, and other connections you have and trust. Getting to the root of the "real" problem, not the presenting problem, is necessary. Are you being honest with yourself about what kind of help you need?

Sometimes your care team including the treatment center staff may be giving you advice or information that you don't want and aren't ready to hear. IF this happens, it is important to communicate your feelings and ask questions so that you can be helped in the way you need.

When you reach out for help, take time to listen and process the help and suggestions offered, even if they are new or uncomfortable ideas for you. It may take a few conversations to dig down and get comfortable with the process before you can find a satisfactory solution.

I am having trouble walking long distances. I think I need a handicapped parking permit. What should I do?

Consider specifically what is causing you to have trouble walking long distances. Is this a short term or long-term situation? If this problem is new, be sure to reach out to your doctors in case treatment for the concern is possible.

These permits come from the Secretary of State or Department of Motor Vehicles. Your health care providers - Primary care, HTC team, or other specialist - can complete this paperwork, but they need to understand the reason for your physical difficulty with walking. This could involve an evaluation by a physical therapist to determine what is physically limiting you and whether it can be improved with intervention.

I am getting hit with huge hospital bills and I cannot afford to pay them.

You have certain rights that are not disclosed to you. Do not be INTIMIDATED by bill collection agencies because once you seek advice you will learn how to deal with them and empower yourself with the knowledge to handle these situations. There was a patient who was so INTIMIDATED, he gave his life savings to pay a bill he did not have to pay at all if he HAD only asked for help.

You should verify the charges which should match up with the explanation of benefits from your health insurance plan. Make sure that if you have Medicare and a secondary insurance plan, both insurances have been billed. Also call the hospital billing department to see if they will reduce any charges, help arrange a payment plan, or if they have any financial assistance programs that are available.

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.

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

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

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

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

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

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

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

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

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

Indications and Usage

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

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

Important Safety Information

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

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

Who should not use Rebinyn®?

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

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

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

How should I use Rebinyn®?

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

What are the possible side effects of Rebinyn®?

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

Please see Brief Summary of Prescribing Information on the following page.
Rebinyn® is a prescription medication.
You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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Brief Summary Information about:
REBINYN® Coagulation Factor IX (Recombinant), GlycoPEGylated

Rx Only

This information is not comprehensive.

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

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

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

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

Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing REBINYN® so that your treatment will work best for you.

What is REBINYN®?

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

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

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

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You should not use REBINYN® if you

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

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

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

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

You should tell your healthcare provider if you

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

How should I use REBINYN®?

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

REBINYN® is given as an infusion into the vein.

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

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

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

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

Use in children

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

If you forget to use REBINYN®

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

If you stop using REBINYN®

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

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

What if I take too much REBINYN®?

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

What are the possible side effects of REBINYN®?

Common Side Effects Include:

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

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

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

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

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

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

Revised: 08/2022

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

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

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

More detailed information is available upon request.

Available by prescription only.

For information about REBINYN® contact:
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800 Scudders Mill Road
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US22REB00153 August 2022

novo nordisk®

EMERGING THERAPIES

BY DR. DAVID CLARK

Several of the studies reported below were presented at the recent annual meetings of the American Society of Hematology (ASH) December 10 – 14, 2022 in New Orleans, and the European Association for Haemophilia and Allied disorders (EAHAD), February 7 – 10, 2023 in Manchester, England. Copies of the abstracts (summaries) can be read or downloaded for free from their respective websites.

You might wonder why the pharmaceutical companies present so many studies at the large scientific meetings. One reason is to get their name and product out in front of the participants, who are mainly doctors and researchers. But another is because they want to know as much as possible about their products. One of the important maxims in the pharmaceutical industry is that a company should know more about their products than anyone else. They look at their data every which way to make sure there are no hidden safety issues or other problems (and also to see if there are any hidden advantages that they could promote). The more they know, the better our products.

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

IMPROVED FACTOR PRODUCTS

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

GC Pharma Acquires Catalyst's Hemophilia Assets

2/27/23 GC Pharma, a South Korean company previously known as Green Cross, has agreed to purchase the hemophilia assets that were being developed by Catalyst Biosciences. Catalyst decided to get out of the hemophilia field and concentrate on some of their other discoveries. They had been developing 1) dalcinonacog alpha (DalcA), a higher-activity longer-acting subcutaneous factor IX product, 2) marzeptacog alpha, activated (MarzAA), a higher-activity longer-acting subcutaneous factor VIIa product for inhibitor treatment and 3) a factor IX gene therapy based on DalcA. These products had been showing very positive results in lab and clinical studies. GC Pharma plans to continue development of these products with an eye toward the global market, including the US. (Note that GC Pharma, formerly Green Cross is not the same company as Green Cross of Japan, which used to own Alpha Therapeutics, a plasma fractionator in Los Angeles that is now owned by Grifols.) [GC Pharm press release, 2/27/23 and Catalyst press release, 2/28/23]

HEMA Presents New Information on SEVENFACT

12/11/22 HEMA Biologics distributes SEVENFACT, an activated factor VII product for treatment of hemophilia A or B with inhibitors. SEVENFACT is manufactured by HEMA Biologics' parent company LFB SA. At ASH, HEMA and LFB presented new information from their Phase III clinical study. In 396 inhibitor patients (both As and Bs) they found that a 225 µg/kg initial dose was much more effective for most patients in resolving mild and moderate joint bleeds than the also-approved 75 µg/kg initial dose. At the 75 µg/kg initial dose they found 82% efficacy after 12 hours and 96.7% efficacy after 24 hours. At the 225 µg/kg initial dose they found 91% efficacy after 12 hours and 99.5% efficacy after 24 hours. [ASH abstract 2469]

12/12/22 They also found a low incidence of rebleeding (subsequent bleeding at the same site) in subjects receiving SEVENFACT at either initial dose, after 48 hours. The incidence of rebleeding through 24 hours was 0.2%, and was 4.1% through 48 hours. All rebleeding incidents were resolved with a single 75 µg/kg dose of SEVENFACT and none required a hospital or HTC visit. [ASH abstract 3805]

Medexus Receives Prophylaxis Indication for Ixinity

11/17/22 Medexus Pharma's Ixinity had lost its indication for routine prophylaxis in children (≥ 12 years old) after the FDA realized in 2021 that they

had mistakenly granted it. Rixubis (now from Takeda) was supposed to have orphan drug exclusivity for the prophylaxis indication for children. Now that Rixubis' exclusivity period has expired, FDA has re-approved the prophylaxis indication for Ixinity. [FDA letter to Medexus 11/17/22]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so a person clots when they should and doesn't clot when they shouldn't. The clotting system is a complex 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 Presents Phase IIa Extension Results for SerpinPC

12/10/22 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. At ASH, they reported on an extension study in which participants in their Phase IIa studies continued on SerpinPC for an additional 18 months. They saw a reduction in the median annualized bleeding rate (ABR) from a pre-treatment baseline of 35.5 to 2.2 after treatment. (The high pre-treatment ABR of 35.5 was due to the subjects being treated on-demand rather than prophylactically before being treated with SerpinPC.) The product was well-tolerated with no product-related adverse events and no evidence of thrombotic issues. Centessa is currently planning additional Phase II studies and a Phase III pivotal trial. [ASH abstract 188]

CHOP Reports on Anti-Factor V Antibody

12/11/22 Researchers at Children's Hospital of Philadelphia (CHOP) have developed GB5, a monoclonal antibody that reacts with factor V, increasing the production of activated factor V, which in turn, works with activated factor X to promote clotting. They believe that activating more factor V, rather than inhibiting anticoagulants may be a safer method to restore clotting with less risk of thrombosis. [ASH abstract 397]

Coagulant Therapeutics Reports on Anti-APC Nanobodies

12/11/22 Coagulant Therapeutics was founded in 2019 by researchers from Bayer. They are developing a recombinant factor VIIa product for acute bleeding. That product will not be useful for inhibitor treatment because it has a very short half-life, which is considered a safety feature in treating acute bleeding. However, they have also started looking at inhibition of activated protein C (APC) as a possible treatment for hemophilia and other disorders.

At ASH, they reported on a library (a collection) of nanobodies that bind to APC. Nanobodies are miniature antibodies modeled after the small antibodies found in llamas and other camelid species. Nanobodies are basically only the section of an antibody molecule that actually binds to another protein. One of their advantages is that they can bind to a smaller section of a protein that is not accessible to a larger antibody molecule.

APC has three known functions. It is an anticoagulant (controls bleeding), an anti-inflammatory (controls inflammation) and a cytoprotectant (protects cells). It turns out that they have developed nanobodies that can inhibit one, two or all three of these functions. Thus, they could potentially inhibit the anticoagulant effect of APC without interfering with its other functions. It is not known whether the company will pursue this further. [ASH abstract 402]

Novo Reports on Progress with Concizumab

12/10/22 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. They presented four papers at ASH: 1) In inhibitor patients (76 As and 51 Bs), concizumab was effective in reducing median ABRs to zero, with no reported thromboembolic events. There were no observed differences between patients with hemophilia A or B. [ASH abstract 191]

2) They looked at health-related quality of life of in 133 male patients with inhibitors on concizumab using both a general questionnaire and a hemophilia-specific questionnaire. On the general questionnaire, they found significant improvements in both general health and mental health. On the hemophilia questionnaire, they found improvements in most categories as well as a significant improvement overall. [ASH abstract 1157]

3) In looking at treatment burden in 133 patients with inhibitors, they found a highly significant improvement for concizumab. [ASH abstract 2466]

4) In 52 male patients with inhibitors, they found a significant increase in physical activity by patients on concizumab. [ASH abstract 2486]

2/9/23 At EAHAD, Novo presented six papers on concizumab: 1) They reported additional data from their Phase III study, again showing median ABRs of zero for both hemophilia A and B patients with inhibitors. [EAHAD abstract OR06]

2) They looked at the effect of concizumab on results from a number of assays used in hemophilia treatment. The assays included the Prothrombin Time (PT) and Activated Partial Thromboplastin Time (aPTT), which are used to look at overall clotting. They also looked at the specific assays for factors VIII and IX by both clotting time and chromogenic substate. They found that "Concizumab had no, or only a minor effect on standard PT and aPTT assays, and FVIII op FIX activity assays." [EAHAD abstract PO010]

3) They looked at treatment burden and patient preference in A and B patients with inhibitors from the Phase III study using questionnaires. The results show that 93% of patients preferred prophylaxis with concizumab for three main reasons: "fewer bleeds" (75% of patients), "requires less time" (43%) and "less painful to inject" (33%). [EAHAD abstract PO104]

4) Another study showed improved patient-reported health-related quality of life for patients on concizumab prophylaxis compared to those treated on-demand with clotting factor. [EAHAD abstract PO133]

5) The next study looked at longer-term results in patients on concizumab prophylaxis. In 104 patients with inhibitors who had completed at least 56 weeks of treatment, they still found a median ABR of zero and no new safety issues. [EAHAD abstract PO140]

6) The final concizumab paper at EAHAD was about active lifestyles of inhibitor patients on prophylaxis. They found that patients spent an increased amount of time engaging in moderate or moderate-to-vigorous physical activity. [EAHAD abstract PO204]

Pfizer Starts New Pediatric Phase III Study of Marstacimab

January 2023 Pfizer is developing marstacimab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) to control bleeding in patients with hemophilia A and B, with or without inhibitors. Marstacimab is a once-weekly subcutaneous injection. They announced that they are starting a new Phase III study of marstacimab in pediatric patients. [Pfizer press release, January 2023]

1/6/23 Pfizer announced that it is stepping back from early-stage R&D for rare diseases. What this means for hemophilia is not yet clear, however, they will continue all projects that are already in clinical studies including marstacimab and hemophilia gene therapy. [Barron's article 1/6/23]

Silence Therapeutics Reports on Anti-Protein S Product

12/12/22 Silence Therapeutics is developing SLN140, an inhibitor of the anticoagulant protein S, to control bleeding in patients with hemophilia A and B, with or without inhibitors. SLN140 is a silent interfering RNA (siRNA) that inhibits the messenger RNA (mRNA) that liver cells use to produce protein S, thus reducing the amount of protein S that is made. At ASH they reported on the effect of the treatment in mice and non-human primates (NHPs).

