THE COALITION FOR HEMOPHILIA B

FALL 2023

# HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

WOMEN'S EDUCATION & EMPOWERMENT RETREAT

IN THIS TOGETHER PARTNERS' RETREAT

MEN'S EDUCATION & EMPOWERMENT RETREAT

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THE COALITION FOR HEMOPHILIA

## MISSION

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

# MY GENE THERAPY JOURNEY

**BY BRIAN O'MAHONY**

I was treated with Factor IX gene therapy as part of a phase three clinical trial in February of 2020, some 45 months ago. At the time of treatment, I was 62 years old and had lived a life encompassing all of the generations of hemophilia treatment. Until I was age 14, I had no access to any regular treatment and bleeding episodes went untreated. As a consequence, I had damage to my left knee, right ankle, and right elbow. At age 14, I had my first treatment with a factor concentrate which was actually a prothrombin complex concentrate.

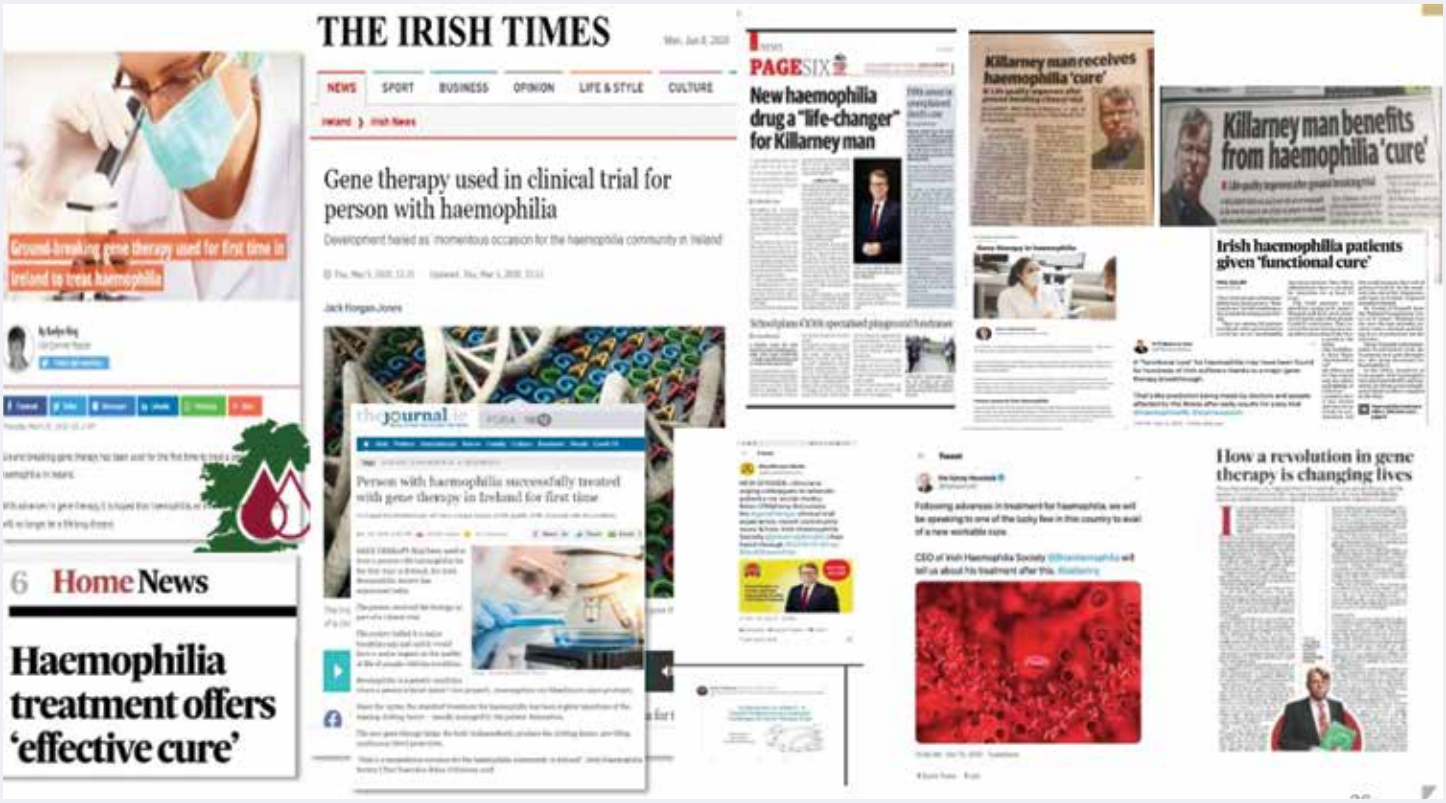
For the next three years, if I required treatment, it was fresh frozen plasma which necessitated a 400-mile round trip to the treatment center in Dublin. Obviously, given the time it took to get to Dublin, a bleeding episode in a joint would be well advanced by the time that the plasma was infused. When I moved to Dublin to go to college at age17, I had access to home treatment with prothrombin complex concentrate. This was the treatment available until my early 30s when I switched to plasma derived FIX concentrate. Then, in my early 40s, recombinant factor IX became available, and this was my treatment of choice until my late 50s, when I switched to extended half-life factor IX concentrate. I did not start prophylactic treatment until my mid 50s.

Of all of these treatment changes, in my view, the availability of home treatment where you could treat a bleed quickly, as soon as it had started, was the single biggest game changer. The availability of extended half-life factor IX was also a significant milestone which allowed me to take prophylaxis once every 10 days while maintaining a trough that kept me in the mild range.

My decision to take part in a gene therapy clinical trial was a carefully considered one. I had been following the science for many years. Together with our clinicians, I had been involved in encouraging several of the companies who were conducting gene therapy clinical trials to consider Ireland as one of their trial venues.

Over the course of the past 10 years, we had several companies who came to Ireland to discuss their gene therapy clinical trials with the clinicians and patients. I organized group meetings usually for 10 to 20 people with hemophilia who had expressed some level of interest in gene therapy to come along and hear more about the trials. We also worked with the Irish regulator to make sure that the trials could take place in Ireland.

During the course of these meetings, and following my reading and research into gene therapy, I became convinced that the benefit risk ratio for me of participating in gene therapy clinical trial was positive. I was convinced by the science of the potential of the gene therapy. Before enrolling I was aware of the unknowns and uncertainties. I knew that there was no guarantee of durability. I knew there is no guarantee of factor expression which you may achieve. I was aware some people may not get a response to gene therapy and would not achieve any significant factor expression. I was aware of the potential requirement to take steroids in the event of transaminitis in order to prevent any potential loss of factor expression achieved. I was aware of the theoretical risk of cancer from insertional mutagenesis. Despite these uncertainties, I felt that participating in a gene therapy clinical trial was the correct decision for me.



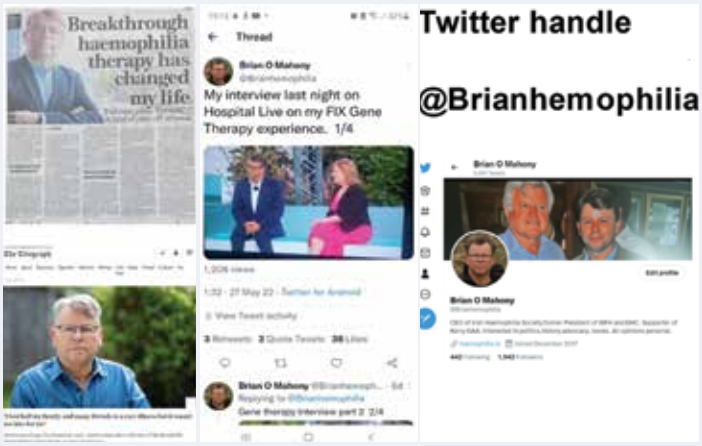

I discussed this with friends and colleagues both clinical and hemophilia organization leaders who had a range of opinions in relation to gene therapy. I discussed this with my family, but they were very clear that they trusted my judgment and knowledge. Within the parameters of the uncertainties in durability factor expression and predictability, I identified for myself my preferred personal outcomes while at all times being aware that these may not be achieved. If these were not achieved, I believed I was ready to accept whatever outcome I did achieve.

I was hoping for a factor expression in the range of 20% to 60%. My preference was for my factor expression not to be too high as I wanted to maintain some of the potential cardioprotective effect of a slightly lower factor level given my age. I hoped for a durability of at least 10 years. I hoped for a decrease in chronic pain in my damaged joints and I hoped for the ability to be more physically active and physically fit. Obviously, if I achieved a significant factor IX level, it would mean I could stop prophylaxis. This in turn would free up some of my time and allow me to have some mental freedom from dealing with my own hemophilia.

I was fully aware that given my roles with the Irish Haemophilia Society, European Haemophilia Consortium, and ongoing work with the World Federation of Hemophilia, I would not and could not achieve anything like a hemophilia-free mind, as my entire working life is consumed with hemophilia and bleeding disorders. I did hope for some freedom from dealing regularly with my own hemophilia including in areas such as not having to take prophylaxis on the days of long-haul flights and carefully planning any significant physical activities to coincide with prophylaxis days.

There were other factors in my decision also. At my age of 62, if it took several years for gene therapy to be licensed and reimbursed in my country, I may have missed my opportunity to get gene therapy. I have lived my entire life with severe hemophilia. I thought it would be interesting to try life possibly without severe hemophilia. I also wanted to lead. Despite the meetings over several years, no person with hemophilia in Ireland had participated in a gene therapy clinical trial. I was the first, and I was quickly followed by two others. With the decision made, I entered into a lead-in period of approximately 6 months before the gene therapy was infused. During that time period, I had to keep an electronic diary recording any bleeding episodes and prophylaxis. I also engaged extensively with the research team at the center where the gene therapy would be infused.

In the year prior to my gene therapy in 2019, I had traveled abroad on 44 occasions as I travel extensively for work. I had several conversations with the research team in relation to ensuring that we could schedule my follow up appointments, ideally allowing me to maintain some of my work commitments abroad. Having said that, I was fully aware of the monitoring and follow up requirements and fully committed to the protocol. I wanted the gene therapy to be successful and I did not want to jeopardize the potential for success or jeopardize the trial protocol because of travel commitments.




The monitoring visits required a visit once weekly for the first 12 weeks, followed by monthly visits for the rest of year one and bi-annual visits after that up to the end of Year 5. I was fully committed to making all of these visits and willing not to travel if that would interfere with my ability to stick to the protocol. Ironically, that was not necessary as two weeks after my dosing, the COVID-19 pandemic struck and I had no travel commitments for at least the next 18 months. This made it easier to manage my diary and schedule all the monitoring visits while simultaneously making it more difficult as it meant I had to attend a hospital setting very regularly during a pandemic at a time when most people did everything possible to avoid going into any hospital. The research team made this easier by facilitating the visits in a non-clinical setting where potential exposure to COVID-19 was very limited.

The gene therapy infusion day was relatively simple and uneventful. I attended the research facility near my hemophilia treatment center with my wife about two hours prior to the infusion to have some blood tests, final checks, and final conversations. I received the infusion over a 90-minute period, waited two hours, and went home. The procedure was simple, although I was aware that they had an emergency team ready if required in case of adverse reaction. I was very relaxed during that day, but that evening, I was emotionally drained as I realized what a momentous day this was.

I then had weekly visits for the first 12 weeks, and indeed, I exceeded this for several weeks by having twice weekly visits. I wanted to make absolutely sure that there was no possibility they would miss any increase in liver enzymes which could result in loss of expression if there was a delay in diagnosing this and in commencing steroids. The visits were managed in a non-clinical space due to the COVID-19 pandemic and they were very well managed. The only adverse event I had was a decrease in my iron due to the sheer volume of blood being taken on a weekly basis.

In the first year, I had twice weekly visits for the first three months, and then monthly visits. In total, in year one, I visited the center 30 times. I adhered fully to the protocol and abstained from alcohol for three months pre-gene therapy and two years post-gene therapy brackets (exceeding the one year recommended.) My outcome from day one was good.

My factor IX level increased from week 1 and has stayed in the high-mild or normal range over the past 45 months. Thankfully, I did not require steroids as my liver enzymes never increased. I did have a decrease in chronic pain in some of my damaged joints partly, I suspect, due to gene therapy and partly due to additional time since my knee replacement in 2018. I was fitter and more active in the first-year post gene therapy due to gene therapy and to the fact that I was not constantly traveling and was able to get into a balanced regimen of exercise and diet.

On the 1st anniversary of my gene therapy, I was walking with my family in the Dublin mountains when I slipped and fell off a low wall onto some rocks. Despite this, I did not get a bleed, and this was a revelation to me. On another occasion in the first year, I dropped a 1.5 kg dumbbell on my barefoot and again did not get a bleed. I have had two bleeding episodes since commencing the gene therapy, one of which was spontaneous, and one was due to a trauma. I have also required factor IX on a couple of occasions to top up my factor level prior to minor surgery or procedure. Now that we're in a post COVID environment, I am traveling extensively again for work. I generally do not bring any factor IX with me when I travel unless it is a long trip or a trip to a developing or emerging country where I would have difficulty accessing treatment, if required.

For exercise, I walk routinely. I commenced using alcohol moderately two years after my gene therapy infusion. I am now dealing with some other minor health issues not related to hemophilia. I continue to follow the science around gene therapy very closely and I have to say that personally I have no treatment remorse. I made the correct decision for me at the time.

My outcome has been good, but I would like to think that even if my outcome had been less satisfactory, I would not have treatment remorse because I had fully thought through all the outcomes that may occur and I had managed my expectations accordingly.

***About Brian O'Mahony, FASCLM***:
Brian has severe hemophilia B. He serves as Chief Executive Officer of the Irish Haemophilia Society and previously as President of the European Haemophilia Consortium (EHC) and President of the World Federation of Hemophilia (WFH). Brian has authored over 80 peer-reviewed scientific journal articles and is a Fellow of The Academy of Clinical Science and Laboratory Medicine (Ireland).

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

FOR ADOLESCENTS AND ADULTS LIVING WITH HEMOPHILIA B

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with long-lasting bleed protection


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**20%** STEADY-STATE TROUGH LEVELS WITH 7-DAY PROPHYLACTIC USE† + **0** SPONTANEOUS BLEEDS‡ + **7 and 14** DAY DOSING§ FLEXIBILITY FOR ADOLESCENTS AND ADULTS

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

† Mean trough Factor IX levels maintained by adults and adolescents on 7-day prophylaxis.

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

§ 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.

## IMPORTANT SAFETY INFORMATION

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

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

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

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

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

CSL Behring

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

## IMPORTANT SAFETY INFORMATION (cont'd)

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

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

**Please see full prescribing information, including patient 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**.

**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 missing 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 in children and adults 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 and hypersensitivity reactions have been reported 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 you experience.