In NHPs, they found that a single dose of SLN140 significantly reduced the amount of protein S that is made over a period of three weeks. Thrombin is the final enzyme in the clotting cascade, so the amount of thrombin produced can be used as a marker for the amount of clotting that would be observed. In normal NHPs, SLN140 appeared to increase the amount of thrombin three-fold. When the NHPs were given anti-factor VIII antibodies to reduce their factor VIII levels and give them acquired hemophilia A, those treated with SLN140, still generated normal levels of thrombin and thus, normal clotting. This study was in support of future clinical studies with SLN140. [ASH abstract 693]

Sanofi Completes Fitusiran Clinical Studies Achieving Both Primary and Secondary Endpoints

4/4/23 Sanofi is developing fitusiran, an inhibitor of the anticoagulant antithrombin, to control bleeding in patients with hemophilia A and B, with or without inhibitors. Fitusiran is a once-monthly subcutaneous injection. It is a silent interfering RNA (siRNA) that inhibits the production of antithrombin, an anticoagulant.

Sanofi has published two papers in the journals The Lancet and The Lancet Hematology reporting the results of their successfully completed Phase III clinical studies, which met both their primary and secondary endpoints. One study was on patients with hemophilia A or B without inhibitors and the other was on A and B patients with inhibitors. The primary endpoints were a significant reduction in annualized bleeding rate (ABR). The secondary endpoints were product safety.

In the inhibitor group, the ABR was reduced to an average of 1.7 bleeds per year compared to 18.1 bleeds per year for subjects receiving on-demand bypassing agents. Two-thirds of the subjects on fitusiran had ABRs of zero.

In the non-inhibitor group, the average ABR was 3.1

compared to an ABR of 31.0 for on-demand treatment with clotting factor. About half of the subjects on fitusiran had ABRs of zero. Note that they didn't report an ABR for subjects on prophylaxis with clotting factor, which should be much lower than for on-demand treatment, as seen in other studies reported here.

The most frequent adverse event was an increase in alanine aminotransferase (ALT) levels in 32% of the inhibitor group and 23% of the non-inhibitor group. Increases in ALT indicate inflammation of the liver. Two early subjects with inhibitors had thromboembolic complications (too much clotting) after which Sanofi reduced the dose to slightly increase the subject's antithrombin levels. No thromboembolic complications were seen in the non-inhibitor group, and there were no deaths in either group.

Studies after the two patients developed thrombosis have suggested that a target antithrombin level of 15 - 35% of normal should give the best results for improving clotting while minimizing the risk of thrombosis. In that light, Sanofi is also studying the safety and efficacy of every-other-month dosing and lower doses.

With the studies completed, Sanofi now has the data required for submission of a license application to FDA for approval of the product, but no timeline has been announced. [Sanofi press release 4/4/23; Srivastava A et al., Lancet Hematol, online ahead of print 3/29/23; Young G et al., Lancet, online ahead of print 3/29/23]

12/11/22 Sanofi presented three papers at ASH: 1) In semi-structured interviews of 24 patients from their Phase III studies they found improving joint health, decreasing bleeds, improving joint mobility and an overall reduction of treatment and disease burden. [ASH abstract 3565]

2) In 65 patients (50 As, 15 Bs, 19 inhibitor, 46 non-inhibitor) from the Phase III studies, they found consistent improvements in health-related quality of life in all categories. [ASH abstract 3559]

3) In today's world, we generally classify bleeding tendencies in relation to factor VIII or factor IX levels. That's not possible with rebalancing agents because they do not change those levels. Therefore, there's a need to be able to correlate levels of anticoagulant lowering with bleeding risk. Using both literature and clinical data, Sanofi was able to show that lowering of antithrombin levels to 15 - 35% of normal is approximately equal to the effect on bleeding of increasing factor VIII levels to 10 - 20% of normal. They didn't look at factor IX levels, but we know that bleeding rates at similar factor VIII or factor IX levels are roughly comparable. [ASH abstract 2472]

2/9/23 At EAHAD, Sanofi reported on an analysis of the efficacy and safety of fitusiran in adolescents with hemophilia A or B, with or without inhibitors from their three Phase III studies. The studies included 43 adolescents (ages 12-17 years) and showed significant decreases in ABR with no safety issues that differed from those in adults in the studies. They also found significant improvements in health-related quality of life. [EAHAD abstract PO119]

New Company Vega Therapeutics Developing Anti-Protein S Treatment

12/12/23 Vega Therapeutics, a recent spin-off from Star Therapeutics, is developing VGA039, an inhibitor of the anticoagulant protein S to control bleeding in patients with von Willebrand Disease (vWD). Although they are focusing on vWD, VGA039 may also work to control bleeding in hemophilia and other bleeding disorders, and Vega seems to be leaving that option open. Protein S is a cofactor for the anticoagulants TFPI and activated protein C (APC). A cofactor is a protein that binds to and increases the activity of another protein. For instance, factor VIII is a cofactor for factor IX. Without factor VIII, factor IX has very low clotting activity. Similarly, inhibiting protein S also reduces the anticoagulant activity of TFPI and APC.

At ASH, Vega presented lab and animal data for VGA039, showing that it has the potential to be a novel hemostatic agent (clotting promoter) for both vWD and other bleeding disorders. [ASH abstract 691]

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.

New WFH Gene Therapy Registry Aims to Fill Knowledge Gap on Hemophilia Treatment

The World Federation of Hemophilia (WFH) has launched a gene therapy registry to monitor the safety and effectiveness of gene therapies for hemophilia. The registry is called the WFH Gene Therapy Registry and aims to collect data from people with hemophilia who have undergone or are planning to undergo gene therapy. The registry is expected to provide valuable information to researchers, clinicians, and patients about the long-term safety and efficacy of gene therapy for hemophilia. WFH Vice President, Medical Glenn Pierce comments "Patient safety is all of our responsibility. Collecting data in one global registry—the WFH GTR—is essential to ensure that rare adverse events, in a small patient population over a large geographical area, will be detected." Patient participation is voluntary.

Hemophilia is a rare genetic disorder that affects the blood's ability to clot, leading to prolonged bleeding and other complications. Gene therapy is a promising new treatment for hemophilia that involves replacing or correcting the faulty genes that cause the disorder. However, gene therapy is still relatively new, and its long-term safety and effectiveness are not yet fully understood.

The WFH Gene Therapy Registry will help address this knowledge gap by collecting data from people with hemophilia who have undergone gene therapy. The registry will track information such as the type of gene therapy used, the patient's bleeding episodes, and any adverse events that occur. This information will be used to monitor the safety and effectiveness of gene therapy and to inform future research and clinical practice. HTCs, where most gene therapy treatments will be administered, will invite patients to participate, once they have made the decision to receive gene therapy. Patient data will be de-identified for privacy. The registry is supported by a number of hemophilia organizations and product manufacturers worldwide.

The registry is an important step forward for the hemophilia community and for gene therapy research. It will provide valuable insights into the long-term safety and efficacy of gene therapy for hemophilia and help to ensure that patients receive the best possible care.

For more information: https://wfh.org/research-and-data-collection/gene-therapy-registry/

CSL Presents Study Results on Hemgenix

12/9/22 CSL Behring's Hemgenix, a gene therapy for factor IX, was approved by FDA on 11/22/22. They presented results from the clinical studies in three papers at ASH: 1) In the 54 patients treated in the Phase III clinical study, 33 had no pre-existing antibodies against the AAV5 vector and 21 did have pre-existing antibodies. CSL looked at whether the levels of pre-existing antibodies made a difference in the outcome of the treatment. They found no significant difference in factor IX levels, ABR or safety for anti-AAV levels up to a titer of 1:700. Only one subject, who had a high level of 1:3212, failed to express factor IX. (These antibody levels, 1:700 and 1:3212 are called titers. The assays work by diluting the sample until they see no more antibody. So, the larger the number after the colon (:), the higher the amount of antibody in the sample.) [ASH abstract 2139]

2) The second paper looked at the durability (ability to keep producing FIX) of factor IX expression. They found no significant decrease in factor IX levels over 24 months and all patients that had discontinued prophylaxis were able to remain off prophylaxis. [ASH abstract 2141]

3) The third paper looked at durability over three and five years for patients in the Phase IIb study of Hemgenix (formerly AMT-061) and in the Phase I/II study of AMT-060 (a similar precursor of Hemgenix), respectively. They found no significant loss of factor IX activity and consistently low ABRs. There were no new safety concerns. [ASH abstract 2142]

CSL presented six papers on Hemgenix at EAHAD:
1) The first paper looked at the elevated alanine transaminase (ALT) levels in 11 of the 54 Phase III subjects. Elevated ALT is a result of liver inflammation. There did not seem to be a difference in the pre-treatment medical data between the patients with elevated ALTs and those without. The average time to the first elevated ALT reading was 46.5 days, range 22 to 120. Nine subjects received corticosteroids to control their liver inflammation. The average length of treatment with corticosteroids was 79.8 days, range 51 to 130. The average factor IX level for the nine subjects was 22.2% maximum, 17.1% immediately before the start of corticosteroid use, and 17.9 two weeks after stopping the steroids. Thus, it appears that the steroid treatments "rescued" the subjects factor levels and kept them from decreasing further. [EAHAD abstract PO040]

2) The second study looked at durability, an update of the ASH paper. They found no issues with durability and no correlation between durability and pre-existing anti-AAV levels (for levels under 1:700). [EAHAD abstract PO155]

3) The third paper was similar showing stable factor IX levels for 24 months and significantly reduced ABRs compared to prophylaxis. [EAHAD abstract PO156]

4) The fourth study was again an updated version of an ASH paper, showing long-term durability in the pre-Phase III studies after up to five years. [EAHAD abstract PO157]

5) The third paper looked at health-related quality of life after Hemgenix treatment in the Phase III study. In the first year, they found no change, but in the second year they started to see small but significant improvements in various categories, including increased optimism for the future. [EAHAD abstract PO165]

6) The last study looked at treatment with Hemgenix compared to prophylaxis with extended half-life factor IX concentrates. This was a study on paper comparing the results of the Hemgenix Phase III study to those for Alprolix, Idelvion and Rebinyn. As expected, Hemgenix showed significantly lower ABRs, as well as lower overall factor usage. [EAHAD abstract PO206]

2/21/23 Hemgenix was given a conditional marketing authorization by the European Commission. It is now the first hemophilia B gene therapy approved in Europe. BioMarin's Roctavian gene therapy for

hemophilia A was approved previously, but it has not yet been approved in the US. [Medscape, 2/21/23]

2/23/23 The data from the Phase III study was published in the New England Journal of Medicine (DOI:10.1056/NEJMoa2211644). Unfortunately, there is a charge to download the article.