***Based on June 2023 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 Department at 1-866-915-6958.**

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# THOUGHTS FROM AN OLD CAR NUT

**BY RENAE BAKER**

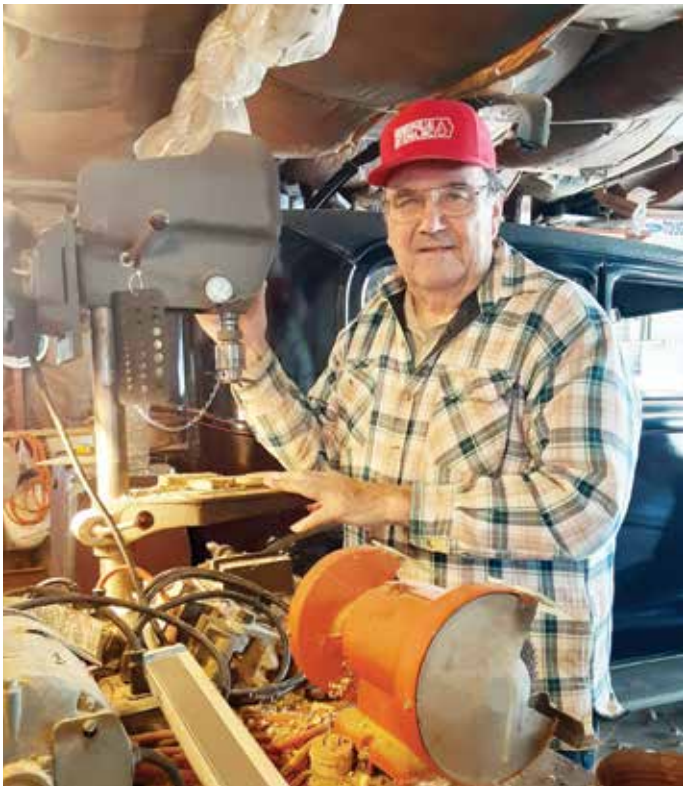
Dave Berkemann, born in 1945, calls himself an "Old Grump" and an "Old Car Nut," only one of which is true!

"When I was a boy, I was told that I was born with two diseases: Christmas Disease and Antique Cars Disease," laughs Dave. "My parents said, when I was three or four, if they couldn't find me, they'd go out in the pasture and look. There was an old Model T laying out there and I'd be sitting in it."

"I bought a Model A when I was just twelve years old. When I was fifteen, I bought a 1930 Coup. They're both still in the garage. They're shiny and they run. All original. Parades are all they're really good for." Dave loves restoring antique cars, including that old 1917 Model T that he used to hide in.

Dave grew up milking cows, feeding chickens, and collecting eggs on a farm in a small town in Iowa. He'd do his chores, bus to school, come home, and start on chores again. "Truth be known, there were many days when I wasn't doing chores because I was laid up with a hemorrhage." Pointing to his forehead, "I don't know if you can see this dent. I fell against the foundation of the barn. I don't know how I lived through it." He says with nonchalant understatement, "There was no treatment at that time."

"Back in the 1940s, they just said 'This guy's a bleeder.'" His condition was not a surprise to his parents, as Dave had a brother three years his senior, who also had the condition. He had injuries too. "His reactions to them were different than mine. I tended to take a pain pill and go on. He tended to lay down," he chuckles.



After high school graduation, "I knew I couldn't farm. I thought of becoming an auto mechanic, but when I was in the job market, many companies wouldn't consider a hemophiliac. Jobs that I would've been good at weren't available to me. The companies provided the insurance and didn't want a high-dollar patient." He decided to go to Oklahoma State Tech and took a two-year appliance repair course. He found employment at a repair company but was tasked with deliveries and pickups of heavy appliances. The work was too heavy. He went on to a variety of jobs, eventually landing in sales of municipal equipment which fit his needs and personality well. All through his working years, hemophilia challenged him. He experienced many injuries on the job, sometimes several hundred miles from home.

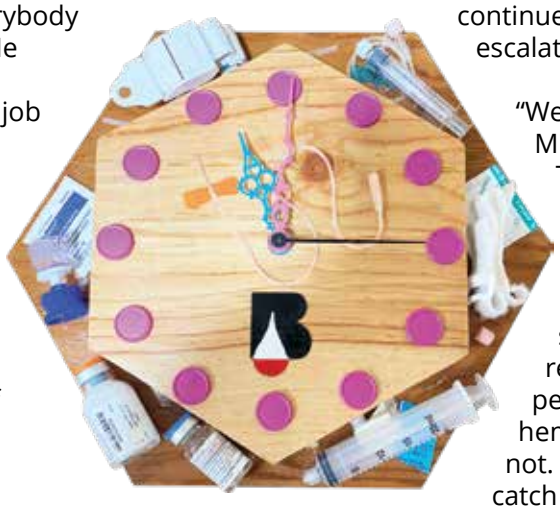
"When they came up with a factor, you had to be near a big city to get it. The factor was so expensive and hard to get that you tended to not do it if it was a minor bleed or something you could live with. You saved the treatment for the extreme bleeds," Dave recalls. "In the 1970s when you could keep it in your refrigerator, I still didn't use it like I should, because it was expensive." On top of that, "I had to pay my own insurance. It was half of my income!"

Dave has never taken factor prophylactically and has only used it for more serious bleeds. Asked if that has led to permanent damage, he says, "Oh yes! My problem is pain caused by bleeds forty years ago that left damage. Even though I know better, I still look at it as something you take when you need it. I'm not being smart about it. It's entirely an old grumpy guy's idea," he says, sheepishly. "I really should take the stuff!"

It can be tough to change your mindset if you lived through prohibitively expensive factor and the blood scandal. Dave remembers, "In 1953, I had been in the hospital forever, and social workers came to me and said, 'You've got to understand; you're not going to live a normal lifetime.' They told me I'd never walk again. They said most hemophiliacs make it to eighteen or twenty before something happens to them, so that was my frame of mind. Then we got factor, so I thought, well gee, maybe I'll make it a little longer!"

"But around 1980 with the blood scare, my HTC said, 'We just assume anybody with hemophilia has HIV AIDS. So sorry, but you're probably going to die of AIDS. Well, a couple of years later, they develop tests for it, and they said, 'You don't have HIV, but you do have hepatitis C. Sorry, there's no cure. The incubation period is around 18 years, and so within 20 years, you're going to have problems.'

So, here I am, 45 years old and everybody keeps telling me I'm going to die!" He laughs for a moment. "It caused problems on the job, too. I lost one job simply because the other workers didn't want to be around me. We shared a drinking fountain and a lunch table, and they thought that was a problem."



Dave didn't begrudge them. "I had a friend who had polio. Lying in the hospital, I knew people who died of diabetes. I just felt everybody's got something. That's the breaks."

"Something I fight yet today is the lack of education about hemophilia." He relates how he has especially seen this outside of metropolitan areas. "This can affect you in emergency situations. If you're in a car accident in the middle of Mississippi or somewhere, emergency rooms don't know what to do for hemophilia. They don't have factor available, or they don't know which factor to use."

"The most life-threatening situation I ever had was a routine visit to the dentist. He proceeded to give me a numbing shot into the jaw area and hemorrhaging occurred. "I couldn't breathe. Another time, the "best" hematologist that we have here was giving my brother one factor product at the dosage of a different product. He wasn't administering enough."

"I had my hip replaced here in Iowa. I got a good hip replacement, but did I have hemophilia problems! They cannot and will not take care of it here." In fact, the plan for his hip replacement was to fill up on factor immediately before the surgery, and then twenty-four hours later, they were going to give me a full 100% dose again." However, the needle infiltrated, and the second dose went into the tissue, not the bloodstream. "They wouldn't give me more factor. They said, 'It's under your skin, so you got it.' Luckily, the only bleeding he experienced was at the site of the bad injection. "My arm was bigger than my leg, but it could've been really serious if my hip had bled."

Recently, his doctor wanted Dave to have a procedure, but Dave brought up guidance from three HTCs that said the procedure was not recommended for hemophiliacs. The doctor wouldn't listen to the research. Dave continued to advocate for himself, which escalated to an argument.

"Well, why don't you just move to Minneapolis?" the doctor suggested. The medical office was located in a small town outside of the Des Moines area. Dave says, "I'm sure there's someone around that knows all about hemophilia but sometimes finding that person is really tough. There are a bunch of people who claim to know all about hemophilia when, in fact, they do not. They want to know, 'When did you catch it?"

Dave would like to share his experiences with young people with hemophilia. He urges them to participate and support the groups such as the Coalition. "They're going to learn a lot and get a lot of help. The landscape of hemophilia is very different today than it was when I was a boy, but there is a lot to be learned about the history of bleeding disorders."

"I don't know that I've run across a youngster with hemophilia today who isn't on prophylactic treatment. They were born with factor available to them. They would never make the silly decisions I have! Today, it's covered by insurance companies and Medicaid. The younger generation couldn't possibly understand how life was before factor."

Dave still lives in Iowa outside of Des Moines with his wife where they enjoy their children, grandchildren, and dog. He volunteers as an Ombudsman for long-term care residents at a nursing home. He still works on his old cars. Just like him, they don't go too far from home these days, but they still work, they look nice and they're all original!

# What I Wish I Had Known

**BY PAM WILLIAMS**

December 1997 forever changed my family's life. That was when hemophilia B entered our lives. My oldest son, Joshua, was 11, had just undergone ear reconstruction surgery at Cincinnati Children's Hospital. As we were leaving the ENT's office for his post-operative check, his ear began to bleed profusely. Lucky for us, his ENT often worked closely with the hematology department and knew exactly which lab tests to order. The next day, we were contacted by the hospital to return, where my son was admitted for additional testing and treatment. I remember telling my husband at the time, "You better not lose your job - we're going to need really good healthcare." Thinking back on that conversation, it probably wasn't my best moment or the best reaction to have.

Back in 1991, Joshua began waking up every morning covered in bruises. Having had a young cousin who died of leukemia, I knew one of the symptoms was unexplained bruising. I instantly took Joshua to his pediatrician at Dartmouth Medical Center. A pediatric resident saw him and ordered bloodwork, but it was obvious he thought the unexplained bruising could only mean one thing – child abuse.

As we stood in the hospital hallway, Joshua's pediatrician came walking towards us, "What's up, Joshua?" Joshua replied, "I have bruises. I haven't been playing King of the Mountain at recess, I promise."

The doctor immediately began checking out the bruises, then hurried him back to an examination room where she found the resident getting ready to write "child abuse" in Joshua's chart. She grabbed the pen from his hand and informed him there was no way Joshua was being abused, and that she had known him since he was born. She reviewed the bloodwork list, added more tests, and sent us home to wait for the results. Several days later, she called to say that Joshua did not have leukemia. That he has a type of blood disorder, but it was nothing to worry about. We didn't question anything and for the next six years, while there were many signs that pointed to something significant, we just chalked it up to life with a  boy and moved on.

As a female, I had abnormal menstrual cycles, but no one questioned them. The typical response to my concerns was, "It's normal, every woman has heavy bleeding and long cycles, you're fine." Since it wasn't known at the time that my dad had hemophilia B, I was none the wiser. Even after a miscarriage and giving birth to Joshua and his younger brother, Thomas, no one was concerned about the amount of blood accompanying those births. Even when I mentioned to my OB/GYN that I bled continually for 6-8 weeks following childbirth, sometimes passing clots the size of quarters, she brushed me off as a new mom who hadn't taken time to recuperate before returning to a full-time job.

Back to 1997, Thomas, as well as my dad, were diagnosed with mild factor IX deficient hemophilia. My dad was 62 years old, had been raised on a farm and spent time in the Army during the Korean War. He never knew; however, his siblings had been told by their father that, "Fred is a bleeder." No one knew what that meant exactly, which meant that Dad had never been

treated for hemophilia B and thus, had no idea having a daughter would mean the disorder would be passed on genetically and the circle would continue.

So, what do I wish I had known? If I had known I had hemophilia B or at least, known I was an obligate carrier, I would not have married the man I did. I would have been able to tell those I dated that I had a bleeding disorder and what it would mean for our future lives and childbearing. They would have known from the beginning giving birth to sons would mean a 50-50 chance the son would have hemophilia B. When my now ex-husband found out Joshua and Thomas had a bleeding disorder, he said, "This is all your fault. My sons will never be 'real boys,' you know, the kind that play football or join the military." It was at that moment I realized had been aware of my genetic history, he would not have been my choice for a spouse. It took me three years to leave the marriage.

Hemophilia B has brought so many wonderful people into the lives of my boys and my life. I met so many families who have approached hemophilia together, no one blaming anyone, and couples staying together.


Sure, some families split, but most often for reasons unrelated to hemophilia. While our marriage dissolved for many reasons, I do feel the diagnosis of hemophilia played a part in the demise of our union. I noticed most hemophilia families stuck it out. They do what they need for insurance and even pursue employment based on the needs of their child/children.

Both of my sons entered their relationships upfront and honest with their significant others regarding their bleeding disorder. They both entered marriage knowing that having a daughter would mean she was an obligate carrier and as we now know from our advocacy and education, would possibly be diagnosed as a female with mild hemophilia B.

I would have entered marriage knowing my mate was fully aware what having children would mean for us... specifically, what having boys could potentially mean. I would have been able to determine in advance how the potential mate would respond to the possible genetic complications and whether he would have what it takes to go the long haul knowing the treatment and costs. I would have made different choices. I would have put all the cards on the table and watched his expression when I explained everything. I would have made sure my mate understood what a bleeding disorder meant for my health as well. We would have entered a relationship and marriage with eyes wide open and been more prepared for our future.

Over the past 26 years, I have learned a great deal about bleeding disorders and hemophilia B. I've learned that being a female with hemophilia is a long road to a diagnosis and treatment. Sometimes, it takes trial and error to find the hematologist who will listen to you, order the proper testing, and agree to treat with the necessary factor replacements. As women, we still have a long road ahead of us to reach equality in these areas. With other hemophilia women, we will continue to advocate and push for diagnosis and treatment until we are taken as seriously as our male counterparts. It is an ongoing mission and trek.

Shoulda. Woulda. Coulda. What I wish I had known.

# MEN'S EDUCATION AND EMPOWERMENT FALL RETREAT

BY RYAN CROWE AND ROCKY WILLIAMS

**We held our annual Men's Retreat in Lake Las Vegas from September 7th to 10th, and it was a phenomenal success! It was an engaging and dynamic event, featuring activities and informative sessions that emphasized advocacy and team building, as well as physical and mental health. Aqua therapy, tai chi, and diverse sessions covering topics like hemophilia treatment, stress management, and healthy eating underscored the retreat's commitment to our community's well-being.**

We had a slew of amazing speakers share their expertise. Dr. Rahasson Ager led a session on *Understanding Novel Therapeutic Approaches for Hemophilia B: Decoding the Science*, shedding light on critical medical advancements. Fernando Reyes, MEd Psy, guided attendees through a session on stress management, equipping them with valuable coping strategies. And Dr. Robert Friedman led a session on *A Path to Inner Strength*, inspiring personal growth and resilience among participants.

While we learned so much together, this event provided a safe space for men in the hemophilia B community to speak openly, fostering unity, camaraderie, and team-building. We held several rap sessions where we shared our stories


and became even closer together. Men with hemophilia B shared their experiences with dads and spouses, and older community members shared with the younger people. It was a great opportunity to connect and learn from our speakers as well as each other.

In addition to the top-notch speakers, we also shared a good amount of laughter. We continued our tradition of hosting our annual Bleeder Olympics, where we challenged each other in fun outdoor recreation games. And we added in some absolutely hilarious team-building activities that had us all rolling. One example was *Dude, Dude, Dude*. In this game, we learned a thing or two about communicating while using the same words. We even brought back an oldie but goodie called *Poetry in Action*. Just imagine our guys reading poetry to each other. It was certainly a sight to behold!

The event reached its grand finale with a thrilling *Casino Night* featuring a strolling magician and a special guest appearance by Elvis, adding a touch of magic to the evening. Yes, Elvis was in the building. It was the perfect capstone to a fabulous weekend together. "Viva, Las Vegas!"

We extend our heartfelt thanks to Pfizer for generously sponsoring this transformative weekend.

## Comments:

"The men's retreat, for me, is an opportunity to reinvigorate myself - to allow myself to renew friendships with old acquaintances and introduce myself to new members of the community. We all have stories to share, and I'm always amazed at the strength and happiness in so many supposedly broken bodies. Their bodies may be less functional, but their spirit is so strong! It's a healing time for me."

"The men's retreat weekend was a perfect blend of education, camaraderie, relaxation, and excitement. We learned about healthy living and mental wellness while having a blast. It was a weekend of growth and connection with our fellow brothers."

"I always enjoy the men's retreats because we can all get together and compare notes. I have learned a lot from the older gentlemen about how to take care of myself and how to overcome tough times."

"I am so grateful to be able to attend the men's retreat. It gives us an opportunity to spend time with our brothers, have fun, and, oh, actually learn new stuff!"

"The men's retreat is an awesome experience; there's no better way to connect with others than in a close and safe community. I never stop learning from the unlimited wisdom of everyone there. As a father without hemophilia, I gain perspective too, and hearing other people's stories is like turning on the lights to prepare for the road ahead."

"The Coalition for Hemophilia B Men's Retreat is a truly unique experience, where the shared journey of our condition creates an unbreakable bond. It's a feeling like no other, coming together with fellow warriors, not just as friends but as family. The camaraderie we share at this retreat is what makes it an event I eagerly anticipate each year."

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

EXPERIENCE 25 YEARS* MATTERS

# BeneFix®

**Coagulation Factor IX (Recombinant)**

Room Temperature Storage

*BeneFix was approved February 11, 1997.

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## BeneFix has been supporting individuals with hemophilia B for 25 years—and our support continues

**ONCE-WEEKLY** PROPHYLAXIS AND **ON DEMAND** WITH INDIVIDUALIZED DOSING

**25 YEARS** OF CLINICAL EXPERIENCE

**20 TRIALS**, INCLUDING MORE THAN **1500 PATIENTS**

**>90% OF COMMERCIALLY INSURED PATIENTS** HAVE ACCESS TO BeneFix

### 1997

BeneFix becomes **the first** recombinant factor IX (rFIX) treatment for hemophilia B approved by the US Food and Drug Administration (FDA).

### 2020

The FDA approves **BeneFix once-weekly** prophylactic use in addition to its on-demand indication.

### 2022

On February 11, BeneFix proudly became the only rFIX **supporting individuals with hemophilia B for 25 years**.

### What Is BeneFix?

BeneFix, Coagulation Factor IX (Recombinant), is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Your doctor might also give you BeneFix before surgical procedures.

BeneFix is **NOT** used to treat hemophilia A.

### Important Safety Information

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash, or hives.
- Your body can make antibodies, called "inhibitors," which may stop BeneFix from working properly.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness, and rash.

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

**Please see Brief Summary of full Prescribing Information on next page.**

**To learn more about once-weekly dosing with BeneFix,**
visit benefix.com/about-benefix/once-weekly-prophylaxis.

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Pfizer

BeneFix®

**Coagulation Factor IX (Recombinant)**

Room Temperature Storage

℞ only

**Brief Summary**

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-438-1985.

Please read this Patient Information carefully before using BeneFix and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

**What is BeneFix?**

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Your doctor might also give you BeneFix before surgical procedures.

BeneFix is **NOT** used to treat hemophilia A.

**What should I tell my doctor before using BeneFix?**

Tell your doctor and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal medicines.

Tell your doctor about all of your medical conditions, including if you:

- have any allergies, including allergies to hamsters.
- are pregnant or planning to become pregnant. It is not known if BeneFix may harm your unborn baby.
- are breastfeeding. It is not known if BeneFix passes into the milk and if it can harm your baby.

**How should I infuse BeneFix?**

The initial administrations of BeneFix should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

**See the step-by-step instructions for infusing in the complete patient labeling.**

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

**Call your doctor right away if bleeding is not controlled after using BeneFix.**

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

**What if I take too much BeneFix?**

Call your doctor if you take too much BeneFix.

**What are the possible side effects of BeneFix?**

Allergic reactions may occur with BeneFix. Call your doctor or get emergency treatment right away if you have any of the following symptoms:

| | |
|---|---|
| wheezing | fast heartbeat |
| difficulty breathing | swelling of the face |
| chest tightness | faintness |
| turning blue (look at lips and gums) | rash |
| | hives |

Your body can also make antibodies, called "inhibitors," against BeneFix, which may stop BeneFix from working properly.

Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFix may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFix is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

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

**How should I store BeneFix?**

DO NOT FREEZE the BeneFix kit. The BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Throw away any unused BeneFix and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

BeneFix does not contain a preservative. After reconstituting BeneFix, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFix if the reconstituted solution is not clear and colorless.

**What else should I know about BeneFix?**

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

If you would like more information, talk with your doctor. You can ask your doctor or pharmacist for information about BeneFix that was written for healthcare professionals.

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-14.0, revised September 2021.





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# WOMEN'S EDUCATION AND EMPOWERMENT FALL RETREAT

BY ERICA GARBER

**The 2023 Women's Education and Empowerment Fall Retreat, September 14th-17th, at the Hilton Phoenix Resort at the Peak in Arizona was more than just a weekend of learning—it was a beautiful experience of empowerment and shared connections, leaving us not just enlightened, but deeply connected and enriched. Our journey started with warm embraces and joyous greetings, weaving the fabric of old and new friendships—a perfect introduction to the weekend ahead.**

Laughter filled the air during the *Queens of Cards Challenge*, a kickoff event where we fused a nostalgic card game with team-building fun. It was a pleasure to see the resulting camaraderie become the heartbeat of the weekend, fostering the spark of connection and joy. Our opening day's round-table discussions carried this spirit forward. Stormy Johnson, a fervent advocate for women with bleeding disorders, led the *Women Bleeders Round-table*, sharing her personal journey with an open heart, creating an environment where stories flowed freely. It wasn't just a discussion; it was a heartfelt conversation exploring misdiagnosis to advocacy, leaving the women in the circle feeling heard and understood.

Simultaneously, Sherry Upton, a dedicated caregiver and co-founder of Common Ground Ministries, and Tiffany Pokrajac, RN, a nurse and passionate advocate for families with bleeding disorders, crafted the *Caregivers' Round Table* into a haven for our unsung heroes, offering a platform for shared insights and tools to enhance the well-being of both caregivers and their loved ones. Co-leading the evening *Rap Session* with Farrah Muratovic, we created a poignant storytelling space, ending the day by learning so much about each other and fostering connections.

After a day of travel and emotional expression, it was only fitting to create space for physical health and mental preparation. Lynn Yaeger, a mother and advocate residing in Cincinnati, OH, led two mornings of nature walks, and Erin DuPree, NASM CPT, FNS, a











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certified personal trainer, fitness professional, and sports nutrition coach, orchestrated some energetic water aerobics classes. These morning exercises served as holistic experiences, aligning the mind and body for the content that awaited.

Patty Eastin, a Patient Affairs Liaison with Pfizer Rare Disease, guided a session on *Tools for Self-Advocacy* that became more than practical resources; they were beacons of empowerment, providing tangible tools to navigate the unique challenges faced by those with hemophilia. Rahasson Ager, PhD, a Field Medical Director with Pfizer Rare Hematology, unraveled the complexities of novel therapeutic approaches for hemophilia B. In this enlightening session, we not only gained a deeper understanding of the evolving treatment landscape but left with a profound sense of empowerment rooted in knowledge. These sessions weren't just about learning; they became catalysts for empowerment, leaving participants with a positive outlook and new information to consider on the road ahead.

Cheyenne Autumn, an education and wellness expert, transformed her *Love and Healthy Living* session into an exploration of the intricate tapestry of love. It wasn't just about self-love; it was an immersive journey into love for others and passion for life. As we delved into the essence of love, we discovered not only a renewed sense of self but a profound connection to the collective heartbeat of the retreat. The evening's *Chit-Chat & Chocolate*, hosted by Heidi Hart, a founding board member of the Rocky Mountain Hemophilia and Bleeding Disorders Association, and Thea Russo, deeply involved in the hemophilia B community, became a moving storytelling experience. Laughter, tears, and the shared comfort of chocolate created an emotional tapestry that


strengthened the bonds forged over the weekend.

Gha'il Rhodes Benjamin, an award-winning, Grammy-nominated spoken-word performance-recording artist, turned her session called *Expressing the Goddess Within* into a collective affirmation of self-expression. We didn't merely witness spoken word; we became active parts of a celebration of individuality and empowerment. Natalie Sayer, the founder of The Blair David Company and an executive and team coach, equipped us with practical tools to navigate internal dialogues, fostering not just a sense of control but an active rewriting of personal narratives. The room resonated with newfound self-awareness, a chorus of mental shackles breaking free.

Vanessa Vitali, a Holistic Nutritionist, Health Coach, and Chronic Condition Educator, explored the *Law of Attraction* in a transformative experience. As we delved into the power of positive thinking to shape not just our weekend but our broader realities, the atmosphere buzzed with a collective understanding of the profound impact of thoughts on the journey ahead. Closing the educational sessions, Aura H. Bermúdez, a licensed psychologist, nationally certified Ayurvedic Health Counselor, and Yoga Practitioner, invited us on a journey of self-discovery and well-being through *Ayurveda for a Balanced Healthy Life*. The session unfolded not just as an exploration of ancient practices but as a road map for personal growth and holistic well-being.

The retreat's final night event was an acknowledgment of shared accomplishments and a testament to the enduring bonds forged. It became a magical evening where we unwound, connected, and reveled in the enchanting atmosphere with friends.

As Sunday dawned, a farewell breakfast turned into a reflective moment. Gratitude filled the air as we expressed appreciation for the connections made and the knowledge gained. A heartfelt thank you echoed through the retreat, underscoring the generosity of Pfizer, our sponsor whose support was pivotal in creating this transformative experience.

The *2023 Women's Education and Empowerment Fall Retreat* was a celebration of empowerment, resilience, and the indomitable power of community, further amplified by the expertise and passion of our esteemed speakers. In each shared moment, a collective heartbeat echoed—a testament to the transformative power of knowledge, connection, and self-discovery.

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

# WE'RE IN THIS TOGETHER PARTNERS' RETREAT

**BY CARL AND GWYN WEIXLER**

**This fall, The Coalition for Hemophilia B held its 2nd annual "*We're in This Together*" *Partners' Retreat* in Nashville, TN from September 28th - October 1st. It was well attended by 16 couples ranging from engagement to 40 years of marriage and coming from all over the US and Puerto Rico. We enjoyed chair massages to make our travel stress disappear.**

We began our days with yoga and stretching to wake our bodies up, then enjoyed many sessions to grow us as couples. First, we explored how our individual comfort zones affect how we tend to respond to our partner's needs with Kim Kilcauski. We all have different levels of comfort, as we learned when we thought about a variety of different scenarios and decided where our comfort zone was.

We found that much of the time, our partner was in a different zone. The levels were: *Our comfort zone where that feels safe and good*; *a bit challenging from our comfort zone but I think I could handle that*; and *way stressful and I really wouldn't want to do that!* In the next session, we enjoyed a roundtable rap session, grouped by patients and caregivers. It was an opportunity to share our stresses, our strategies, and our successes! We had a fun and relaxing time after dinner talking! All of this was just our first evening!

The next topic was covered over the course of two days. We had a return visit with Natalie Sayer, MSMSE , ACC as we explored and expanded our understanding of the *Core Values Index* assessment. We had a session on this on our first retreat, but it was so helpful that even those of us who experienced it last year, found it very helpful and pertinent to our relationships, not only as


















couples, but as family members, work associates, and friends!

On the second day, we continued with Gena Kay Shealy. We discovered practices that nurtured our well-being while strengthening our bond with our partner. We learned to recognize our default stress responses and how to manage them. We explored ways to create a thriving relationship that prioritizes both our individual and collective wellness.

We ended the day with Coriss Bragg. In this workshop we learned how to connect to our own body and energy. We learned about tools that slow down together and cultivate presence, as well as what the body needs to truly open up and receive physical and energetic connection.

Presenters, Karen Boyd, LMSW, ACSW, DCSW, and David Rushlow, LMSW, returned from last year's retreat to facilitate a very interactive session exploring intimacy and communication that allows both partners to "win" as we wrestle with difficult aspects of our relationships.

Our final session was led by Matthew Barkdull MS, MBA, LMFT, MedFTas we discovered the pitfalls of perfectionism in terms of our stress levels and expectations in relationships and individual pursuits. It affects our performance, happiness, and success in embracing life's experiences. In other words, we need to embrace imperfection!

We ate very well with the hotel food services and enjoyed time getting to know each other informally. We ended the wonderful weekend with a fun night of line dancing and country music! We are thankful to the Coalition for recognizing that couples carry a great responsibility in managing hemophilia in our daily lives and appreciate the opportunity to grow as partners and feel rejuvenated by time away!

Many thanks to our generous sponsor, CSL Behring.

CSL Behring

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

# HEMOPHILIA LANDSCAPE UPDATES

**BY DR. DAVID CLARK**

**Fall 2023**
Several of the updates below are from the annual meeting of the American Society of Hematology (ASH) on December 9–12, 2023 in San Diego. Copies of the abstracts (summaries) can be obtained for free on the meeting website, https://www.hematology.org/meetings/annual-meeting.

## X-CHROMOSOME INACTIVATION AND BLEEDING IN CARRIERS OF HEMOPHILIA B

12/11/23 Women have two X-chromosomes, unlike men who have an X and a Y. The factor IX gene is on the lower right arm of the X-chromosome, the one that is missing on the Y-chromosome. Therefore, women have two copies of the factor IX gene in each cell, while men only have a single copy.