Pfizer Announces Phase III Results for Hemophilia B Gene Therapy

12/29/22 Pfizer is developing fidanacogene elaparvovec, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. The results of their Phase III study in 45 patients were published in a press release. The study met its endpoint of showing superiority in annualized bleeding rate (ABR) compared to prophylaxis with factor IX concentrate. The pre-treatment average ABR for all bleed types was 4.43, compared to an average ABR of 1.3 after 15 months of treatment. The average factor IX level after 24 months was 25%.

The product was generally well-tolerated. Two serious adverse events connected with the product were reported. One was an elevation in liver enzymes, a marker of liver inflammation. The other was hemorrhage of a duodenal ulcer that was associated with corticosteroid treatment of liver inflammation. There were no deaths, no inhibitor development and no evidence of thrombosis. The subjects will continue to be evaluated over the course of 15 years. Pfizer plans to discuss the results with the FDA and the European Medicines Agency (EMA) in early 2023. Pfizer licensed the product (then known as SPK-9001) from Spark Therapeutics in 2014. [Pfizer press release, 12/29/22]

2/6/23 The Padua variant of factor IX is a mutated form that has an activity about 8 – 9 times greater than that of normal factor IX. It was a natural mutation discovered in a family in Padua, Italy. Because of its higher activity, that family had problems with thrombosis, too much clotting. However, the Padua variant, also called FIX R338L, is being used by all of the current factor IX gene therapies. uniQure, the original developer of CSL's Hemgenix, managed to patent the Padua variant. Now Pfizer is suing uniQure to disallow that patent. [Law360 article, 2/6/23]

2/9/23 Pfizer presented three papers on gene therapy at EAHAD: 1) The first was a study of gene therapy treatment preferences for people with hemophilia in the UK. The study correlated responses to questionnaires by 125 people with hemophilia (122 male, 3 female, 81% As, 19% Bs). The most important concern was safety followed by treatment burden. Interestingly, treatment effectiveness was among the least important concerns. [EAHAD abstract PO338]

2) Another study looked at the prevalence of antibodies against AAV vectors in Europe. Pfizer's product cannot be used in patients with anti-AAV antibodies. They found that there is variation in the numbers of patients with antibodies across the five countries studied. They also found that the types of AAV against which the subjects had antibodies varied by country. Therefore, there was no consistent result that would apply to all of Europe. [EAHAD abstract PO164]

3) They also looked at the patterns of joint bleeds in patients that had been treated with fidanacogene elaparvovec. The majority of patients already had target joints or arthropathy prior to the study. All new bleeds, except one, were in target joints or those with pre-existing arthropathy. [EAHAD abstract PO163]

Takeda Reports on Long-Term Safety and Efficacy of BAX 335 Gene Therapy

12/12/22 When Shire acquired Baxter/Baxalta, Shire decided to discontinue work on Baxter's original gene therapy for hemophilia B, BAX 335. However, Shire, now part of Takeda, continued to monitor the long-term safety and efficacy of BAX 335 in the eight patients who had been treated in the clinical studies between 2013 and 2015. BAX 335 used the Padua version (also called FIX R338L) of the factor IX gene, as does CSL's Hemgenix and several other gene therapies in development. So, we now have results from the longest ongoing gene therapy study using the Padua variant.

At ASH, Takeda presented the results of the long-term follow-up. Although three of the eight patients have dropped out of the long-term study, one of the remaining subjects is the only one who showed actual factor IX production. His factor IX levels have ranged from 10% to 40% of normal. He's now back down to about 10% after 7.2 years. He hasn't needed factor IX infusions since the third week of treatment.

Although this treatment wasn't successful, the method was similar to those for the other factor IX gene therapies, so it may give us some evidence of long-term safety. No additional adverse events have been reported and no instances of malignancy (cancer) or thrombosis (too much clotting) have been observed. There was also no evidence of inhibitor development. However, anti-AAV antibodies have persisted at levels that would preclude re-dosing with any of the other current treatments. [ASH abstract 4780]

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THE GREATEST PARADOX TO GROWTH AND FULFILLMENT

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

The years between 2006 and 2009 will go down in my history as the most intense and volatile season in my life. I cannot imagine anything, nor dare comprehend a more burning crucible than those burdensome and intense years.

In 2006, just a year following the largest business promotion in my life up to that point, my world turned upside down. While signs of something wrong were somewhat evident for about a year, the first nuclear warhead was dropped when my hematologist contacted me. I still remember his grave voice over the line. As a direct result of my severe hemophilia B diagnosis at birth, I contracted hepatitis C due to tainted factor injections during the mid to late 1980s. The doctor reported that hepatitis had now caused cirrhosis and scarring on my liver and invasive treatment was required to save the organ. There was a problem, however. The treatment was potent on the body and would likely destroy my transplanted kidney my father donated 16 years earlier. I fought with the doctor, indicating that there had to be a different alternative. "Sorry, Matt. I'm afraid we have no other options." His response was empathetic but those words stung like a hornet. I was the sole provider and my wife and I were raising three young daughters—ages six, three, and one. How could we possibly do this?

This proved to be just the beginning of many warheads yet to drop. I could fill volumes of all that occurred but, to be brief, not only did my kidney fail as predicted, but that kidney turned out to be full of cancer—renal cell carcinoma. The kidney was surgically removed and I was completely reliant on dialysis treatments every other day at a clinic in the next county. Even worse, doctors were unsure if they were able to get all the cancer, so I couldn't seek out a kidney transplant for three years. One by one, other systems started shutting down. I developed a bad case of pneumonia and experienced respiratory failure on two different occasions requiring doctors to induce a coma each time so they could ventilate and give my lungs time to heal. On top of everything else, if you can believe it, new symptoms surfaced that worried my treatment team including grand mal seizures, paralysis, dangerously high blood pressure, anemia, and crushing fatigue. The diagnosis? West Nile Virus, one of the most severe forms that went straight to my brain. Some infected, stray mosquito decided to feast upon my luscious blood! It came to the point that no one knew which symptom came from which malady.

I don't share these intimate details to be pitied. I share them to make a point—one of the greatest principles that I believe exists. A principle that I've dedicated my life researching and applying to every person I treat in clinical practice. In fact, this is a principle so fascinating, I've dubbed it the greatest of all paradoxes. To adequately explain this principle, I have to finish my story.

I have been fortunate to have been born into a large family with parents who have a depth of knowledge and wisdom that astound me even now. I've always hoped I could one day be at least an equal. Seeing me struggle so hard during the apex of my crucible, not to mention the corresponding trials my wife and family were experiencing, Dad and Mom came by to visit. After some small talk, Dad stepped forward and shared a thought that has never left me. "Matt, it seems to me that you're advancing faster in your professional career right now than you would be if you were able to work." I remember staring at him blankly, considering the absurdity of the idea. "How could I be advancing faster in my career? I'm on long-term disability!" I thought. "I live in a broken body that can barely sustain life!" He admitted his uncertainty about how everything would come together but he was adamant that I could expect this outcome in the near future. "It's a law," he said. "The greater the pressure, resistance, and opposing forces, the greater the strength. If harnessed correctly, the outcome is more polished and refined. How could you not be advancing faster in your career?"

Years later, while doing research on this subject, I came across a poem entitled "Good Timber" by Douglas Malloch that taught this very same principle.

The tree that never had to fight
For sun and sky and air and light,
But stood out in the open plain
And always got its share of rain,
Never became a forest king
But lived and died a scrubby thing.

The man who never had to toil
To gain and farm his patch of soil,
Who never had to win his share
Of sun and sky and light and air,
Never became a mighty man
But lived and died as he began.

Good timber does not grow with ease:
The stronger wind, the stronger trees;
The further sky, the greater length;
The more the storm, the more the strength.
By sun and cold, by rain and snow,
In trees and men good timbers grow.

Where thickest lies the forest growth,
We find the patriarchs of both.
And they hold counsel with the stars
Whose broken branches show the scars
Of many winds and much of strife.
This is the common law of life.

The greatest of all paradoxes, the grandest of all principles is found in an idea that many philosophers, religious texts, scientists, and even engineers have coined differently. For the purpose of this article, I'll simply call it the law of opposition. The greater the opposition, the greater the power upon the agent it is acting upon. Like a crucible, with the chaos, burning, and intensity that it inflicts, the final result is an agent that is powerfully tough and unbreakable. The crucible refines and violently twists off the dross, perfecting the agent for its intended purpose. What's the paradox? It's simply this: We can't move forward without moving backwards! Another way this principle can be stated is that we can't expect to progress (the desired outcome) without first paying the price (the opposition). This principle, as powerful as it is, will always lie dormant in a state of inertia unless something or someone harnesses, acts upon, and channels it by putting forth governing laws that propel it to correct action. Let me share with you how my wife pulled me out of my own inertia.

Shortly after my father taught me the principle of opposition and predicting an advancement in my career, things turned for the worse and I was practically bed ridden. My wife became a nurse of sorts and always brought me my breakfast in bed. I hardly took a bite due to feeling so awful. I found it difficult to bathe as well and often lounged around in pajamas, getting out of bed later in the afternoon unless I needed to be at dialysis.

One day, my wife sat down at the edge of my bed and had a blunt but concerned conversation with me that changed the course of my prognosis. "Matt, you're not giving your body a chance. You're not trying. I understand your condition but you can't expect to heal if you don't put some ounce of muscle into your recovery. This is what I'm going to ask you to do for me. I'll bring you breakfast every morning. I want you to eat every bite. I expect you to be out of bed at noon, bathe, and put on clean clothes each day. Finally, I want you to come downstairs and join the rest of the family on the couch. You don't have to do anything, but I want you to be with us."