In order to prevent hazardous interactions between the two X-chromosomes in women, one copy of the X-chromosome in each cell is inactivated. X-chromosome inactivation (XCI) is thought to be a random process in each cell, so a woman who has one X-chromosome with a mutated factor IX gene and one X-chromosome with a good factor IX gene should end up with about 50% of her cells containing the X with the mutation and 50% with the good X without the mutation. (This is like flipping a coin. If you do it enough times, you should come out with about a 50-50 ratio of heads and tails.)

Therefore, the average carrier should have about 50% of normal factor IX activity: half of her liver cells are producing "good" factor IX and half of her liver cells are producing mutated factor IX. However, we know that the XCI process can be skewed – that instead of a 50-50 split, it can vary to as much as an 80-20 or worse split. If the 80% of her liver cells are producing "good" factor IX, a woman's factor IX level would be expected to be about 80% of normal and she should not have hemophilia.

On the other hand, with an 80-20 split, if the 80% are the cells producing the mutated factor IX, she would only have a factor level of about 20% and she would have hemophilia. In either case, she would still be a carrier, because she could still pass on the mutated gene to her children.

Currently the best theory about why some carriers have hemophilia is that they have a skewed XCI, although we don't know why that happens. That may be about to change. At ASH, a group from Penn State Hershey Medical Center presented data suggesting that skewed XCI might not be the reason after all, or at least not the whole reason. They found in 15 subjects that there was no correlation between the degree of XCI skewing and factor levels. This finding adds to a growing list of studies of XCI and hemophilia, some of which show no correlation with XCI skewing and some of which do show a correlation. This simply tells us that we don't know enough.

The ASH authors recognize this and propose that the answer might be much more complex, especially for hemophilia B. For one thing, now that we know that the factor IX bound inside the blood vessel walls is important for clotting. Since a carrier has both "good" factor IX and mutated factor IX in her bloodstream, one might be out-competing the other for the binding spots in the vessel wall, and that could affect her bleeding tendencies.

Now that we're recognizing that women do get hemophilia, there is bound to be more research to find out why. That research could also help males with hemophilia, since there is still a lot we don't know there. [Cygan PH et al., ASH abstract 3990]

## WOMEN GET INHIBITORS, TOO!

1/14/20 While researching the above piece on women with hemophilia, I came across an article in my files that could be important for some. On 1/14/20, Shellye Horowitz wrote an article in *Hemophilia News Today* that pointed out that women with hemophilia can get inhibitors, too. An inhibitor is an antibody that blocks the action of infused clotting factor. Men are now recommended to get inhibitor testing at least once a year, and women should be tested, too.

The CDC now recommends that anyone with hemophilia or with von Willebrand Disease type 3 be tested annually for inhibitors. One of the early signs of an inhibitor is bleeding that doesn't stop, even after a substantial amount of clotting factor has been used.

[Horowitz S, Hemophilia News Today article 1/14/20]

## THE CARRIERS ULTRASOUND PROJECT

12/9/23 Not much is known about joint damage in women with hemophilia. The *Carriers Ultrasound Project* (CUT) wants to change that. CUT recruited 28 carriers of hemophilia (24 As; 4 Bs) and 30 controls, women who had no family or personal history of hemophilia. The carriers showed a higher prevalence of bleeding and more joint-related symptoms, including pain, than the controls. Curiously, the carriers showed their symptoms on the *Hemophilia Joint Health Score* (HJHS) questionnaire but showed no difference from the controls on the *Hemophilia Early Arthropathy Detection with Ultrasound* (HEAD-US) survey. HEAD-US is designed to detect early changes in joint health in men with hemophilia but may fall short for women.

A high body mass index (BMI) was also correlated with increased joint bleeding among the carriers. Joint damage is apparently common among carriers, even those without hemophilia. More research is needed. [Kronenfeld RS et al., ASH abstract 29]

## FACTOR LEVELS AND BLEEDING RISK IN HEMOPHILIA PATIENTS PLAYING SPORTS

5/24/23 A group of researchers in The Netherlands looked at bleeding risk as a function of factor level for hemophilia patients playing sports. In 125 subjects aged 6–49 (90% As, 10% Bs, 48% severe), they found that sports injuries were rare. Only 26 sports-related injuries were recorded in 15,999 sports exposures (0.16%). Most of the subject's injuries and bleeding episodes were not sustained during sports activities.

There was no correlation between sports-associated bleeding and hemophilia severity, joint health, sports risk category or sport intensity. The only correlation was with factor levels at the time of injury. They found that people with factor levels below 10% had more than twice the bleeding risk as people with factor levels above 10%. This shows the importance of keeping your factor levels high when playing sports. [Versloot O et al., Haemophilia, online ahead of print 5/24/23]

## NO DIFFERENCE IN QUALITY OF LIFE BETWEEN PEOPLE WITH SEVERE HEMOPHILIA A AND B

2/15/23 We periodically see articles looking at differences in severity between people with hemophilia A and B. Some see this as a "competition," but the real value is in trying to find differences between the two hemophilias that might provide clues to better understanding of both diseases.

A large group in Scandinavia looked at health-related quality of life (HRQoL) using several questionnaires in 63 people with severe hemophilia B compared with 63 people with severe A. The As in the control group were matched by age, gender and treatment modality (on-demand or prophylaxis) to the Bs. No inhibitor patients were included.

Mobility problems were reported by 46% of Bs and 44% of As. Pain or discomfort was reported by 62% of Bs and 56% of As. Strikingly, anxiety or depression was reported by 33% of Bs and only 17% of As. Looking at the questionnaire responses overall, there was no significant difference in HRQoL between the As and Bs. The largest negative impact was from poor joint health in both groups.

The real takeaway from the study is that both As and Bs had impaired HRQOL, even though most subjects were on prophylaxis. There is still a need for improved treatments for hemophilia. [Kihlberg K et al., Haemophilia, online ahead of print 2/15/23]

## IS MILD REALLY MILD? IS MODERATE REALLY MODERATE?

People with mild or moderate hemophilia B (including women) often don't receive the treatment they need, even in the U.S. Much research focusses on severe hemophilia, so we know relatively little about mild and moderate hemophilia. Several papers over the past year are trying to change that.

10/12/22 One study looked at the use of clotting factor in people with non-severe hemophilia by doing an analysis of past studies. They found some information for hemophilia A but only sparse information for non-severe patients with hemophilia B and for women with hemophilia. In general, they give a rationale that prophylaxis be started early in life in people with significant bleeding, regardless of their factor level. [Iorio A et al., Haemophilia, online ahead of print 10/12/22]

1/25/23 A group of researchers from Argentina looked at the "mildness" of mild hemophilia. They focused specifically on arthropathy (joint damage) in 85 subjects (19 Bs) and 510 joints (ankles, elbows and knees). The cohort's average age was 35.9 years, and they found that 90.5% of patients over 20 years old had arthropathy. Only 28% of patients had no joint damage and 72% had at least one joint with arthropathy. They found a significant difference in body mass index (BMI) in which the group with arthropathy had an average BMI of 28, while those without arthropathy had an average BMI 0f 20.3.

The median age at diagnosis was 3 years (range 1 to 7) for the no-arthropathy group and 10 (4 to 19) for the subjects with arthropathy. That shows the

importance of early diagnosis, especially if the child is from a family with a history of hemophilia. Another significant predictor of joint damage is a history of muscle hematomas. A history of muscle hematoma was present in 65.2% of those with arthropathy but only 20.8% of those without. [Daffunchio C et al., Haemophilia, online ahead of print 1/25/23]

5/10/23 At the WFH Comprehensive Care Summit in May in Buenos Aires, the same group of Argentinian researchers presented data on the physical and social impact of mild hemophilia. They report that the key determinant of quality of life for those with mild hemophilia is joint health. Studying the same 85 subjects/510 joints, they found that 61 patients had at least one joint with damage (defined as a HEAD-US score ≥1). The most affected joint was the ankle.

A total of 56 subjects had at least one hospitalization due to hemophilia with the most common reasons being an illiac psoas hematoma, musculoskeletal surgery and hemarthrosis (joint bleeding), in that order. Probably because they tend to go for so long without treatment, joint damage and quality of life were both associated with age. [Landro M et al., WFH Comprehensive Care Summit, abstract PP-TH-019. Abstracts in Haemophilia, 29(S2) 2023]

10/7/23 A group of researchers from The Netherlands looked at bleeding patterns in patients with moderate hemophilia who bleed like severes, i.e., those who have a severe bleeding phenotype even though their factor levels are ≥1% of normal. (Your phenotype is how you actually bleed. Your genotype predicts your factor level, which predicts how you are expected to bleed. They don't always agree.)

In 116 subjects, they found that 21% had a severe bleeding phenotype and 46% of those were on prophylaxis. Moderate patients with a severe bleeding phenotype treated on-demand had a higher median ABR of 7, compared to those on prophy who had a median ABR of 2. The on-demand patients also had lower qualities of life. [Verhagen MJA et al., J Thromb Haemost, online ahead of print 10/7/23]

12/11/23 Finally, at ASH a group of researchers from Spain updated a previous study on joint damage in non-severe hemophilia patients. They looked at the effects of age, baseline factor level and global hemostatic capacity (GHC) in 98 non-severe patients, including one moderate hemophilia B patient and four mild B patients. Even with so few Bs, the findings are probably applicable to most mild and moderate hemophilia patients, A or B.

About 56% of the moderate patients and 45% of the mild patients had joint damage. Target joints were observed in about 9% of the mild patients. There was no correlation between factor level or GHC and joint damage. Joint damage did increase with age, reinforcing earlier findings. Their conclusion is that more attention should be paid to joint damage in milds and moderates so it can be determined whether new protocols are needed for diagnosis, prevention and treatment. [Rico AM et al., ASH abstract 3986]

These studies have a common pattern. Many patients with mild and moderate hemophilia (including women) need as much medical attention and clotting factor treatment as patients with severe hemophilia. They definitely experience joint bleeds, but those often go unnoticed because of their low frequency. However, noticed or not, those bleeds contribute to joint damage and can lead to arthropathy and lower quality of life down the road.

## IMPROVED JOINT HEALTH WITH EXTENDED HALF-LIFE (EHL) PRODUCTS

5/10/23 Continuing on the subject of joint health, two papers at the WFH Comprehensive Care Summit looked at improved joint health after use of EHL products. In the past, when patients were on standard half-life (SHL) products and many patients were still using on-demand treatment, we never knew whether joints would heal if one could take enough factor IX. Back then, we were happy just to get severe patients' factor levels above 1% of normal.

Imagine the cost (not to mention the number of infusions) it would have taken to get them even above 5% and into the mild range. EHL products were originally developed to reduce the number of infusions, but serendipitously they also give patients higher trough levels and fewer joint bleeds.

Sanofi presented a theoretical analysis of historical data for their EHL product Alprolix. Using Pettersson scores to rate joint damage (zero is the best and 13 is the worst), they projected that a person starting prophylaxis with Alprolix at age 12 with no joint issues (a score of zero) would only have a score of 5.0 by age 70. In contrast, a patient using on-demand treatment from age 12 on would have a total score of 32.0 by age 70. (The scores add up, so a score of 32 could be four joints with scores of 8.0 each, or any other combination.) This shows the value of prophylactic treatment compared with on-demand care.

An interesting aspect of their model is that it shows that if an on-demand patient switches to prophy, even as late as age 51, their score at age 70 would be reduced to 18.4, a big difference. Switching earlier could lead to even lower joint damage scores by age 70. [Olive M et al., WFH Comprehensive Care Summit, abstract PP-WE-024. Abstracts in Haemophilia, 29(S2) 2023]

A group from Greece looked at 63 patients (17 Bs: 12 on-demand, 5 late prophylaxis) switching to prophylaxis

with EHL products. They monitored the patients for ABR and joint health before switching and every six months thereafter for up to four years. They found that the median ABR was reduced from 4.0 (range 2 – 10) in the group that had been on prophy to 2.0 (0 – 5) after switching to EHL products. The on-demand group who had a median ABR of 20 (18 – 35) before switching also ended up with an ABR of 2.0 afterward.

In this study, they used the Hemophilia Joint Health Score (HJHS) to rate joint damage and found an improvement over time. The median HJHS was reduced from 31.25 (17 – 58) to 21.3 (14 – 54) by year four. This shows that joints can actually improve with adequate treatment. [Christidi SD et al., WFH Comprehensive Care Summit, abstract PP-WE-040. Abstracts in Haemophilia, 29(S2) 2023]

## IMPORTANCE OF YKL-40 PROTEIN IN JOINT DAMAGE

12/11/23 We don't really know the cause of joint damage in hemophilia. We know that it is caused by blood in the capsule surrounding the joint, and it seems that iron from that blood is involved. But drilling down deeper, we don't really know the causes of the chemical changes taking place in the joint. Now a group from Greece has shown that a protein called YKL-40 is probably involved. YKL-40 is a known protein that is associated with joint diseases like rheumatoid arthritis and osteoarthritis. It is produced in arthritic joints by immune cells and chondrocytes (cells that produce cartilage).

They found that levels of YKL-40 in the bloodstream were significantly higher in hemophilia patients than in the control group without hemophilia. They also found that patients with arthropathy (joint damage) tended to have higher circulating levels of YKL-40 than either hemophilia patients without joint damage or hemophilia patients with active bleeds. Thus YKL-40 might be a cause of joint degradation, and even if it isn't, it appears to be a marker of joint degradation activity. Measuring YKL-40 in the blood might be a good alternate indicator of ongoing joint damage, even at otherwise undetectable levels, rather than imaging. [Michalopoulou A et al., ASH abstract 3996]

## SHOULD ADULT SEVERE PATIENTS BE ON PROPHYLAXIS?

5/10/23 At the WFH Comprehensive Care Summit, another group of Argentinian researchers presented an evaluation of real-world bleeding and factor use in adults with severe hemophilia. A total of 89 patients (74 As; 15 Bs; all male) over 21 years of age with severe hemophilia A or B were included in the study. The average age was 35.8 years (range 22 – 67) and all patients were on standard half-life (SHL) products.

The 46 on-demand patients have an average age of 38 and an average ABR of 24.5 bleeds/year. The prophy group has an average age of 43 and an average ABR of 2.5 bleeds/year. Those results suggest pretty convincingly that older severe patients would probably do better on prophylaxis. The only advantage that the on-demand group had is that they used about half the amount of factor. That might help pay their joint surgery bills.

We always point out that every patient is different. If you are older, severe and using on-demand treatment with few bleeds, you may be fine as you are. However, if you are having more than a couple bleeds per year, you might want to talk to your physician about switching to prophylaxis. It could significantly improve your joint health and your overall quality of life.

If you are concerned about the number of infusions, you could potentially try an extended half-life product. [Martinez M et al., WFH Comprehensive Care Summit, abstract PP-WE-007. Abstracts in Haemophilia, 29(S2) 2023]

## HETEROTOPIC OSSIFICATION IN HEMOPHILIA

12/9/23 Heterotopic ossification (HO) is growth of bone where it's not supposed to be growing, often in muscle tissue. This is a newly found complication of hemophilia, which has been discovered because of the increasing use of ultrasound in hemophilia care. It is emerging as an unforeseen complication of contusions (bruises) and injury-related muscle bleeding in hemophilia. A group of U.S. and international researchers looked at 29 cases from nine HTCs who represented hemophilia A and B in all severities.

The most common areas for HO were the thigh and hip, but also included the upper arm and hand. All cases were due to blunt trauma except for four in the psoas (large muscles in the hip) and one elbow. It happened mostly from deep muscle bleeds where the muscle is close to bone (24/29 cases: 83%).

Only three patients were on prophylaxis at the time of injury (one severe A, one severe B and one moderate B). HO was discovered on average at 23 days post-injury (range 11 to 60) with most cases discovered by a physical therapist (22/29 cases) or physician (2/29 cases). The increased detection of HO is primarily due to the increasing use of ultrasound imaging.

This study confirms that HO is a complication of deep bleeds, especially in patients not on prophylaxis. More study is needed. [Steiner BUK et al., ASH abstract 1248]

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etranacogene dezaparvovec-drlb

**FIRST AND ONLY FDA-APPROVED GENE THERAPY FOR HEMOPHILIA B**

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

*Patient portrayal; HEMGENIX not intended for women.*

## A ONE-TIME INFUSION DELIVERS GREATER BLEED PROTECTION*

**37%**
AVERAGE FACTOR IX ACTIVITY
**SUSTAINED AT 2 YEARS**

**GREATER BLEED PROTECTION**
VS. ROUTINE FACTOR IX PROPHY*

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

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

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

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## IMPORTANT SAFETY INFORMATION

### What is HEMGENIX?

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

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

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

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

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

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

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

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

### What should I watch for during infusion with HEMGENIX?

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

### What should I avoid after receiving HEMGENIX?

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

**Please see full prescribing information for HEMGENIX.**

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

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

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# HEMOPHILIA LANDSCAPE EMERGING THERAPIES

**BY DR. DAVID CLARK**

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

**Many of the updates below are from the annual meeting of the American Society of Hematology (ASH) on December 9 – 12, 2023 in San Diego. Copies of the abstracts (summaries) can be obtained for free on the meeting website, https://www.hematology.org/meetings/annual-meeting.**

## IMPROVED FACTOR PRODUCTS

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

### STANDARD HALF-LIFE (SHL) VS. EXTENDED HALF-LIFE (EHL) FACTOR PRODUCTS

8/11/23 Two recent studies have looked at treatment outcomes in patients on EHL factor products. A group from treatment centers in Australia examined the data for 174 hemophilia patients, 115 with hemophilia A (HA) and 59 with hemophilia B (HB), from the Australia Bleeding Disorders Registry. All patients had been on EHL products for the entire year of 2019. Adherence to treatment was 87.2% in the HB patients (85.7% for HA) and 64.4% of the HB patients had no spontaneous bleeds for the year.

About one-third of the patients had their doses adjusted during the year with the main reasons being their response to the products, body-weight changes and breakthrough bleeds. The data also showed a significant impact of non-adherence (not taking the product as prescribed). [George C et al., Haemophilia, 29(5) 1283, 2023]

12/10/23 Another study of EHL vs. SHL products was performed by researchers from Pfizer using the data in the *Adelphi Hemophilia Disease-Specific Programme*, a survey of HA and HB patients in parts of Europe and in the U.S. In the 81 HB patients, 33 (42%) were on SHL products and 47 (58%) were on EHL products. They represented a range of severities from mild to severe.

The average annualized bleeding rates (ABRs) were 1.4 for those on SHL products and 0.9 for EHL products, not a statistically significant difference.

The adherence rate for those on SHL products was only 12%, while that for those on EHL products was 51%. This demonstrates one of the theoretical advantages of EHL products, that fewer infusions should result in better adherence. However, note that even on EHL products, half of the patients weren't adherent. The study also showed that non-adherence led to higher ABRs, especially on SHL products. The average ABR for non-adherent patients on SHL products was 1.5 compared to an ABR of 0.5 for those with 100% adherence. There was a much smaller difference for those on EHL products, 1.0 for the non-adherent patients and 0.9 for the 100% adherent patients. Factor levels tend to fall much more slowly with EHL products than with SHL products.

Thus, both studies show that there are advantages to being on an EHL product. Both also show the importance of adherence. One take-away may be that if you tend to be non-adherent, you might be better off with an EHL product. [Thakkar S et al., ASH abstract 2616]

Despite the studies, one of the most important principles in medicine is that every patient is different. One of the advantages of having a number of different products available is that most patients will be able to find a product that works well for them. These are not interchangeable, commodity products. Some patients even do best on SHL product which includes Plasma. The choice of product should be left to the patient and their physician to find the product that has the best clinical benefit for the patient.

### EXTENDED HALF-LIFE FACTOR IX FUSED TO FACTOR XIII SUBUNIT

9/14/23 A group of U.S. and European researchers are developing an extended half-life factor IX, rFIX-LXa-FXIIIB, in which a factor IX molecule is attached to the B-subunit of factor XIII. Factor XIII forms crosslinks between fibrin fibers to strengthen a new clot. It is also the clotting factor with the longest natural half-life in circulation. The group has shown in rats and mice that rFIX-LXa-FXIIIB forms clots that are indistinguishable from those formed by normal factor IX. [Desage S et al., Haemophilia, online ahead of print 9/14/23]

### NOVO'S REBINYN PERFORMS WELL IN SURGERY

9/21/23 Novo Nordisk markets Rebinyn, an extended half-life factor IX that uses polyethylene glycol to stabilize the molecule in circulation. There is limited real-world experience on its use during surgery, so the group identified 31 actual surgical cases in which the patients were covered with Rebinyn. In the majority of procedures (27/31; 87%) hemostasis was judged to be "very good" or "excellent." There were no cases of thrombosis and no reported surgical complications related to the use of Rebinyn. [Phua CW et al., Res Pract Thromb Haemost, online ahead of print 9/21/23]

## REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so the blood clots when it should and doesn't clot when it shouldn't. The clotting system is a complex system of clotting factors that promote clotting and anticoagulants that inhibit clotting. In a person without a bleeding disorder, the system is in balance, so it produces clots as needed. In hemophilia, with the loss of some clotting factor activity, the system is unbalanced; there is too much anticoagulant activity keeping the blood from clotting. Rebalancing agents mainly reduce or inhibit the activity of 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 REPORTS NEW DATA FROM PHASE II STUDY OF SERPINPC

12/10/23 Centessa Pharmaceuticals is developing SerpinPC, a rebalancing agent that inhibits the anticoagulant activated protein C (APC) to restore clotting. SerpinPC is a once every two weeks subcutaneous treatment for hemophilia A and B patients, with or without inhibitors. At ASH, Centessa presented data from subjects in their Phase II clinical study who have been on the product for about three years. In 20 patients, the median ABR fell from 35.6 prior to treatment, down to 1.0 with SerpinPC. There were no adverse events or thromboembolic episodes. The researchers also measured levels of D-dimer, which is a sensitive indicator of thrombosis and found no issues. [Baglin T et al., ASH abstract 2619]

### PFIZER'S BLA AND MAA FOR MARSTACIMAB ACCEPTED BY FDA AND EMA

12/11/23 Pfizer is developing marstacimab, a rebalancing agent that inhibits tissue factor pathway inhibitor to restore clotting. Marstacimab is a once-weekly subcutaneous treatment delivered via an auto-injector pen for treatment of hemophilia A and B patients, with or without inhibitors. On 12/11/23, Pfizer announced that FDA has accepted their Biologics License Application (BLA, the application for a product license) for review. Their European Marketing Authorization Application (MAA) was also accepted for review by the European Medicines Agency (EMA). Decisions on their applications are expected in the 4th quarter of 2024 in the U.S. and in the first quarter of 2025 in Europe. [Pfizer press release 12/11/23]

### PFIZER PRESENTS PHASE III DATA FOR MARSTACIMAB

12/9/23 At ASH, Pfizer presented the Phase III data for marstacimab that is the basis for their BLA and MAA (see above). In 116 subjects, the six-month lead-in phase of the studies before treatment found average ABRs of 7.85 for prophylactic treatment and 38.0 for on-demand treatment with clotting factors. The twelve-month treatment phase with marstacimab showed a reduction of the average ABR to 5.08 for the group that had been on prophy and to 3.18 for the group that had been on on-demand treatment. Patients who completed the twelve-month treatment phase were able to continue treatment with marstacimab in an extension study. The ABRs for the extension study (87 subjects for 16 more months) were 2.27 for the group that had originally been on prophy and 3.88 for the group that had been on on-demand treatment. There were no significant safety issues and no deaths. However, the quality of life data showed no significant difference from the subject's earlier prophylactic or on-demand treatments. [Matino D et al., ASH abstract 285]

### SANOFI REPORTS ON THE PHARMACOKINETICS OF FITUSIRAN

12/10/23 Sanofi is developing fitusiran, an RNA interference drug that decreases the body's production of antithrombin (AT), an anticoagulant. At ASH, they presented an updated pharmacokinetic (PK) model to correlate the fitusiran dose with AT levels in circulation. The data for the new PK model was obtained from

subjects in the Phase I, Phase I/II and Phase III clinical studies, a total of 339 participants. At one point, the Phase III study was placed on hold because some subjects developed signs of thrombosis (too much clotting), so Sanofi has been working on lowering the dose and better understanding the PK for fitusiran. They found that a starting dose of 50 mg by subcutaneous injection every two months would keep the AT level at the desired 15 – 35% of normal level. (The original clinical dose was 80 mg once a month, which gave an AT level below 10%, apparently too low to prevent thrombosis.) They are currently confirming that finding in clinical studies using the new dosage model. [Madrasi K et al., ASH abstract 2614]

## GENE AND CELL THERAPY

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

### BE BIO REPORTS ON GENETICALLY-ENGINEERED B CELLS FOR FACTOR IX PRODUCTION

12/10/23 Be Biopharma is developing a cell therapy for hemophilia B in which B cells are genetically modified using CRISPR/Cas9 techniques to produce factor IX. B cells are a type of white blood cell that are produced in the bone marrow from stem cells that continuously produce all of the body's blood cells. They are part of the immune system; they recognize antigens (foreign materials that cause an immune response) and produce antibodies against those antigens. B cells continuously divide to produce new B cells that have a "memory" so the new cells can produce the same antibodies as the original cells.

The technique harvests B cells from a patient and then genetically engineers them in the laboratory using the CRISPR/Cas9 method to insert good factor IX genes into the cells. The transformed cells are then expanded (grown into additional cells) and transplanted back into the patient's blood stream where they will eventually find their way to the bone marrow. The cells re-engraft into the bone marrow where they will continuously produce factor IX.

At ASH, Be Bio presented pre-clinical data from experiments in mice and non-human primates that suggests that the method works and appears safe. [Liu H et al., ASH abstract 463]

### CSL GIVES UPDATES ON HEMGENIX

12/9-11/23 CSL's Hemgenix gene therapy for hemophilia B has been on the market for over a year, and CSL continues to provide updates from their clinical studies. One of the unique features of their clinical studies was that they included subjects infected with hepatitis B and C viruses (HBV and HCV) and with HIV, the AIDS virus. People with active HBV/HCV infections or with uncontrolled HIV infections were excluded, but those with historic infections or controlled HIV infections were admitted. At ASH, they reported on the results for the infectious diseases.

Of the 54 Phase III study subjects, 31 (57.4%) had a history of chronic HCV infection while seven of the 31 also had a history of chronic HBV infection. Two subjects were co-infected with HCV and HIV and two were HBV positive, but negative for HCV and HIV. These subject's conditions did not appear to impact the success of the treatment or their factor IX expression. Both the HBV/HCV group and the non-infected group had the same average factor IX level prior to treatment, <1%. Interestingly, the HBV/HCV group had a higher median factor level after three years, 40.5% (range 4.8 to 80.3%) than did the non-infected group at 32.8% (8.6 to 55.5%), but the difference is probably not significant given the wide ranges. The bottom line is that Hemgenix appears safe and effective in patients with chronic HBV or HCV infections. [von Drygalski A et al., ASH abstract 2258]

Similarly, the studies also admitted subjects with HIV infections as long as the infection was under control. Of the 57 participants in both the Phase IIb and Phase III studies, five were infected with HIV. Four of the five had a history of treated HCV infection as well. As with the HBV/HCV subjects reported above, the treatment appeared to be safe and effective with a median factor IX level of 32.3% (range 31.5 to 58%) three years after treatment. However, because of the small number of subjects with HIV, CSL recommends continued study of HIV patients receiving Hemgenix. [Pipe S et al., ASH abstract 2256]

CSL also presented an update from the Phase III clinical studies three years after treatment. They found an average ABR of 1.52 compared to the pre-treatment level of 4.17. The average factor IX levels in the study subjects were 41.5% (range 5.9 to 113%) after the first year, 36.7% (4.7 to 99.2%) after year 2 and 38.6% (4.8 to 80.3%) after year 3. After three years 51 subjects (94%) did not need prophylaxis. [Pipe S et al., ASH abstract 1055]

9/9/23 At the earlier BIC conference in Italy, CSL presented data on the success rate for Hemgenix from the clinical studies. One of the main principles of pharmaceutical development is that you want a product

that has a predictable, reproducible effect. One of the main issues with the current hemophilia B gene therapy products is that their effect is neither predictable nor reproducible. Patients achieve various factor levels after treatment.

Of the 54 total subjects, two (3.7% of total) did not express factor IX. One of those had an extremely high anti-AAV level before treatment and one quit partway through the infusion because of infusion-related reactions. The remaining 52 subjects were all able to quit prophylaxis. Of those, one subject (2% of total) had a factor level <5% of normal; 62% of subjects had a level between 5 and 40%; and 36% had levels above 40% after two years. Nine of the subjects (11%) received oral corticosteroids because of liver inflammation. [Pipe SW et al., BIC abstract OC-10]

## FREELINE BEING TAKEN PRIVATE BY SYNCONA

11/22/23 Freeline Therapeutics has been developing verbrinacogene setparvovec (FLT-180a), 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 treatment appears promising, being able to increase factor IX levels into the normal range. Unfortunately, they ran into money problems and in mid-2022 decided to stop their work on FLT180a until they could find a development partner. Now Syncona, a British investment firm, is working on a deal to take Freeline private (off the stock markets). The deal is expected to close in the first quarter of 2024. The company's plans for further development of FLT-180a have not been announced. [Biopharma Dive article, 11/22/23]

## PFIZER GIVES UPDATES ON GENE THERAPY

12/9-10/23 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. They presented two updates at ASH on the results from their clinical studies.