To this day, I'm not sure why those stern but loving words from my wife had such an impact upon me. As she was talking, I knew I couldn't be defensive or lay out the reasons why I couldn't fulfill her requests. Just like turning a key and igniting a vehicle to life, I made a commitment to give my body a chance. The task was often extraordinarily difficult. While I got used to eating, getting in and out of the shower was exhausting let alone dressing and going downstairs. I would often close my eyes afterwards but I kept doing it and each time it would get a little easier. What my wife had taught me was how to harness the opposition I was experiencing and, by utilizing correct laws and principles, channeling it towards my desired outcome—improving my current health circumstances. All of this could not have been possible without the patient yet persistent guidance of a loving spouse and by her introducing another principle that is beyond the scope of this article—the principle of transcendence, complimentary to everything that has been discussed.

As an ending to this story, three years after my crucible began, I finally received another kidney transplant by a loving sister. My liver healed, the West Nile virus cleared, and cancer was no longer detected in my body. Eight months later after I started working again, I was offered another position in the same company that promoted me to a management role with a nice raise to boot.

It is not my intention to paint a simplistic picture with a fairy-tale ending although I was remarkably blessed with the outcomes just illustrated. Nothing was easy and it was a fight to the end. I'm not necessarily even saying to expect jaw-dropping results. Still, the evidence and laws that support the law of opposition with its activating agents must not be treated lightly. Evidence of the laws are found everywhere including in nature, exercise science, psychology, biology, philosophy, physics, and a number of other fields of science and even religion. I invite readers to reflect upon the opposition that's being played out in their lives, reframing the struggle as an opportunity, and discovering the activating laws that will harness and channel opposition into desired outcomes. Truly, one cannot move forward without moving backwards—the greatest of all paradoxes!

DANNY'S DOSE

TAKE OFF THE ROSE-COLORED GLASSES AND CREATE YOUR TREATMENT PLAN

INTERVIEW WITH DARLENE SHELTON BY RENAE BAKER

"Do you know that it's terrifying for a family to think that a paramedic wouldn't be able to administer the life-saving medication they need in an emergency?" That's what Darlene Shelton asked her local EMS after an eye-opening 911 call for her grandson, Danny, who has severe hemophilia B.

The EMS didn't have the necessary factor and wouldn't have been allowed to administer the product the family might have had on hand. She said it as gently as she could, "It's terrifying that you guys don't have more of the education that you need to help us." Darlene and her family wanted to be part of the solution, so they asked, "How can we help you to do your job so that we – as patient families, and you as paramedics – are protected and have peace of mind?"

Little did Darlene know, back in the 1990s when she became certified as an accountant, that she would one day become the founder of Danny's Dose, a 501(c)(3) non-profit, representing over 28 million people, which educates both medical personnel and families with rare diseases. However, that became the answer to the question she asked that terrifying day! "God put it on my heart that there were lives to be saved," Darlene says as she muses about how this has become her mission work.

"When we started Danny's Dose, we had three main goals:

1. To raise awareness
2. To address the lack of proper care due to outdated protocols, which prohibit paramedics from administering patient-carried medications and/or deviating from standing protocols when it comes to specialized care. This potentially puts patients with rare and chronic conditions in danger of extensive pain and suffering, increased recovery time, organ damage, brain damage, and even death.
3. Reduce the fear of the unknown for both patients and medical staff

That fateful day, when Danny was just four years old, Darlene realized that she, her family, and countless other people with rare and chronic diseases had been "going around with rose-colored glasses on" believing that they could call 911 in an emergency and have the proper medication administered. That was unacceptable to her, and she was determined to do something about it.

"At the very beginning," she starts, "I knew that there was a multitude of information that I didn't know, because we're not medical. So, I made it a plan to befriend as many EMS and emergency specialists as I possibly could."

"I said, 'Tell me everything that I need to teach families from your perspective.'"

Three hours later, Darlene had a legal pad full of notes which she turned into an ever-expanding resource to help families with rare diseases protect their loved ones. Now, not only does Darlene have many friends in the EMS world, but she sits on the board of Missouri EMS for Children, is a national liaison voice for the rare and chronic disease patients for EMS for Children and has been asked to serve on the committee for the Pediatric Pandemic and Disaster Preparedness Network. Shelton takes pride in having built a very strong relationship with HRSA throughout the country and has been exhibiting at state and national EMS conferences, including the World EMS conference. The work Darlene has done through Danny's Dose, has pushed forward legislation that is making it possible to educate medical staff and equip EMS to be able to save the lives of people with rare diseases in several states. The work is far from done, however.

Darlene also wants to highlight EMS For Children. "Their role is to assure that care for children is evaluated regularly and help with any needed changes in education and community outreach. State-to-state, the quality of these boards varies," Darlene says, diplomatically. "But a few phone calls from families can help change that."

The number one complaint she hears from EMS is that they don't know the local families and therefore cannot be prepared for them, Darlene feels that the most important information she can give the families afflicted

with rare diseases is, "It is *your responsibility* to go meet with your local EMS and ER to create a treatment plan." Due to HIPAA laws, they cannot reach out to you.

A treatment plan is a plan of action for treatment the day that an event happens. However, the execution of the details is key. When the family goes to their local EMS or ER for the purpose of setting up a treatment plan, they can expect to sit down with their health professionals and cover as many scenarios as they can think of and how they'll handle them before there is a big accident or injury.

"You need to go make sure that it's taken care of ahead of time and that you have a good plan."
Darlene knows that many families are unaware that they need to do this or are too intimidated to initiate the process, so she teaches them what to do and say. She has created an easy-to-use outline which includes what to expect, what information you should take to the meeting, and what constitutes a good treatment plan. An early version of this is on the website, **www.dannysdose.com**, under "***Tips and Products for Protecting Your Loved One***." It provides these five tips with detailed bullet points:

1. Obtain EMERGENCY MEDICAL ORDERS from your specialist.
2. For medications: Create an EMERGENCY MEDICINE KIT.
3. Contact your local Ambulance Service.
4. Contact your local hospital if you don't live near your Treatment Center.
5. Always have Personal and Auto Medical Alerts.

On this page, you can also find the Danny's Dose Safety Products: Emergency Headrest Covers (which include a pocket for items listed on the outline as well as "Emergency & Medical Information Card") and Automobile Alert Decals.

Darlene explains how to start the process. "I tell the families to google 'EMS/ER near me.' Call and say, 'We are the (name) family. We wanted to let you know we live in your service area, and we'd like to make an appointment to come in and work on a treatment plan.' It's literally that simple."

When asked if the treatment plan might be created over the phone or online, Darlene says, "I tell families they should go in. You are going to need all your physician information and as much of your diagnostic information as you have." Don't worry that you aren't educated in medicine. Families know a lot about these conditions!" She reminds us. "And they have hands-on, real-life experience."

As an example, "Sometimes physicians say, 'Maybe it's a bleed…maybe it's not,' but families have been taught that for a blow to the head, stomach, or kidney area – you treat. Period. That's what EMS needs to be told. When the family member says, 'This is what we have been taught are the standard treatment protocols," it makes the EMS/ER staff feel better.

"Meeting the staff in person," Darlene continues, "also gives them a chance to get a feel for 'who's the calm one.' You know – 'Do we have a family that's really timid?'" Unfortunately, sometimes, the way a person is diagnosed with a bleeding disorder is because of a brain bleed that caused brain damage. Letting the medical teams meet your family in a controlled setting will let them know if there are any cognitive issues ahead of an emergency.

"They need to know if there are pre-existing cognitive issues that go along with the disorder," Darlene emphasizes. "A really good treatment plan will include a trip to your home so that they can see, when they bring the stretcher in, the best door to go in. Where's the factor kept? What if you have a babysitter that day, and you didn't think to tell them? If the EMS already has that in their notes, they will know where to go."

They will also be interested to know what your childcare schedule is. Who is the secondary level of contact? "What if it was a major car accident, and Mom or Dad are not able to give any authority? They can have Grandma's or Grandpa's phone number on the treatment plan, and they can make that call.

"When you read the outline, you'll see," Darlene teases, "that a really good treatment plan includes all the way down to the point of where a helicopter would land at home, school or even the soccer field.

You may learn, as Darlene did, that your local hospital tells you that they don't know how to treat hemophilia and that they prefer you don't go to them.

"I'm not angry. I'm glad they told me." Darlene says. You don't want to be in the middle of a major emergency and lose time learning that and having to be air lifted to a different hospital."

There is so much life-saving information that Darlene has for you, that there isn't space in this article to include it. She invites you to not only go to the website, but also to call her personal number if you have any questions.

What can you do to help Darlene continue to help you? Contact her and tell her what state you live in and if you've had good and/or bad emergency experiences with your local EMS and ER.

Thank you, Darlene, for helping us take off our rose-colored glasses and take our health into our hands!

women & girls with hemophilia

articles to support, educate, and empower

Laughing Matters

AN INTERVIEW WITH KATHY PERKINS
BY RENAE BAKER

Kathy and her family are part of a long succession of bleeders. "Survival humor" seems to have become part of their DNA!

Her great-grandmother had six boys with hemophilia. She remembers a great-uncle who lived into his 60s back when that was very rare for someone with hemophilia. "My great aunt had 13 kids, including twin boys; one who had it and one who didn't." Kathy's grandmother was a carrier, and she had two daughters who were carriers, including Kathy's mother. "My mother only had me, so she had no idea, although she had severe bleeding every menstrual period."

Knowing hemophilia ran in her family, it was important to her to tell Dennis before they got married. "He responded, 'The Perkins genes are so strong, they'll overpower anything!'" She is laughing, of course.
Kathy, Dennis and their sons, Matt and Joe, made a home in Michigan where Kathy and Dennis still spend half of the year. The other half of their year is spent in Florida.

My sons, Matt and Joe are Irish twins. Both have severe Hemophilia B. By the time they learned that Matt had the diagnosis, Kathy was already pregnant with Joe. Coming home from the hospital to a newborn and a one-year-old didn't leave Kathy any time to think about whether her hemorrhage meant that she was more than a carrier.
"It never crossed my mind," Kathy admits. "When the boys were nine and ten and we were doing homecare, I got tested. I was at 30-40%, and the doctors said that I shouldn't have any bleeding issues."

Kathy wanted to become a nurse but made the decision to stay home with the boys, while taking cleaning jobs and running her own resale store, Patina.

"Infusing at home was a hard thing for me to do," Kathy admits, "But, we were trained on a dummy arm, and then we practiced on each other!"

During this time, Dennis was a mechanic and the International Service Representative for Getlin Mining Equipment. "When I couldn't get the boys' IVs started," Kathy remembers, "the boys would say, 'Mom, just take me to Dad. He'll start it!'" Dennis continues, "I was the engineer of the boiler system at the plant. I couldn't go off the property, and they couldn't come into the factory so –" Kathy chimes in, "They had to do it on the loading dock. He'd always get it started, and then I'd finish it."
Kathy eventually learned how to start the IV, which enabled her to become a nurse's assistant and phlebotomist.