The first paper looked at six (out of 45 total) Phase III clinical study subjects who had returned to factor IX prophylaxis (RTP) after initially good results from the treatment. The six RTP patients switched back to prophylaxis either because their factor IX (FIX) levels dropped below 2% or because they had bleeding episodes. All six had initially responded well with FIX levels ranging from 7 to 22% of normal. At the time they returned to prophylaxis, however, their levels had fallen to a range of 0.5 to 5.8%. Their time of switching ranged from 155 to 623 days after treatment. All of the subjects had been given corticosteroids to reduce liver inflammation and hopefully stabilize their FIX production. Pfizer is still looking at the cause of their RTP. [Frenzel L et al., ASH abstract 2257]

Pfizer's other paper reported on health-related quality of life (HRQoL) for 14 subjects in their Phase I/IIa study using questionnaires. At week 156 after treatment, the subject's results from the Haem-a-Qol questionnaire fell by an average of 15.2 points, which represents a significant improvement in HRQoL. EQ-VAS scores had risen by 7 – 8 points two years after treatment indicating an improvement in overall health. A greater proportion of the subjects also reported increasing their physical activity by week 156 compared to their baseline before treatment. [von Mackensen S et al., ASH abstract 3628]

## ACTIVATED FACTOR X STORED IN PLATELETS TO PROMOTE CLOTTING

10/1/23 A group of Chinese investigators is developing a hemophilia B treatment method involving activated factor X (FXa) stored in platelets. Factor X is a pivotal molecule in the clotting cascade. It can be activated by either activated factor IX (FIXa) or activated factor VII (FVIIa). FXa then recruits other clotting factors and other molecules to form a complex that reacts with prothrombin (factor II) to form thrombin (factor IIa). Thrombin then turns fibrinogen (factor I) to fibrin, which is a "sticky" molecules that binds to other fibrin molecules to form the clot.

Platelets are small blood cells that are also part of the clotting system. They are activated when the clotting cascade starts producing activated clotting factors. Activated platelets are also "sticky" and bind to other activated platelets as well as to fibrin. The combination of the clumped platelets covered by a network of fibrin strands, as well as other blood cells that get caught up and tangled in it, forms the actual clot that seals up a hole in the blood vessel.

The Chinese researchers found that they can genetically engineer the hematopoietic stem cells in the bone marrow to produce platelets that contain FXa. When those platelets are activated to form a clot, they release the FXa, which increases the amount of clotting occurring. They showed that these modified platelets could produce significant clotting in hemophilic mice, even in mice with inhibitors. The group initially was looking at treatment of inhibitor patients with the FXa-platelets, but have now expanded their target to include hemophilia patients without inhibitors. Whether this will be a viable alternative to factor IX gene therapy remains to be seen. [Han W et al., Nature Sci. Rep., 13(1), 16488, 2023]

## JAPANESE GROUP REPORTS ON GENE THERAPY

9/14/23 Interest in gene therapy seems to be booming. A group of researchers in Japan is developing an improved AAV vector and delivery technique for gene therapy of factor IX. They are attempting to solve the

problems of the high vector doses needed because of inefficient transfer of genes to cells and the immune issues caused by the viral vector. They have developed a vector called AAV.GT5 that is about 100 times more efficient at getting genes into liver cells. They have also shown that injection of AAV.GT5 directly into the liver, rather than into the general circulation, enhances gene transfer. AAV.GT5 also appears to be less immunogenic than current AAV vectors. Their recent report on experiments in pigs and macaques shows promising results. [Kashiwakura Y et al., Mol. Ther. Methods Clin. Dev., 30, 502, 2023]

## ST. JUDE/UCL REPORTS ON ORIGINAL GENE THERAPY AFTER TEN YEARS

12/9-11/23 Around ten years ago, a large group of researchers from St. Jude Children's Research Hospital, University College London (UCL) and other institutions performed the first successful hemophilia gene therapy clinical trials. At ASH, the group gave an update on those early patients. As of the end of 2022, the median follow-up period was 10.7 years (range 4 to 12) for the ten subjects in the study. The subjects received various doses with six subjects in the high-dose cohort.

Four of the six high-dose subjects developed liver inflammation and were treated with oral corticosteroids, which resolved the issue without any recurrence. Two other serious adverse events occurred in two of the patients. One patient developed lung cancer five years after treatment, but this was shown not to be associated with the treatment. Another 72-year-old subject developed prostate cancer twelve years after treatment. That case is still being investigated. The subjects developed low but persistent factor IX levels of 1.7%, 2.3% and 4.9% in the low, medium and high dose cohorts, respectively. Their median ABR over the 10-year period was 1.6 compared to an ABR of 14 before treatment. In the high-dose cohort, the median ABR was 1.0. [Reiss UM et al., ASH abstract 1056]

The St. Jude/UCL group also reported on the persistence of anti-AAV antibodies after treatment. Antibodies against AAV vectors could complicate future treatments using AAV vectors. First, they found that the levels of anti-AAV antibodies in the study subjects were much higher than would normally be obtained from an actual AAV infection. They also found that the antibodies have some cross-reactivity to other AAV types. The antibody levels gradually fell over the ten-year period but still remained too high for the subjects to receive another dose of gene therapy. Interestingly, they found that AAV5, which is used in Hemgenix, causes the mildest antibody responses, which may allow repeat treatment. [McIntosh JH et al., ASH abstract 2255]

## RED BLOOD CELLS LOADED WITH FACTOR IX

12/11/23 A group from the University of Pennsylvania and Temple University has been exploring the possibility of attaching factor IX (FIX) molecules to the surface of red blood cells (RBCs). RBCs have a lifetime in circulation of about 120 days, so this would give the FIX an extra-long half-life. The FIX is linked to a mouse glycophorin A (GPA) antibody and then incubated with RBCs. RBCs have glycophorin A molecules on their surfaces, so the GPA antibodies bind to those molecules bringing the factor IX along with them. The group also found that if they inject just the bare FIX-GPA molecule into mice it will find the mouse's RBCs and bind to them. Therefore, the FIX-GPA molecule could be used by itself without first attaching it to RBCs. The researchers showed that the FIX-GPA-RBC complex retains the clotting ability of the FIX molecule. [Peshkova AD et al., ASH abstract 5006]

## SEN. BILL CASSIDY WANTS TO IMPROVE ACCESS TO GENE THERAPIES

12/6/23 Senator Bill Cassidy (R-Louisiana) is looking for feedback from stakeholders (involved parties) with the idea of crafting legislation that would make cell and gene therapies more accessible for patients. Cassidy points out that the small number of current gene and cell therapy treatments can be handled adequately, but as the number of approvals skyrocket over the next few years, the market is not sustainable. This is a balancing act because prices need to be low enough to let all patients have access, but still need to be high enough to give incentives for future research and product improvement. [Biospace article 12/6/23]

## FRENCH STUDY SHOWS PATIENTS' PERSPECTIVES ON GENE THERAPY

12/6/23 A French study looked at patient perspectives on hemophilia gene therapy. They received 137 responses to a questionnaire (about 3.5% of French people over 16 years old with severe or moderate hemophilia) with 80.3% from hemophilia A patients and 19.7% from hemophilia B patients. About 64.2% of respondents were curious about gene therapy, with 33.6% of the total being ready as soon as possible and 38.7% wanting to wait until more patients have been treated. Only 3.6% of patients stated that they would never be interested in gene therapy. About 39.5% of patients said that they were not knowledgeable enough about the treatments, and more than 60% had not discussed gene therapy with their physician or treatment center. [Pietu G et al., Haemophilia, online ahead of print 12/6/23]

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MEDEXUS PHARMA

# AGING WITH HEMOPHILIA B CAN'T STOP THIS COMMUNITY!

**BY KATIE CROMBE**

**Community members joined the *Aging with Hemophilia B* virtual programming for individuals 50 and over from the summer through the fall! This new online series consisted of six monthly meetings for our aging hemophilia community members, from June 22nd to November 9th, to come together in fellowship and education.**

Thanks to a sponsorship from Sanofi, the Coalition for Hemophilia B was able to invite an expert speaker to each event. The series opened with advice on navigating the legal landscape of aging with long-time community supporter, Donnie Akers, Jr. Donnie empowered attendees with knowledge about wills, powers of attorney, trusts, and tools for safeguarding against potential financial exploitation.

Two of the sessions focused on mental health and finding companionship later in life. Matt Barkdull led a discussion on how to reignite love and connection in mature relationships – dating, platonic, and long-term - followed by a discussion among community members regarding their own successes, failures, questions, and advice.

Dr. Mina D. Nguyen-Driver also led a session on nurturing relationships in late life, along with guest speaker and nurse, Robi Ingram-Rich. Dr. Mina and Robi shared advice on self-care, emotional intimacy, and maintaining a vibrant connection within the hemophilia B community.

In addition to presentations, our community members were encouraged to get active with Michael Zolotnisky's session, *Stay Fit and Fabulous*, where he taught the group ten simple exercises for strengthening balance to prevent slips and falls.

Continuing the emphasis on healthy living, Dr. Robert Lawrence Friedman shared information on *The Blue Zone Lifestyle* and what we can learn from centenarians and their secrets to living a long and fulfilling life. Finally, Dr. William Patsakos, a clinical pharmacist and member of the hemophilia community, discussed how to balance and manage medications, which is especially important for those aging with hemophilia.

While each expert brought valuable insight and knowledge, the strength of the series was the fellowship among the community members themselves. Every session was wrapped with conversation, support, advice, and laughter that was meant to last 30 minutes and sometimes carried on well past 'bedtime' for many.

The quality of our hemophilia community was reinforced as members returned month after month to share updates about their lives with friends, new and old - even finding times to connect outside of the virtual event series!

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## COMMENTS:

"I think, for the first time, we felt relaxed and tension-free after meeting friends and family in this meet-up. We most appreciated how everyone was very open, and everyone appreciated us when we discussed our family background, how we moved from India to the USA for treatment and our future concerns. Definitely, we prefer to have the elder group where we can communicate with each other easily as family."

"Listening to those who were my age and older who also attended the meeting added to the daily satisfaction I have in proving that the Negative Nellys in my life were wrong. Your meeting gave greater affirmation to my motto, 'The Best Revenge Is a Life Well Lived.' Such meetings and opportunities you provide for interaction with those who may have faced the same difficulties and setbacks that I have had in life are greatly appreciated. I no longer feel isolated, or excluded, or as if I have missed out on life. Thank you for being a positive and self-affirming part of the lives of those who wrestle with the ongoing complications of living with hemophilia."

"I love the 50 and over group. The information is really helpful, and it's currently my favorite gathering. I thought having the men and women together might be awkward, but it's actually been really wonderful!"

"I enjoyed the monthly meetings with our "over 50" group.

Having meetings with others of the same age bracket, experiencing some of the same physical and emotional concerns was helpful. The speakers were awesome and engaging. This is a group of people where my comfort level was at 100 in terms of being able to "bare my soul" and speak from the heart - no topics were off base, and we could talk about anything. Looking forward to new meetings in the new year!"

# VIRTUAL CAREER AND FINANCIAL WORKSHOPS

**BY ROCKY WILLIAMS**

Our first career-building session on October 25th with April Willis, PhD, was nothing short of amazing! April took us on a transformative journey, showing us how to craft a resume and cover letter that truly make us look like works of art on paper. Her insights were not only eye-opening but also incredibly practical, equipping us with the tools to stand out in the job market. The session didn't stop there; she guided us through the intricacies of building an impressive LinkedIn profile, ensuring that we not only capture attention but also leave a lasting impact on hiring managers.

Then on November 15th, we kept the momentum going as we continued our journey towards career and financial success! We explored how to maximize the LinkedIn job search feature, key interview tips, how to negotiate a promotion, and how to leverage performance reviews!

Each time, the energy in the room was electric, and the groups were highly engaged, making the experience all the more enriching. Our burning career and finance questions were answered with precision, leaving us all feeling more confident and ready to take on the professional world. It was a session we won't soon forget!

# FINDING COMMUNITY THROUGH PARENTING

**BY KATIE CROMBE**

Parenting in itself can be quite challenging; however, parenting a child with hemophilia introduces even more complexities. For new parents of children with hemophilia, the experience might often feel like navigating life one day at a time. Fortunately, the Coalition for Hemophilia B has launched a new virtual parenting support series to lend a helping hand!

Hosted by community members Cassandra Starks and Megan King McCormick, the series titled, *New Parents and Hemophilia* comprised four sessions held from June to November. Each session commenced with icebreakers and discussions guided by Cassandra and Megan. Following was a presentation which culminating in further conversations, fellowship, and support.

Thanks to the generous sponsorship from Sanofi, these sessions featured expert presentations by Dr. Mina D Nguyen-Driver, a Professor in the Department of Pediatrics at the University of Arkansas for Medical Sciences. Drawing from her experience in hemophilia care and mental health within the community, Dr. Mina crafted a comprehensive four-part series covering various aspects of parenting a child with hemophilia B. From coping with a diagnosis to advocating for your child, managing medical appointments and infusions, and establishing a support network among other parents of children with hemophilia, attendees gleaned valuable insights and strategies from Dr. Mina throughout the series. Each session concluded with a Q&A segment where parents could share their stories and seek advice related to the topic.


Following the expert presentations, community members engaged in fellowship facilitated by the hosts. Cassandra and Megan with eight children between them and extensive experience in dealing with hemophilia, shared their wisdom and experiences during these sessions. Their conversations, filled with stories of struggles and successes, evoked laughter and tears, creating a safe and inclusive space where every community member felt acknowledged, understood, and supported, despite the virtual setting. Through their guidance, attendees not only gained information about parenting a child with hemophilia but also forged new friendships within the community.

Feedback from participants speaks volumes:

"I truly valued the Virtual Parenting events! It was a fantastic way to connect with other parents who are new to the hemophilia B community. While conversations with families having older children provide hope and insight into the future, connecting with peers at a similar stage proved incredibly beneficial. The themed sessions allowed us to focus our discussions, and Dr. Mina's facilitation was outstanding!"

"Empowering. As a somewhat experienced mom, meeting other parents has been amazing."

"Spending time with other new parents was exactly what I needed. Knowing others are going through similar struggles gives me the strength to face this challenging disorder!"

"Attending the new parents and hemophilia meetings has made me feel less alone and more understood than ever! Conversing and connecting with parents who truly understand has eased our journey into this new phase. These meetings taught me how to be my children's greatest advocate. I'm immensely grateful to the Coalition for organizing these events and for the invaluable knowledge I've gained within our hemophilia B family."

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# women & girls with hemophilia


articles to support, educate, and empower

# Katy Haigood is Coming Out on Top and Soaring to New Heights!

**BY SHELLY FISHER**

Katy Haigood is a unique individual, and not just because she is a woman with hemophilia. She is also a wife, mother, and entrepreneur with a thriving business. The only thing more infectious than her smile is her laugh. She's the kind of person you want to have with you at the hospital when you're scheduled for surgery, and that's just what she was doing when we visited. When I asked her if we needed to reschedule, I heard a cheery voice off screen say, "I'm okay!" At that point, Katy and her pre-op bestie erupted into schoolgirl giggles, and if it hadn't been for the doctor and nurses coming in and out of the room, I would have guessed she had zoomed in straight from a slumber party. She's no stranger to a hospital. Her calm demeanor and supportive humor belied the fact that she's been on the reciprocal side of some difficult surgeries and long recoveries.

When I asked Katy about her diagnosis, her face took on the determined look of someone who had fought a few rounds and emerged triumphant. Family history, years of undiagnosed bleeding requiring emergency operations, including hemorrhaging after the birth of her daughter, and internal hematomas after necessary, risk-reducing surgeries due to positive test results for the BRCA1 gene, led Katy to push for a diagnosis. She wasn't surprised when her hematologist told her she was "fine." After all, "doctors don't really take women seriously on hemophilia issues." The young mother and business owner persevered, was officially diagnosed, and received infusions before and after her final surgery. This resulted in less pain and substantially shorter recovery times. Finally, she had an answer to all of her questions and confirmation for what she instinctively knew was the issue all along.

Katy attributed much of her ability to "rise above it all" to her support system made up of her dad, stepmom, husband, children, and communities like The Coalition for Hemophilia B. Her dad and many of her many male cousins were diagnosed with hemophilia B and it is thanks to them that she recognized her symptoms early on in the journey to resolve her health issues. Her dad has been a member of The Coalition for Hemophilia B for years and Katy was excited to attend the Symposium with him this past March. In fact, she and her family, along with her stepmom and dad, headed to Orlando a little early. "We did some deep-sea fishing and went to Universal Studios - my daughter was finally tall enough to ride some of the big kid rides."

Her devotion to eight-year-old Lisa was evident when

she talked about the impact that the Symposium had on her whole family. “Lisa absolutely loved it there,” and the Coalition gave them the opportunity to meet families facing similar issues, confirmation that Katy was more than a diagnosis and assurance that she wasn’t alone in her journey. “It was just nice to not feel like I was the only one going through these things. They were teaching kids to infuse; they were poking their parents and it was the coolest thing.”

As an entrepreneur, she has taken “rising above it all” to a whole new level, and when I asked about her family business, Tower M&L Services, LLC., Katy’s face lit up. Her pride in her husband Aaron’s abilities were obvious. “My husband has been climbing for 15 years and it just made sense for us to start our own business providing lighting services to cell companies. He’s really good with the electrical systems on the towers, all of the operations, and managing our crew.” Katy shared she handles all the accounting and human resources aspects, and then it became crystal clear that it is truly a family business when she explained the name they chose. “For our customers, M & L stands for maintenance and lighting, but for us, it stands for our children Max and Lisa.” Their clients have taken them across the nation, and they recently visited Washington and California with plans to travel to Maine and New Hampshire in the coming weeks.

After overcoming so many years of unexplained issues, Katy would like nothing better than to build a legacy for her children and keep pushing to new heights in every area of her life. “I’d like to put my kid through college on my dime and leave a thriving business.” After losing her mom at 45, she understands how important it is to make family a priority, but she also cautions moms to take care of their health. “Don’t feel guilty. Take the time you need for yourself.”

Even in her free time, Katy manages to soar to new heights as a roller coaster enthusiast. Her goal is to visit every Six Flag theme park across the U.S., Canada, and Mexico. But just how many are there? There are 27, and I have every confidence Katy and her family will visit each one. “Even with all of the problems I have, I’m still coming out on top and living life.” Thanks to her determination and a heart as big as the state that hosts the original Six Flags Over Texas, the sky is the limit for Katy Haigood and her family.

# INSURANCE

BY ELLEN KACHALSKY, LMSW, ACSW, CCM

If you are working, you may have commercial insurance through your employer. If so, it probably has an annual deductible before coverage, with copays, starts, and an out-of-pocket maximum. If you meet that, you should pay $0 after meeting that maximum.

If you do not have insurance through your employer, you can contact www.healthcare.gov to see if you can get an "Affordable Care Act" health plan (a.k.a. Marketplace plan). Open Enrollment for each upcoming year is November 1st through December 15th, for plans to go into effect January 1st. There may also be other times you can enroll. For additional information, you can call them at 1-800-318-2596.

MEDICARE is the health insurance from the federal government because you worked at least 10 years and you paid Medicare and FICA taxes. You enroll at age 65 or if you've been disabled and received Social Security Disability (SSDI) for 24 months. Here are some things you need to know:

**Medicare has several parts:**

**Medicare Part A = Inpatient Hospital Care**

- $0 monthly premium
- $1,600 deductible for 2023, before Medicare starts paying when you are hospitalized

**Medicare Part B = Outpatient Care (covers doctors, labs, tests, therapy, etc. AND FACTOR!)**

- Monthly premium of $154.90/month in 2023 (the minimum based on income)
- Annual deductible $226/year for 2023

**Medicare Part D = Prescription Drug Plan**

- Monthly premium depends on the plan you choose - Annual deductible $505/year
- After meeting the deductible, you pay co-pays for medications. The co-pays depend on which tier or level the medication is placed by the prescription drug plan carrier. Some classes of drug may not be covered.

***Since Medicare pays about 80% of charges, you need additional coverage. You can choose EITHER:***

**1.) A MEDICARE SUPPLEMENTAL PLAN (MEDIGAP)**

- Monthly premium depends on the plan you choose. Medicare supplements are ranked with letters A through N, and each letter designated plan has minimum standard features which must be provided by the carrier. So, if you have plan G, every company, such as Blue Cross, Aetna, United Healthcare, etc. must provide the same benefits. These plans can help pay some or all of the deductibles listed above.

See the chart on the next page below for more information about coverage features these plans must have.

**2.) A MEDICARE ADVANTAGE PLAN**
(also called Medicare Part C). There is a monthly premium for these, but it rolls parts A, B & D together with a supplement.

Monthly premium depends on the plan you choose. These plans include Medicare Plus Blue, Aetna Medicare Advantage, or whatever plans are available in your state. You may have already started receiving mail from many of these plans. These plans may offer some additional services and may cap your out-of-pocket expenses, but they may limit your choice of healthcare providers to certain networks and hospitals, specific to your state, although there are a few national plans.

They may also require you to use a specific specialty pharmacy for getting your factor and maintenance medications. Make sure your HTC is in network.
If you have both Medicare and another insurance, perhaps through a spouse who may still be working, or you are still working, or you have health care insurance as part of a retiree benefit, there are rules about which plan pays first. In that case, if your other insurance has prescription coverage that is better than that from Medicare Part D, you may choose to not enroll in Part D.

If you are working, your health insurance through your employer pays first. To determine which coverage

comes first and how insurance can work together in your situation, please call the *Benefits Coordination & Recovery Center* at 1-855-798-2627.

Once you are on Medicare, there is an *Open Enrollment Period* every October 15 through December 7 for the upcoming year. You can compare plans and then decide to remain with the same supplemental plan and prescription drug plan or to change your plans.

For help, reach out to your HTC Social Worker or Financial Counselor, Bleeding Disorder chapters and organizations. You can also call the Medicare Medicaid Assistance Program (MMAP) at your local *Area Agency on Aging*, phone 800-803-7174; they can help guide you. Another resource is www.eHealthInsurance.com, a free service to help shop, compare and buy coverage. The phone number is 855-809-6114.

## Some things to remember:

Budget for the costs of Medicare and the supplement, including the annual deductibles and co-pays. If your income is lower you may qualify for help with some, or all, of the Part A & B premiums, deductibles, co-insurance and co-pays under the Medicare Saving Programs (SLMB, QMB, or QI segments).

Contact your local Medicaid office or state health insurance assistance program (SHIP) at www.shiptacenter.org. You may also qualify for "Extra Help with Prescription Drugs" for Part D prescription drug plans, depending on income and assets.

For more information, call 1-800-772-1213 or apply online at:
Apply for Medicare Part D Extra Help program | SSA

(See charts on the next two pages.)

If your income is very low, you may qualify for Medicaid through your state, and this will be your secondary/ supplemental insurance. Again, check if your HTC and doctors are in network if you are told to choose a Medicare Managed Care Plan for dual enrollment.

MEDICAREFAQ
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**MEDICARE SUPPLEMENT (MEDIGAP) STANDARDIZED PLANS - EFFECTIVE AFTER JAN.1, 2023**

★ = POLICY COVERS 100% OF BENEFIT  % = POLICY COVERS THAT PERCENTAGE  BLANK = POLICY DOES NOT COVER THAT BENEFIT

| BENEFITS | MEDICARE SUPPLEMENT (MEDIGAP) PLANS | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | A | B | C[4] | D | F[4] | HD-F[1,4] | G | HD-G[1] | K | L | M | N[3] |
| Medicare Part A Coinsurance hospital costs up to an additional 365 days after Medicare benefits are used up. | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ |
| Medicare Part B Coinsurance or Co-Pay | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | 50% | 75% | ★ | ★ |
| Blood (First 3 Pints) | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | 50% | 75% | ★ | ★ |
| Part A Hospice Care Coinsurance or Co-Pay | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | 50% | 75% | ★ | ★ |
| Skilled Nursing Facility Care Coinsurance | | | ★ | ★ | ★ | ★ | ★ | ★ | 50% | 75% | ★ | ★ |
| Medicare Part A Deductible: $1,600 | | ★ | ★ | ★ | ★ | ★ | ★ | ★ | 50% | 75% | 50% | ★ |
| Medicare Part B Deductible: $226 | | | ★ | | ★ | ★ | | | | | | |
| Medicare Part B Excess Charges | | | | | ★ | ★ | ★ | ★ | | | | |
| Foreign Travel Emergency (Up to Plan Limit) | | | ★ | ★ | ★ | ★ | ★ | ★ | | | ★ | ★ |
| Medicare Preventive Care Part B Coinsurance | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ | ★ |
| | | | | | | | | | OUT-OF-POCKET LIMIT[2] $6,940 | | $3,470 | |

**FOOTNOTES:**

[1] Plans F & G offers a high-deductible plan. If you choose this option, this means that you must pay for Medicare-covered costs up to the deductible amount of $2,700 (2023) before your Medigap plan pays anything.

[2] After you meet your out-of-pocket limit and your yearly Part B deductible ($226 in 2023), the Medigap plan pays 100% of covered services for the rest of the calendar year.

[3] Plan N pays 100% of the Part B coinsurance, except for a copayment of up to $20 for some office visits and up to $50 copayment for emergency room visits that don't result in an inpatient admission.

[4] Plan F, High Deductible Plan F & Plan C are ONLY available to those who were considered Medicare-eligible prior to 2020.

# Medicare Savings Program financial eligibility guidelines

To qualify for a Medicare Savings Program (MSP), you must meet your state's income and asset limits. Listed below are the baseline federal income and asset limits for each MSP. Most states use these limits, but some states have different guidelines. For example, Alaska, Connecticut, the District of Columbia (DC), Hawaii, and Maine have higher income limits. Alabama, Arizona, Connecticut, Delaware, DC, Mississippi, New York, and Vermont do not apply asset limits.

## 2023 MSP eligibility standards*

### Qualified Medicare Beneficiary (QMB)

Gross monthly income limits: 100% Federal Poverty Level, or FPL, + $20**

| | | |
|---|---|---|
| Most states: | $1,235 – Individual | $1,663 – Couple |
| Asset limits: | $9,090 – Individual | $13,630 – Couple |

### Specified Low-income Medicare Beneficiary (SLMB)

Gross monthly income limits: 120% FPL + $20

| | | |
|---|---|---|
| Most states: | $1,478 – Individual | $1,992 – Couple |
| Asset limits: | $9,090 – Individual | $13,630 – Couple |

### Qualifying Individual (QI)

Gross monthly income limits: 135% FPL + $20

| | | |
|---|---|---|
| Most states: | $1,660 – Individual | $2,239 – Couple |
| Asset limits: | $9,090 – Individual | $13,630 – Couple |

*Income limits, which are based on the FPL, change each year. New limits are typically released in either January or February and take effect January 1.

**The amounts listed above include a standard $20 income disregard. Your state may disregard other income as well. Contact your local Medicaid office or State Health Insurance Assistance Program (SHIP) for state-specific guidelines and information. Visit www.shiptacenter.org or call 877-839-2675 to locate your SHIP.

See the next page for information on income and asset disregards applicable in all states.

# Medicare Savings Program financial eligibility guidelines

## Income and asset disregards

If your income seems above Medicare Savings Program income and asset guidelines in your state, you should still apply. This is because you may still qualify for an MSP because certain income and assets may not be counted when determining your eligibility.

### In all states, the following income is not counted:

- The first $20 of your monthly income
- The first $65 of your monthly wages
- Half of your monthly wages (after the $65 is deducted)
- Food stamps (Supplemental Nutrition Assistance Program (SNAP) support)

Some states exclude more of your monthly income than the examples listed above.

### In all states, the following assets are not counted:

- Your primary house
- One car
- Household goods and wedding/engagement rings
- Burial spaces
- Burials funds up to $1,500 per person
- Life insurance with a cash value of less than $1,500

Remember, how your income and assets are counted to determine eligibility varies from state to state. Call your local Medicaid office or SHIP (visit www.shiptacenter.org) to find out if you are eligible for an MSP in your state.

 Helpline: 800-333-4114 www.medicareinteractive.org

# FAMILY HEALTH INSURANCE IS NO LONGER AFFORDABLE THROUGH SMALL EMPLOYERS

**BY GLENN MONES**

In an article discussing its 25th annual employer health benefit survey, Drew Altman of KFF-Kaiser Family Foundation suggests that "small employers no longer offer affordable coverage for workers with families." Altman supports this assertion by referencing a broad range of data from the survey. For instance, employees working at small firms—defined as those with fewer than two hundred employees—would need to pay an average of $8,334 annually for family coverage premiums.

Additionally, a quarter of covered workers at small firms must shell out $12,000 or more each year to enroll in family coverage. Altman further illustrates that "what workers pay for deductibles or other out-of-pocket costs" tends to be notably higher at small firms.

Altman's conclusion is clear: "For many of the nearly 50 million individuals employed by the 3.2 million smaller companies in America, family coverage is no longer within affordable reach."

To read Altman's full article, please view it on the KFF website at https://www.kff.org/health-costs/perspective/family-health-insurance-is-no-longer-affordable-through-small-employers/

The Coalition for Hemophilia B

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

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# CYBERBULLYING AND SOCIAL MEDIA PART II: EMPOWERING TOOLS FOR PARENTS

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

**"I had the opportunity to write an article for the Coalition's 2023 summer newsletter entitled "Cyberbullying and Social Media: A Troubling Trend." In that article, I outlined current statistics that paint a shocking picture regarding the problems concerning cyberbullying, the repercussions of these problems, and tips as well as resources that can immediately be implemented to better protect our youth against this growing epidemic. Following its publication, Coalition leaders recommended a follow-up article be written as a companion and extension of the first to further explore more what parents can do to empower their youth WHEN cyberbullying is encountered. Parents and guardians, let's explore two empowering principles that can give us necessary tools to best combat cyberbullying.**

### Empowering Idea #1 – Follow the "Prophy" Principle

To explore learning how best to conquer any opposing force, let's learn from a practice that's all too familiar in the bleeding disorder community—prophylaxis or "prophy". Prophy is all about preventing bleeding episodes so long-term damage to our precious joints and other vulnerable areas can be avoided. Yes, it comes at the cost of regular needle sticks, but the benefits of better health and protection often always outweigh the burdens.