During those years, the boys had to deal with teachers not believing they really had hemophilia and making them do things in class that were dangerous for them. There was a breach of confidentiality when a hospital worker, the wife of the gym teacher, looked into one of the boys' records to "make sure he wasn't lying about" having hemophilia. Once it was confirmed, the school wouldn't let them go on field trips, and they had to take a special bus to school. One of the boys had spent three weeks hospitalized, and the school administration told Kathy and Dennis he wouldn't be able to graduate. Dennis, remembers, "I told them, for someone with hemophilia, missing three weeks is nothing!" Because of Dennis' advocacy, his son graduated. One of the boys contracted hepatitis B and had to be homeschooled for three months.

"The boys were always lying on the couch with their arm or leg up over the back, wrapped up and elevated," recalls Dennis, "Back then, every day was a life-or-death situation." Kathy continues, "From about three to ten years old, they were in the hospital every week."

But there were such good times too. They bought a sailboat and saw adventures sailing the Great Lakes. They were on a shoestring budget, but they found lots of ways to have fun. When the boys were older, they went into business with Dennis

repairing mining vehicles.

The whole family is very involved in The Coalition for Hemophilia B, including granddaughter, Emily. “The Coalition has been wonderful!” Kathy and Dennis rave. They’re impressed with the information the Coalition puts out and the ways in which they are working with women. “They’ve been amazing! They’re doing so much! They seem to know what’s needed, and they do it!”

Even though she and Dennis are in their 70s now and at a different stage of life, they are very impressed with the Coalition retreats for couples. “This is especially good for young families, because [hemophilia] is hard on a marriage. It takes over, and your marriage is put on the back burner.” Kathy notes “You’re so involved with your children, that you don’t even think about it. So, it’s good that the Coalition is focusing on the husband and wife because they really need that.”

Another thing that isn’t a laughing matter to Kathy is what she sees as dismissive attitudes of doctors toward women. When she was in her 50s, she was riding her bike when she felt a “pop” in her groin. “I didn’t know it was a bleed,” Kathy remembers of her iliopsoas muscle injury. “It really hurt. I let it go for about a week, thinking it would get better. By the time I went to the hospital, it was really bad, and the doctor came in asking, ‘How do you know it’s a bleed? I don’t see any blood!’ This was at the HTC!”

“They did everything they could to prove that it wasn’t a bleed,” Dennis recalls, protectively. Luckily, there was a good hematologist on hand who asserted, “Stop testing, and just treat.” “At that point, the doctors said, ‘Yeah, we think you need to have factor on hand,’” Kathy laughs.

“But the doctor still wanted to wait until I had a severe bleed to treat, and I said, ‘Now wait a minute. If I know a bleed is starting, then I want to treat before it gets bad. Just like any man.” She was able to get the doctor to finally agree with her.

Kathy believes that if she, herself, hadn’t had a good hematologist, early on, who recognized the bleeds and gave her factor, her current doctor would not have given it to her. She found she needed to take pictures of her bleeds and give them to the doctor. With these, she included articles, brochures, and information to help educate the doctor and advocate for herself.
At 75 years old, Kathy only infuses as needed, “But I’m thinking I’m going to ask about prophy, because even my friends say if I treated, then I could walk with them.” Kathy explains that she’d like to be more active, but if she walks a mile or so, she experiences knee bleeds. She would also like to get back to golfing, which causes bleeds in her thumbs.

A message Kathy wants to give others: “Know your factor level and even if it shows high but you have severe bruising, a heavy menstrual cycle or feel a bleed happening, take action! Prevent joint damage!

I get so frustrated with women who have children with hemophilia who are clearly bleeders and still call themselves ‘just carriers’ or ‘symptomatic carriers.’ It’s important to know, even if you think you’re ‘safe.’ What if you have a serious injury or require surgery? You could bleed, and the doctors are not going to give you what you need to stop it.” Kathy hates to think of her friends not living their best lives because of unnecessary pain.

Kathy is proud of her two boys (now grown men) They’ve grown up to be really good men. They learned how to tolerate pain, which helps them tolerate a whole lot of other things. They’re patient, kind and positive! The glass is always half full. They will always be “my boys.”

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

Together, they're the Heroines of Hemophilia B.

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Medexus Pharma, Inc., Chicago, IL 60606

MEDEXUS PHARMA

ADVOCACY NEWS

BY GLENN MONES

NEW PLASMA CAUCUS IN THE HOUSE

A new plasma users' caucus has been established in the 118th Congress. It is composed of legislators who have identified the American plasma supply as an important issue for Americans.

Although the number of Americans with hemophilia B using plasma derived therapies is very small, there are still some patients who use them. In addition, there are many Americans with a variety of other conditions who need these treatments. This represents significant recognition of the importance of plasma to the American people.

Currently, the caucus is comprised of a small number of legislators, but it is expected that others will be recruited over time. The Coalition for Hemophilia B works closely with a variety of other patient organizations to ensure that plasma receives appropriate attention.

WASHINGTON DAYS

Bill Patsakos, Bradley Schoenfeld, and other community members visited elected leaders and staff during NHF's Washington Days. The event took place in person from March 8-10. A central focus of the agenda was asking Congress' support for the HELP Copays Act (H.R. 5801).

The legislation is designed to stop insurance companies from using copay adjusters that prevent patients from counting outside assistance against their insurance payments. The effort is being conducted through a diverse group of organizations in the All Copays Count Coalition.

You can learn more about this campaign here: allcopayscount.org/take-action/ and about a variety of advocacy efforts from the CHB website at hemob.org.

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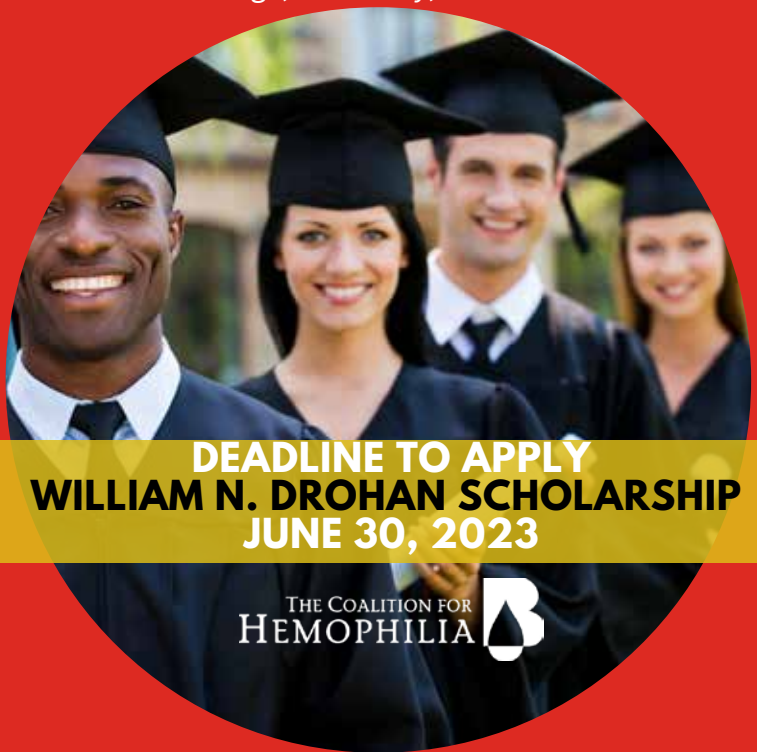

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TRANSFORMATIVE SISTERHOOD: REFLECTIONS FROM THE 2022 VIRTUAL WOMEN'S RETREAT

BY ERICA GARBER

The ***2022 Virtual Women's Retreat*** was a heartwarming and unforgettable weekend filled with joy, sisterhood, and personal growth. Held entirely online from December 2nd to 4th, 2022, this retreat was an inspiring gathering of women who came together to learn, share, and connect. Like many of our retreats, this was not just an event, but a transformative experience for all those who participated. It was a chance for women to connect, to be seen and heard, and to feel supported by a community that understands their struggles.

From the powerful and inspiring sessions led by renowned speakers and experts to the intimate conversations shared over chocolate and pajamas, the retreat was a safe haven where women could let down their guards and truly be themselves. For many, it was a chance to heal, to find hope, and to renew their spirits.

The retreat began on Friday, December 2nd, with a warm welcome from our beloved, Kim Phelan. We celebrated a few birthdays, our newest members donned their tiaras, and we all flew balloons into the air in unison, setting the tone for the fun and creative activities that lay ahead. We were also welcomed by our sponsor Sanofi, where we had the chance to thank them all for joining us and making these events possible.

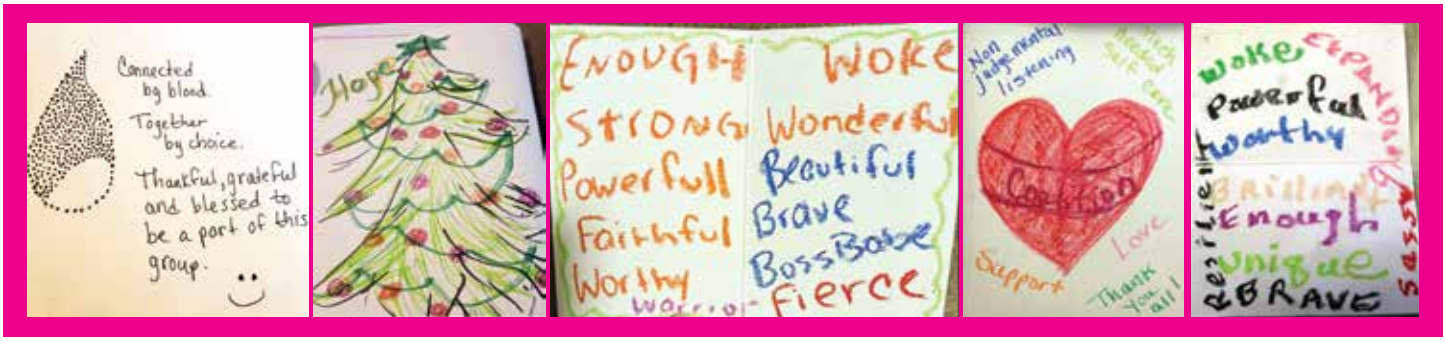
Rev. Cazandra Campos-MacDonald kicked off the retreat with her moving session on "The Comparison Trap: Break Free from the Lies We Tell Ourselves." Using her own experiences, Rev. Campos-MacDonald shared tools and strategies for breaking free from the cycle of comparisons and finding true self-acceptance. Her message was powerful and uplifting, setting the stage for a weekend of empowerment and transformation. Later that evening, Renk Kocturk led an intention setting session that encouraged participants to set clear intentions for their personal growth and self-care. Participants stretched their bodies and minds, getting ready for a weekend of deep exploration and growth. The evening continued with an inspiring rap session led by Jacquie "Lady J" Maddix, a caregiver and beloved member of the community. Lady J shared her wisdom and love, creating a safe space for participants to open up and connect with one another.

Saturday morning started with a rejuvenating practice led by Renk Kocturk, followed by a session on "Balancing Overwhelm and Implementing Self Care" led by Tracye Hamler, an up-and-coming leader in the world of women's retreats. Her session provided participants with tools and strategies for organizing their lives and taking control of their health and well-being.

After a delightful tea party with Erica Garber, participants broke out into two sessions that explored the medical and educational aspects of bleeding disorders. Led by Rachel Kroouze and Ashley Smith, these sessions were informative and interactive, providing participants with in-depth information and practical strategies.

The day continued with a creative arts therapy session led by Shanay Johnson, who showed participants how to use art and music as a powerful tool for coping with chronic illness. Donnie Akers then led an informative session on finances and debt management, helping participants navigate common financial pitfalls and plan for a more secure future. The night ended with an empowering session led by Grammy-nominated poet and spoken word artist Gha'il Rhodes Benjamin, followed by a fun-filled pajama party and pocketbook bingo. The atmosphere was electric, with participants laughing and connecting late into the night.

Overall, the 2022 Virtual Women's Retreat was an unforgettable experience that brought together a community of women who shared their love, strength, and resilience. It was a weekend of joy, bonding, and learning, and an opportunity for each participant to set their intentions for a more fulfilling and meaningful life.



The impact of the retreat goes beyond just the weekend itself. The connections made and the lessons learned continue to ripple out into the lives of the participants and the community at large. Women who attended the retreat have reported feeling more empowered, more informed, and more resilient in their daily lives. They have formed new friendships and support networks and have gained the tools and strategies to better manage their bleeding disorders and their overall health and wellbeing.

The 2022 Virtual Women's Retreat was a shining example of the power of community and the resilience of the human spirit! As the hemophilia B family, we all extend our deep thanks and endless gratitude to our sponsor Sanofi for continuing to support women, prioritizing relationships, empowerment, and community.

COMMENTS:

"The women's retreat was so powerful and connected. I met amazing women and obtained very useful and valuable information!"

"The retreat was wonderful. Well planned and executed. I really enjoyed myself!!"

"This Women's Retreat was amazing. The sessions were amazing, and the speakers were awesome. All of them. I am a new member to the Coalition for Hemophilia B. Being able to engage with this group of beautiful women is truly an amazing experience for me. I could feel the love and compassion that we all felt for one another. I learned a lot about myself and that I'm not alone in this journey. So grateful for this opportunity and for many more to come."

"It was one of the best virtual women's retreats. I cannot wait to see more such sessions in the future."

"The 2022 Virtual Women's Retreat was a fantastic way to unwind, learn new things, laugh and cry, and connect with other women who experience or support a family member with Hemophilia B. The stories and experiences shared by the women were raw, tender and brought me to tears. It was great to learn relaxation techniques and it has been a long time since I enjoyed unique child-like activities that brought joy and laughter to my life. Thank you for the experience and I look forward to the next women's virtual retreat!"

"The women's retreat was so nice. It's refreshing and encouraging to be with others that are going through the same things as I am. The retreat is also an intimate time to meet those newly diagnosed, to be there to support and answer questions in a casual way. Thank you CHB for bringing us together."

"It was a wonderful weekend. I am convinced that the community of The Coalition for Hemophilia B is the right place for my son and our family. It makes me understand that I'm not alone and that we are a wonderful support team."

"I am so thankful to the Coalition for having this event for the women of our community. It is awesome to feel the support of other women who are going through some of the same situations you are going through. To be able to relax and take care of YOU first is a welcome change. Thank you!"

"Women's retreat helped me feel so much less lonely in the hemophilia world. I see so many people struggling. I am not alone and that gives me energy to be my best self!"

"It's a great experience for blood sisterhood and education. It's good for expressing concerns and confidence and we're all in the boat."

sanofi

GINGERBREAD FUN!

BY ROCKY WILLIAMS

Deck the Halls! On Saturday, December 17th, the hemophilia B community gathered around their computers for a festive treat. We celebrated by hosting our annual Gingerbread Decorating Contest, and participants showed extraordinary creativity. There were gingerbread houses with snowy roofs, pretzel windows, candy wreaths, gumdrop fences, and licorice trim. There were even snowmen, campfires, and miniature elves that made their way into these festive scenes.

We danced to holiday music and delighted in holiday themed games. There were prizes for the best decorated houses as well prizes for our holiday Kahoot trivia winners. But most importantly, families shared a fun evening together with lots of cheer and laughter.

Our thanks to our event sponsors, Medexus Pharma and Paragon Hemophilia. Stay tuned for more fun family events in the near future.

PARAGON HEMOPHILIA

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NACCHO

BY ROCKY WILLIAMS

This year's NACCHO was a stellar success and privilege to attend! Held in Phoenix from Jan 5–8, the AZBD (Arizona Bleeding Disorders) held this year's conference designed for and created by community members to enhance youth and camping programs for the bleeding disorder community. NACCHO stands for North American Camping Conference for Hemophilia Organizations, and it brings together a wealth of knowledge from across the globe.

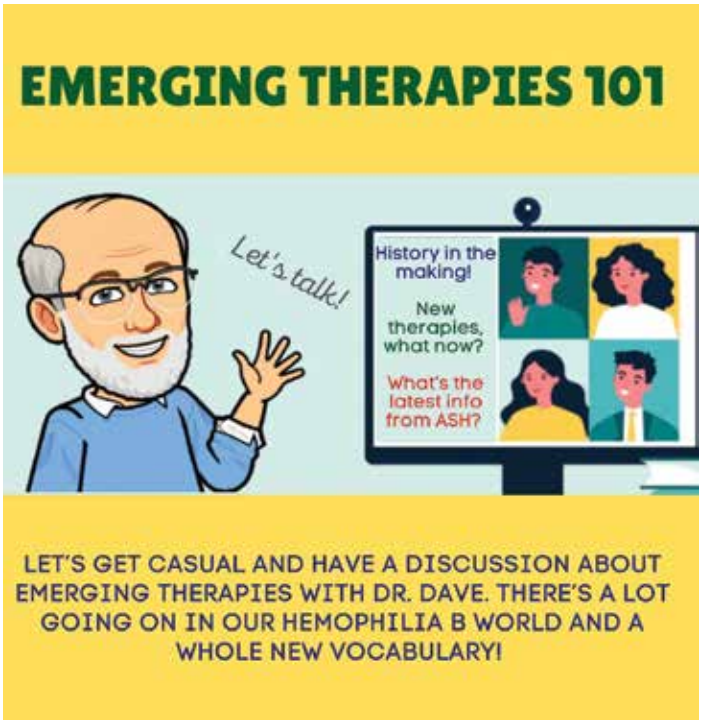
It was an honor to learn from so many camping programs here in the US and from our friends abroad. We learned best practices that foster community connections, inclusive belonging, and ways to teach independence. NACCHO is truly an event that leaves you motivated, prepared, and excited to serve the community. A giant thanks to the AZBD, their sponsors Pfizer and Sanofi, and everyone who helped to create such an empowering program!

EMERGING THERAPIES 101:
AN EVENING WITH DR. DAVID CLARK

BY GLENN MONES

Only a few years ago, the number of therapies available for hemophilia B were very limited. That situation has changed dramatically with the emergence of many new therapies and more coming every day. To help our members make sense of all of it, the Coalition for Hemophilia B has created a variety of programs to explain not only the existing and recent treatments but also those in development and envisioned just down the road.

Among the most popular of these are our virtual "Emerging Therapies101" evenings with Coalition Chairman Dr. David Clark. Dr. Clark has a PhD in chemical engineering from Cornell University and more than 35 years of experience in the development and manufacture of plasma and tissue products, including Factor IX concentrates.



Much of that experience comes from the time he spent working with the American Red Cross. Dr. Clark has repeatedly used his expertise and verbal skills to help community members gain the understanding they need to navigate hemophilia B and its treatment.

In its latest incarnation, Dr. Clark led an engaging session on January 12, 2023, from 7:30 – 9:00 PM ET. The program was made possible through the generous support of CSL Behring.

Accompanied by a very comprehensive slide presentation, Dr. Clark broke down the therapies into a variety of categories including standard half-life extended, extended half-life, inhibitor treatments, gene therapy and others. What many members found particularly exciting what is the fact that this was the first time that one of Dr. Clark's presentations included the first gene therapy product that has received government approval and will soon be widely available. He also highlighted several products that are likely to receive approval soon, as well as some that may not come to fruition.

The event continued with plenty of time for members' questions and answers. Finally, we had a fun, interactive quiz that allowed members to see how much they had learned during the session.

Many thanks to our sponsor CSL Behring, to our members, staff and everyone who makes our programs possible. Stay tuned for more virtual and in-person opportunities to learn about these vital subjects.

CSL Behring

GEN IX ADVOCACY

BY JACOB POPE

The Coalition for Hemophilia B and Medexus recently hosted Generation IX Advocacy at YMCA Camp Surf in Imperial Beach January 26-29, 2023, which was attended by a diverse group of individuals committed to advocating for those affected by hemophilia B. The retreat provided an opportunity for advocates of all backgrounds, ages, and personal experiences to come together, learn from one another, and develop skills for creating positive change in our community.

One of the key themes that emerged from the retreat was the importance of building strong relationships with others who share similar values and goals. Participants explored the theme of Community Advocacy through several lenses- personal, interpersonal, social, and policy advocacy. The need for collaboration and partnership to achieve meaningful impact across each of those domains was emphasized. Through a series of engaging workshops and creative group activities, we were able to connect with one another and forge lasting relationships that will support our work going forward.

Another important theme that emerged from the retreat was the need to be strategic and intentional in advocacy efforts. Participants discussed the importance of understanding the political and social landscapes in which we are working within and developing strategies that are tailored to the specific contexts in which we operate. We also emphasized the need to be flexible and adaptable in the face of changing circumstances, and to be willing to adjust our approaches- but not our values -to achieve our goals.

Throughout the retreat, we had opportunities to learn from one another and share best practices for effective advocacy. We discussed a variety of approaches, including grassroots organizing, Washington Days, and involvement with HTCs and the Coalition for Hemophilia B. We also explored the role of caregivers, friends, and family in advocacy efforts, and discussed strategies for engaging with policymakers and other decision-makers.

One of the highlights of the retreat was a day trip to the Museum of Us in Balboa Park, near the San Diego Zoo. The exhibits emphasized the importance of persistence and resilience in the face of setbacks and challenges, and what actions and behaviors are harmful or beneficial to communities. After immersing ourselves in these exhibits, group reflections then highlighted the need to build broad coalitions and engage a wide range of stakeholders in advocacy efforts.

Overall, Gen IX Advocacy 2023 at YMCA Camp Surf was a valuable opportunity for advocates to come together, learn from one another, and develop strategies for creating positive change in our community. We left the retreat feeling energized and inspired, with a renewed sense of purpose and commitment to our work. One participant, Jeron, described the experience as “one of love” and “empowering, because we are all bettering ourselves...for the bleeding disorder community as a whole.”

CHB staff member, Erica Garber, joined the group for the first time at one of our Gen IX events. It was clear from day one that this experience was so instrumental in making an even deeper connection with our community around a shared passion for advocacy and experiential learning. “I learned so much about what matters most to the hemophilia B community, and ways to stand up for those values through action. I am so



inspired by the ways, once again, we came together as a group and are stronger for it. I left feeling ready to step up and use my voice in support of all that was shared and more!" they shared in reflection. On the final day the GutMonkey team invited every attendee to make one promise to themselves about taking advocacy action, put it in writing and handed them all to Erica in solidarity. The next step, soon to come, is for Erica to check in with all attendees in three months' time to encourage and support their advocacy goals.


Looking forward, it is clear that advocacy efforts will continue to be essential in addressing the many social and political challenges facing our community. By building strong relationships with each other, being strategic and intentional in our efforts, and by persisting in the face of obstacles, we can make a real difference in the world around us- whether the difference we make is to the heart of one person in pain, to the minds of decision-makers and those in power, or in the smiles of 30 people who come together from all across the country united by shared experiences, is a decision left to each of us to make. Gen IX Advocacy at the YMCA Camp Surf was an important step in that direction, and we thank the Coalition for Hemophilia B and Medexus for enabling this powerful experience that we look forward to seeing the positive impact of in the months and years to come.

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Y.E.T.I. 2023: THE GAME OF LIFE

BY ROCKY WILLIAMS

What's your next move in the game of life? For teenagers, the transition into young adulthood is the perfect time to meet new people and learn from youth education experts from across the bleeding disorder community. That's why Pacific Northwest Bleeding Disorders (PNWBD) hosted this year's edition of Y.E.T.I. from February 9–12, 2023 at Camp Collins in Oregon.

Y.E.T.I stands for Youth Effectively Transitioning to Independence, and it gathers youth ages 14 - 20 for an immersive weekend of experiential learning, networking, and FUN! This year's theme was Game of Life, and GutMonkey led the participants through a team game where groups were given the opportunity to participate in a number of challenges spread throughout the campsite. PNWBD and GutMonkey partnered together to stage an incredible weekend of sessions on mental health, stress, movement, insurance, and staying warm.

It was an honor to attend Y.E.T.I. this year. The opportunity to watch GutMonkey and PNWBD work with these impressive teenagers and young adults made me proud and excited to be a part of the program. It was also so great learning from others attending as well. Sharing ideas and learning from my peers in the bleeding-disorders community is always such a powerful experience. It was a privilege to discuss how to shape events and interactions for the highest-quality experience for our attendees and celebrate all types of success. Huge thanks to their inaugural sponsor Pfizer, as well as sponsors Bayer, Genentech, Sanofi, and Takeda for supporting an event wholly focused on providing new and creative ways to teach our youth.

LOVE AND CUPCAKES: A SWEET VIRTUAL EVENT WITH DANIELA'S LITTLE WISH

BY ERICA GARBER

Love was certainly in the air at our annual Virtual Valentine's Cupcake Workshop on February 11th. It was a delight to have Daniela's Little Wish, a non-profit that has been baking smiles for kids for over 10 years, as our partner in this sweet event sponsored by Medexus Pharma. Our community members gathered virtually with their families to enjoy creating some delicious and beautiful treats. With Daniela as our instructor, everyone was excited to learn new baking techniques.

Before the cupcake decorating began, Grant Belsham from Medexus Pharma hosted a love-themed trivia, where we learned all kinds of fun facts about love, love letters, and the history of Valentine's Day celebrations. Then, it was finally time to get our hands dirty and start decorating cupcakes with Daniela!

We all tried our best to follow Daniela's expert instructions, carefully measuring out ingredients and skillfully piping frosting onto our teddy bear cupcakes. It was hard to resist the temptation to lick our fingers or sneak a bite of candy, but we reminded ourselves to save room for our finished creations. Daniela's clear and concise instructions made the process easy to follow, even for the novice bakers and decorators among us.

We joked about our messy kitchens and our attempts to keep the supplies away from our pets and kids, but with Daniela's patient guidance, we managed to create some truly stunning cupcakes that looked almost too good to eat (almost)!

After the decorating was done, we wrapped up the event with some hilarious Kahoot games and raffles. It was wonderful to see so many smiles over the computer screen, as families came together to enjoy some virtual Valentine's Day fun. But we soon realized

that the big kids (aka the adults) were having too much fun, so we decided to give the kids a chance. We had an extra bonus hangout just for our kid cupcake decorators, where we learned fun facts about animals, tested our knowledge of superheroes, and battled it out over cartoon trivia.

Thanks to Medexus Pharma for sponsoring this ever-popular workshop, and thanks to Daniela for sharing her expertise and her infectious love of baking with us! We can't wait for our next baking event with her, and in the meantime, we'll be enjoying our delicious (and beautiful) cupcakes.

COMMENTS:

"I truly enjoy the food decorating events."

"This is the third one we did, and my daughter and I enjoyed it each time. And it is fun that it was a little different from last time."

"It was great to take a break from our busy schedules and decorate the cupcakes as a family!"

"The bears were adorable."

"I am not a baker, but I enjoyed making the cupcakes and the time with my boys."

"We enjoyed the event so much. It was just the two of us, so it was such a perfect time for just us. I'm getting better at ZOOM, but still not great, and probably won't get much better till the grandkids get a little older and are able to teach MiMi. Thank you so much."

"I really missed my friends and am so glad I got to see them again!"

"We loved decorating the cupcakes. My kids made some of their own unique creations with the supplies provided and that was fun too. We had so much fun with the Kahoot trivia! Thank you for having virtual events like this one."

"My son really enjoys this opportunity to be creative and spend some time with the bleeding disorders community."

"It was awesome and a lot of fun! We are looking forward to next time!"

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THE COALITION FOR HEMOPHILIA

WHAT I WISH I HAD KNOWN...

Uncensored & Uncut

REAL-LIFE LESSONS FROM **YOU**- THE EXPERT

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IN THIS TOGETHER: CHB COUPLES' RETREAT REUNION

BY ROCKY WILLIAMS

In 2022, we held our first-ever couples' retreat, called "In This Together," in person, in Atlanta, Georgia. It was an incredible experience where 14 couples spent a weekend sharing their stories, bonding, finding deeper community with one another, and learning about themselves and their partners.

To rekindle the connection, we hosted a virtual reunion on February 13, 2023, the day before Valentine's Day! Perfect timing for a session on relationships! Sponsored by CSL Behring, the event brought the couples back together, and the couples were thrilled. The best part of the virtual couples' retreat was "seeing our friends again!!" said Sandy. Others agreed and added they also enjoyed the game we played called the Dynamic Duo Game, which you may remember made famous by the TV show The Newlywed Game.

To play, we split up the men and women and asked them a set of questions. I asked the men, for example, "Who would your wife say made the first move? What one word would your wife say best describes her driving: Safe, Fast, Angry, or Whoops?" Then, we asked the women to answer the questions: "Who made the first move? What one word best describes your driving?" We also swapped roles and asked the women to answer about what their husbands would say, such as, "What would your husband say is a hidden talent that he has… that should stay hidden forever?" The results were pretty hilarious.

Social workers, Dave and Karen, who led sessions at the 2022 in-person retreat in Atlanta, also signed on for the reunion. They joined the men's and women's breakouts, and the group warmed up quickly to the vulnerability and openness they had shared in Atlanta.

"I was so impressed with the resiliency of these women!" Karen said. "They were able to look at their strengths as well as what they wanted to have happen in their relationship. I think having been with the couples in person and spending time with them after our session contributed to this relationship. In other words, David and I just did not come to the retreat, facilitate a workshop, and leave. [It was great] to stay and interact with the couples…they got to know us and accepted us."

David agreed that the evening was a meaningful one. "I had many powerful discussions with the men – most who seemed hungry to talk," he said. "I could

have talked with a few of them for an hour or longer. It was very apparent the breakouts with Karen and myself were needed and I am glad you gave them the opportunity to connect with us. Many of them remembered me and I sensed they were comfortable being honest and open."

"Thank you again for the honor of walking with your community," Karen added. "To be invited into their journey is a trust that David and I take very seriously."

Overall, the best part of the event was giving the couples the space to connect. From the breakouts in men's and women's groups to the rap session led by Carl and Gwyn at the end with everyone, it was great to just have the time to talk. Carrie reminisced, "We enjoyed spending quality time with other hemo couples. Having Dave and Karen too was a nice bonus! Thank you." And James said, "The retreat itself was awesome. It was a great to get away without the kids and reconnect. And the retreat reunion was a lot of fun to see everyone again and play The Newlywed Game again. Hopefully there will be another couples' retreat in the future, we would love to attend again."

We would like to give a huge thank you to Carl and Gwyn for leading the rap session and to Karen and David for hosting the breakout sessions. We would also like to give a giant thank you to CSL Behring who made this program possible through their generous support. We hope the couples enjoyed receiving a framed photo of themselves from the in-person retreat to put on the mantle and have a daily reminder of the friendships and community they are building!

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THE COALITION FOR HEMOPHILIA

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GETTIN' IN THE GAME WITH ZAYDEN

BY ROCKY WILLIAMS

More than 50 children and their caregivers gathered in Phoenix, Arizona, in October 2022 to participate in CSL Behring's Junior National Championship (JNC) program. Attendees learned the fundamentals of baseball, golf, or swimming, competed in friendly matches with other kids with bleeding disorders (age 7-18), and attended educational seminars reiterating the importance of physical fitness.

Representing the Coalition for Hemophilia B was 9-year-old Zayden! Zayden earned his spot at age 8 after submitting an application highlighting his love of baseball, which he's been playing for more than two-thirds of his life at this point, since age 3. He won his first baseball ring at age 5, when he hit the ball to the fence and got an infield home run to win the game! The team rushed the field. "It was a dream come true," Zayden said. He told CHB that he would love to play in the JNC, and CHB agreed that would be an excellent idea.

Zayden was surprised to win and go to JNC for the first time. As soon as he got there, he wanted to get onto the field and start playing. An avid first basemen, he loved the coaches and meeting all the new friends on the team. "The coaches cheered me on and pushed me…like my mom." Mom is always there to cheer him on, both in Phoenix and at home, where he plays two games a week all summer.

One of the coolest moments, according to Zayden? "When one of the coaches pitched me the ball and I almost hit him with the ball." Nice swing! "Another time was when one of the older boys threw me the ball as hard as he could. I caught it and got the runner out. It was exciting." Wow, what an experience!

One of the coolest moments, according to mom? Meeting professional baseball player Kameron Loe, who pitched to the kids during competition day. According to Zayden's mom, "He spoke to the kids prior to playing and they all really seemed enthralled that a current professional player was with them for the day!"

"My favorite thing about baseball is being a role model to younger kids," says Zayden, specifically his cousins, who he helps with batting practice. He learned how to play baseball from his dad and brother, and he said he'd definitely recommend CSL Behring's JNC program to other kids in the hemophilia community. "It is great to meet new people who have hemophilia like me. It helps you learn how to manage your bleeding disorder and how to play with your bleeding disorder," he said. "Gettin' in the Game is the best."

What's next for Zayden? "Go pro!" We can't wait to watch and cheer you on, Zayden!

TEEN PALOOZA

BY ROCKY WILLIAMS

CHB is grateful to have hosted an incredible set of virtual teen events from November through January, with the help of Sanofi and our teen leaders: Nick, Gabriella, JC, and Alex.

Each event was championed by exceptional hemophilia and advocacy trivia, a captivating game, a stirring rap session, and time to just connect and be together. We would like to give a giant thank you for the teens that helped promote, create, and facilitate these events. We're also super grateful to the therapists who helped facilitate our teen rap sessions, which brought so much value and structure to those conversations.

NOV 17 WITH NICK: NOW THAT'S JUST DRAWFUL!

The silly was on full display in November with Nick's event, which kicked off in earnest with a welcome from Sanofi representative, Ashley, and a conversation picking up from a previous event about the best type of pizza toppings. Do peppers or anchovies belong? It's a hotly contested debate, and we encourage you to weigh in next time! Therapist Matt Barkdull and Nick led the discussion, which also included a discussion of ice cream and monster trucks. Conversations definitely left teens laughing and knowing each other much better.

The main event was a game of Drawful Animate, which has players draw two-frame animations based on positively absurd prompts. These included "parts of Linda have become octopus," and "opening an accordion folder of all my issues." It was hilarious! Watching the drawings come to life was absolutely ridiculous and so much fun!

After the event, one parent later shared, "The teen events have been very beneficial and helps the teens who participate to network and meet others in the community. It has been a positive experience for both of my children who have participated. Thank you for including siblings as well!! They look forward to each event."

"I thoroughly enjoyed making an event with CHB," said Nick. "It was fun to be able to bounce off Rocky when co-hosting. I learned that it's important to express yourself, and it was great to see how willing the other teens were to bounce jokes, comments, etc. off each other. I would 1000% recommend teen events for anyone wanting to talk with other teens in the community, and I would be more than thrilled to get the opportunity to host another event in the future!"

DEC 8 WITH GABRIELLA: WHICH CAMP ARE YOU IN: ARE YOU A CAT PERSON OR A DOG PERSON?

Gabriella gave an enthusiastic welcome to the teens gathered on December 8 before launching into an icebreaker discussion of collections. Our game that night was about sorting things, so we wanted to know, "What do you collect?" If you were one of the participants that night, you might have said Pokémon cards, seashells, water bottles, or silverware. I, for one, collect board games. What fun to compare collections! What do you collect?

The rap session led by Robert Friedman was quite lively. We battled with the age-old questions of dog vs cat. After clearly delineating the cat and dog people, the teens played Quixort: a trivia game in which teams sort falling answers into their proper order before they hit the floor. "It was so much fun!" said one teen.

DEC 15 WITH JC: WHAT'S A PIRATE'S FAVORITE COOKIE? SHIPS AHOY!

Our last teen event of 2022 was riddled with my AMAZING pirate jokes, including, "Why did the pirate refuse to say, 'Aye, Aye, Captain?' Because he's only got one eye!" After everyone got done laughing their faces off, all the teens got the chance to share about where they're from and their most prized possession.

A rap session with JC and Robert Friedman included a discussion of what we'd spend money on if we had all the riches in the world, to which one teen responded, "Laser tag on the moon." I'm ready to be player 2 for that one.

As the main event, we played Poll Mine from Jackbox: a survey game of survival! Players divide into two teams, secretly answer survey questions, and try to guess the results. "I had a lot of fun hanging with the other teens," commented one participant. "My favorite part was making the event with Rocky," said JC.

JAN 19 WITH ALEX: ARE YOU A CAR DEALERSHIP TYCOON?

After a welcome from the hosts and Sanofi, Alex led the group through an icebreaker about their favorite types of cars. A rap session with Matt Barkdull and Alex was followed by Hyper Car Dealership Tycoon, played on Roblox, in which each player owns their own car dealership and must build their collection of unique and exotic-looking cars.

When asked if there was anything that Alex would like to share, he commented, "If you can dream it, you can do it!"

He shared that this was a great experience to create an event with CHB and it was fun to cohost one. He says that he learned, "anything can be done if you try."

THIS IS JUST THE BEGINNING!

Are you interested in planning an event that will WOW your teen friends in the CHB community? Come and join the fun! Come and create the next virtual teen experience that will have us all saying we learned a ton, participated in some exciting trivia, met new friends, and had a blast! Email me at Rockyw@hemob.org.

We would also like to give a giant thank you to Sanofi. These incredible teen events were made possible by their generous sponsorship.

sanofi

TEEN OF MANY TALENTS: RYAN

BY ROCKY WILLIAMS

Watch out world: 16-year-old Ryan from Michigan is coming your way! This teen with hemophilia B doesn't come close to letting hemophilia keep him from the things he loves, from music and chess to woodworking and "anything that has a motor and two or four wheels."

Ryan got into bikes and motorcycles about five years ago, when his dad started riding with him. The biggest challenge? "Keeping myself from getting hurt," he said. "I have to be extremely careful when driving or riding." Safety is the priority, and Ryan has worked hard to build his skills and confidence on the motorcycle. "I get a thrill out of riding." But more importantly, "This has helped me by being a stress management system."

When he's not riding, Ryan enjoys woodworking, including creating shelves, shadow boxes, and other woodshop products. He also enjoys playing the challenging and strategic game of chess, and he recently started learning to play the guitar. His favorite songs to play are "Smoke on the Water" and "Astronaut in the Ocean." He recognizes that music "is a way of decompression and a way to communicate."

His love for music has brought him to our Beats Music Program where he says that "The CHB Beats Music Program is a good way to get to know others that are into the same thing you are into." At Beats, he continues to learn more on the guitar and more about music. He loves learning and loves learning from others in the hemophilia B community.

In addition to the Beats Music Program, Ryan attended the Symposium in Orlando, Florida where he appreciated the chance to meet other people in the community and learn what resources are available to him. He says that the trip "showed me that there's more things than meets the eye" meaning there are many things that a person with hemophilia must do and that there are people here to help you.

The trip not only expanded his knowledge and understanding of hemophilia, he gained friendships and connections with fellow teens with hemophilia B. Ryan believes, "these events are a good way to connect with people with the same thing as me." Since his first symposium, Ryan has continued to take on a leadership role. He participates in our virtual teen events year-round. He comments, "I like them, and they are a good way to get the hemophilia B community together." So, come join Ryan at the next teen event. They are a lot of fun, and a great place to meet others like this teen of many talents!

FOOTBALL, SKIING, WRESTLING, AND GOLF – OH MY!
PLAYING SPORTS WITH HEMOPHILIA

BY MONTANA

Hi, I'm Montana. I live in rural Montana and am a senior in high school. My two brothers and I have severe hemophilia B and my mom has mild/moderate hemophilia.

I was very fortunate to have my mom and two older brothers pave the way for me in sports and advocacy. My mom always said that "hemophilia can't define you. You define your hemophilia." I am very passionate about three sports that have helped define me: football, skiing, and wrestling. I learned to play football and downhill ski at age 5, and I have skied recreationally all my life. I wrestled nationally through my sophomore year of high school. I have several major national wins.

My real passion was always football. I was able to participate in all the local football programs and learned to play safely and intelligently. I am very aware of the fact that most parents and hematologists disagree with the impact of the three sports I compete in. The truth is I have had less injuries in these "controlled sports" than I have in any other physical part of my life. Especially in football, I have state of the art pads and safety equipment for protection.

As an athlete with hemophilia, sports have given me comradery and a strong knowledge of being in physical shape for vein health and living a healthy life. I manage a prophylactic schedule and take it seriously. I do not infuse to play sports. I infuse to be healthy. Playing sports is an added bonus because I am healthy.

As a senior in high school and a varsity football player, I was selected as "All State Defensive Lineman" and chosen to play in the state's All-American Game in June. I was selected from all the players in the entire state based on my statistics. I played varsity all four years of high school and was a defensive and offensive lineman. I was fortunate to basically play the entire game. To accomplish this, I worked hard for months before the season to be in great physical shape by hiking, running, and lifting weights.

After high school athletics are complete, my newest passion is to pursue playing golf and outscore my golf icon, Wayne Cook. Last year, I participated in the Coalition for Hemophilia B's golf tournament and am hooked on golf!

I tend to be very goal-oriented and have also concentrated on my academics. I know I need to have good insurance as an adult. I am going to college in the fall and am majoring in pre-med. I am very passionate about hematology and pursuing a career in the pediatrics field.

When I was 6 years old, I was able to attend my first Coalition for Hemophilia B Symposium in New York City. Other than my brothers and mom, this was the first time I had ever met anyone else with Hemophilia B. I have been blessed to attend the Symposium throughout the years and participate in events run by Gen IX. I have numerous friends throughout the United States with hemophilia B. The advocacy, educational components, research, and friendship through the CHB is unmatched! The Coalition has helped define me as a young adult and given me the skills to prepare for the future. At 6 years old, I invited Kim Phelan to my birthday party because she made me feel like a part of her family. That's how I think of the Coalition community—family.





B inspired!

Stories and artwork from teens in the Hemophilia B Community

Winter 2022

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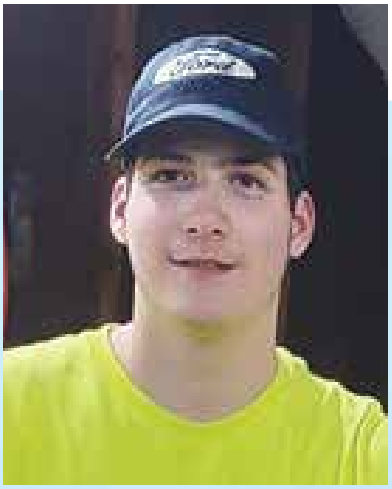

TEEN OF MANY TALENTS
RYAN

FOOTBALL, SKIING, WRESTLING, AND GOLF – OH MY! PLAYING SPORTS WITH HEMOPHILIA
MONTANA

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!