Let's liken this principle to a war. In battles, victory is not only attained by withstanding strikes or by continually taking on a defensive posture. An offensive strategy is also needed. Inflicting critical hits against the enemy promotes weakening and demoralizing the troops until surrender is secured. General George Washington's following quote is instructive: "Make [the troops] believe that offensive operations oftentimes are the surest, if not the only, means of defense." In other words, as is oft repeated in sports, "The best defense is a good offense". Therefore, prophy is one offensive strategy we can use to keep our bodies healthy and protected.

Now, let's use this offensive-based prophy principle in relationship to protecting our youth from cyberbullying. The following points encompass some of the most powerful and offensive strategies that inflict the critical hits necessary to protect our youth:

- Before our children are old enough to discover or utilize social media, create a home-based culture where their goodness is continually reinforced through noticing and giving genuine compliments, accentuating the positive, and building their sense of confidence by continually reinforcing a can-do attitude. Parents' pièce de résistance of parenting is showering their children with genuine confidence so when they do encounter cyberbullying, they have a firmer foundation from which to draw and increase their resilience when such attacks occur. Don't worry if a child is older or already exposed to the dark side of social media. While yesterday is the best time to plant a tree, the second-best time is right now. Creating such a culture requires parents to exercise a higher degree of patience and awareness of their child's activities that may prove initially difficult, but the rewards can be breathtaking. If not already practiced, begin now to learn the joys of planting the magical seeds that grow into greater self-esteem and self-confidence for children.

- Protecting our youth through education is another powerful way to promote an offensive strategy to cyberbullying. Exposing the dangers of online activity opens the door of establishing trust with children. Education does not always come in the form of outlining dangers, however. More importantly, education also helps empower children so they know what to do when they encounter cyberbullying including talking to parents or a trusted adult about what they've experienced. Our children are more confident and likely to confide in us because of the affirming culture established and the past empowering words and actions we've deposited into their fertile minds.
- If/when children do come to discuss problems they've encountered on-line, know that doing so may be very difficult for them as they may be experiencing shame based on what they've experienced. Parents

reinforcing their love, iterating being proud that they made the issue known, and promoting the message that they will be kept safe are powerful parenting moments that often strengthens the welded bond between parent and child. Regularly emphasizing the child's goodness that builds self-esteem and self-confidence sends a beautiful, affirming message; thereby, preserving and nourishing their powerful identities.

### Empowering Idea #2 – Defend the Fortress

While there remains plenty of good around us, it's not a far cry or a foreign principle that the world in which we now live is also wrought with dangers. Such dangers often come in many forms and wear different disguises, often making them difficult to discern their true desires and intentions. What may seem harmless and even commonplace, can act as the proverbial wolf in sheep's clothing. All seems to be well until the wolf shows its teeth. As the poet Alexander Pope warns:

> Vice is a monster of so frightful mien
>
> As to be hated needs but to be seen;
>
> Yet seen too oft, familiar with her face,
>
> We first endure, then pity, then embrace.

With such dangers lurking to snare our children, areas of defense and safety must be erected to counter these influences. Unfortunately, even the most reinforced of such places still has its cracks and fissures. The solution is not to smother, distract, or cutoff our children from experiencing life; in fact, these actions have what I call the Rapunzel effect where our children begin to rebel, stall in their development, and eventually grow to resent their "captors". Protection is not black and white—it's taking a balanced approach where mistakes are allowed but learned from, contention exists but worked through, and shame is experienced but consoled with.

While the balanced approach is most healthy, parents still stand as the sentinels to what should be the safest, most secure location a child can experience—the home. Even when children venture outside the home, their parents' influence can still be felt as they face a barrage of messages and stimuli that often run counter to what they've been taught. When it comes to taking this defensive posture, consider these strategies when protecting children from cyberbullying:

- Be the example. The greatest learning our children experience often comes from their parents. They are powerfully shaped and molded from what they routinely see and experience at a very young age and that largely stems from a parental source of observation. Be the one to lead out by setting the standard. This means taking care to limit social media and screen time yourselves. As parents, we can set the thermostat as to what should be expected.

- Fire the electronic babysitter. Screen time has too often replaced human connection; thereby, diminishing the critical and beautiful experience of interaction, bonding, and securing healthy attachments. While some screen time can be appropriate, anything that promotes excessive and indulgent behaviors will become problematic. Finding opportunities to research, plan for, and execute activities that don't involve the screen are equivalent to healthy eating, exercising, and getting enough sleep. As parents build a culture of balancing screen and non-screen activities, children won't be so tempted to rely on the Internet to fill their social and entertainment vacuum; thus, limiting the potential impact of cyberbullying.
- Toe the social media line. It's interesting to observe how many potentially dangerous areas are almost always equipped with barriers to entry. Barriers include locks, gates, fences, alarms, passwords, security personnel, barbed wire, among many other common means to block and protect access. When it comes to social media, however, most sites allow kids as young as 13 (and often much younger) to gain access to a world full of dangers where not everything is always as it seems. Parents must assume the role of sentinels to guard against these dangers by toeing the social media line. Don't assume that these sites will protect the user. Parents must assume the responsibility of deciding how little and how much social media is used, when such access should be given, the degree of parental control that's implemented, and other important tasks and decisions.

This article may be seen as taking a hard stance against social media and that the author may even hold a strong bias and prejudice against such sites. As a social scientist, there's evidence that has also been uncovered showing social media, when properly utilized, is also a positive means to make and maintain important connections and spreading goodness around the globe. Unfortunately, with every good thing comes its vice and one of these vices comes in the form of cyberbullying. It's my hope that this article has helped educate parents as to the problem and that solutions presented herein have provided food for thought. When it's all said and done in the end, it's my hope that we collectively do everything in our power to preserve and protect the greatest of all resources—our precious children."

# AIDEN – AN OVERCOMER AND A TRUE LEADER ON AND OFF THE FIELD!

**BY SHELLY FISHER**

**I caught up with Aiden in the middle of a newsworthy football season, and after an interview with Danny Chinos from "Nobody Cares Sports." As the captain of a young football team, this senior not only had the plays on the field, but he also has the wisdom of someone much older than 17. As I watched the clip of his 2-point conversion that gave his team the win, I was even more impressed by his interview after the game. He spoke with grace, a humble nature and a sense of humor.**


He was every bit as unassuming in person while he talked with me about the importance of spending time with family and friends, football, getting his driver's license, and the two things that he valued most: loyalty and love. Though he wore the usual hoodie typical of someone his age, Aiden was anything but typical.

It's not surprising that the majority of our time was spent talking about friends and family. Aiden told me he "keeps his circle small," and he truly values those who are in it. He spoke fondly of the two weeks he spent in Florida last summer with cousins he doesn't get to see much, and even though they hit the beach and some trampoline parks, he enjoyed the time they spent just hanging out at their house. The senior credited his family with teaching him to do "tough and great things," and he said that his parents raised him to be who he is today.

Football seemed to bring a lot of purpose and passion to Aiden's life. Having joined the varsity team as a sophomore, he told me he was now one of only eight seniors. In the interview with *Nobody Cares Sports*, he credited his coaches for "calling the plays," and encouraging the team throughout the week. He felt that "they will go far" this season due to the heart they show for the game.

Church is an important part of Aiden's life and he and his family go to communion every Sunday morning. He shared he enjoys the teen camp in the summer as well.

What's the star of the team looking forward to right now? Driving! Though he hadn't taken his driving test just yet, he already had his eyes on his mom's new Nissan Altima, and he was thinking they could

probably share it. It seemed that Aiden was not a huge fan of having to ride public transport to school and games, but he shared that riding with friends made it more fun.

Diagnosed at birth, Aiden has always known that he had hemophilia B. After sustaining multiple injuries, such as a broken wrist, sprains, fractured bones, and a head injury while playing laser tag as a toddler, he's no stranger to infusions. He viewed his condition as an obstacle to overcome and he had this advice for anyone newly diagnosed with hemophilia B:

"Don't let it limit you as to what you can do. You can do a lot of things. I have hemophilia. I play football. I play baseball. I play soccer. It's just an obstacle to overcome."

Loyalty and love were two things that this athlete valued most. He felt that loyalty especially was important, "Because to be loyal you need to trust something, or you need to trust each other. To trust you need loyalty, and with loyalty, you need love." He also added, "Loyalty is stronger than love because love only lasts for so long. Loyalty is forever in a relationship because it's strong like a bond or a chain."

Though his high school courses are preparing him for a technical degree in electricity, Aiden confided that he's been thinking about coaching like his dad lately. Based on what he's doing as the captain of his team, his communication skills, and his drive, it seemed like a natural career choice.

In addition to being a coach, Aiden's dad is also the recipient of the Stanley Lifetime Achievement Award for extraordinary lifetime service, including his work as a volunteer nationwide, his professionalism, and his leadership in the bleeding disorder community. The Coalition for Hemophilia B is extremely grateful for his tireless efforts and dedication.

It is clear that Aiden has an old soul with high standards for his game and life in general. Though he was determined to put his school on the map, it was uncertain if this would be achieved, but one thing is for sure...Aiden's impact on this world is certain.


# JAYDEN –
# RIDING THE ROLLER COASTER OF LIFE AND LOVING EVERY MINUTE OF IT!

**BY SHELLY FISHER**

**Jayden, an 8th grader with a penchant for chemistry, took time to visit with me about school, his favorite hobbies, and his passion for volunteer work. A dedicated "Swiftie," he also talked about his love for music and gave a special shout-out to his mom and someone at The Coalition for Hemophilia B.**


At school, Jayden shared he is a little competitive. After competing in all subjects, his academic team made it all the way to state. Not surprising for a fan of chemistry, whose favorite element is magnesium because he likes magnets!

A "Swiftie" at heart, this music fan confided that he also loves the artist Melanie Martinez and collecting records. When he's not pursuing his favorite jams, his hobbies also include building Lego sets. One of his largest constructions to date was a replica of the Statue of Liberty in celebration of his trip to New York for the Coalition's Annual Awards Gala.

Near and dear to his heart, Jayden followed in his mother's footsteps and enjoys volunteering. He was proud to celebrate her during our visit and told me she got an award for volunteering and spoke at a symposium.

A member of the Wild Horse Gang, Jayden served by picking up trash on the highway and filled seventeen 5-gallon buckets with pop tops for the Ronald McDonald House this past June. He enjoyed working the

registration desk and helping with a sponsor booth at the symposium as well.

Roller coasters hold a special place in Jayden's heart, but they were just the cherry on top for his many trips to Florida to attend The Coalition for Hemophilia B Symposium.

In addition to meeting new people every year, Jayden said, "You get to learn about stuff not a lot of people would teach you." One person in particular made an impression on Jayden: the coalition's own Rocky Williams. "He's really nice and funny. We got along well after we met at the symposium," Jayden said with a smile.

When asked what his friends would say about him, Jayden thought they might say he was funny. His mom added, "He is very supportive of his friends and family," and Jayden agreed by calling himself the "therapist friend."

It wasn't until he was around four years of age that he had an issue after having his tonsils removed. After his pediatrician advised him to eat chicken nuggets to remove a scab at the back of his throat, he began bleeding profusely.

Three hospital days and a very concerning hemoglobin level later, it was determined that he had hemophilia B and von Willebrand Disease. Though his pediatrician assured his mom that it was a "chance encounter," an ENT thankfully referred Jayden to the Jimmy Everest Center in Oklahoma.

What advice would he have for others diagnosed with hemophilia B? "Don't freak out over it." Jayden said he kind of lives by Taylor Swift's song, "Are You Ready for It?" because "life is crazy, so be ready for it." Wise words for us all to live by, Jayden.

# IT WAS A KAHOOT!

**BY ROCKY WILLIAMS**

We lit up the virtual scene with two spectacular family game nights on October 10th and November 14th! These exciting events, tailor-made for our tweens ages 6 to 12, left us buzzing with unforgettable moments.


Each event kicked off with a burst of energy as we dived into the world of Kahoot. Our tweens clashed in good-natured trivia battles spanning cartoons, movie quotes, superheroes, and a whole hodgepodge of kids' trivia. But that was just the beginning! The "Which Movie Is It Emoji Edition" had everyone decoding film titles with emojis, creating a whirlwind of laughter and friendly banter.

We carved out special segments for Marvel and Star Wars enthusiasts, setting the stage for lively discussions on favorite characters and movies. My daughter's favorite character is Baby Yoda! What's yours? She also loves Spiderman, or should I say, Spidey! How many of you have seen Spidey and His Amazing Friends?

The tween game nights were such a great way for kids to see each other and connect. Each evening transformed into an immersive space for shared passions, punctuated by whimsical family talks—like the timeless debate of choosing between a pet dragon or unicorn, or deciding whether to live in Disney World or have a water park in your backyard.

As each event unfolded, our conversations evolved and each night grew more and more fun. What would you do with a billion dollars? If you worked at the zoo, what role would be your calling? These thought-provoking questions sparked creative responses, adding an extra layer of magic to our virtual gatherings.

But wait, there's more excitement on the horizon! We are taking our game nights to the whole family in January. Please join us on Thursday, January 11th for our next game night. We will also be hosting a game night in Spanish on Thursday, January 18th. It is going to be a blast! Can't wait to see you all there!

## 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.**



# VIRTUAL TEEN NIGHT – GUESSPIONAGE

**BY MUHAMMAD**

**Rocky and I kicked off the Teen Event on Thursday night, November 2nd with *Mission Impossible* music setting the stage for what was a pretty awesome night! The theme for the event was "Guessing." We did lots of that right out of the gate. We had the perfect icebreaker to learn more about each other. We started the night with two truths and a lie.**

We talked about the costumes that we sported for Halloween. We shared our musical talents and which instruments we play. We also found out some of us have many, many cats at home. And we learned that one of us used to have blue hair. So cool!

Following that, licensed family therapist Matt Barkdull did an excellent job leading a rap session. We had lots of fun talking about also sharing how we celebrated Halloween. Topics like what everyone had done on Halloween initiated the conversation and led to deeper issues among teens, such as a dislike for Twix and questioning what in the world Boston Baked Beans are. (Rocky's favorite candy).

Afterwards, we played *Are You Smarter Than Your Hemophilia B*. We learned about factor levels, hemophilia history, and how to be involved with the CHB.. We also learned about advocacy, discussed how we can all be good advocates, and learned more ways to advocate for ourselves.

Finally, the main event of the night started. Guesspionage is a game where you guess the correct percentage that correlates to a fact. For example, one of the questions you can find in the game is, "What percentage of people have gotten a hole-in-one in a game of mini-golf?" From there, the guesser tries to come up with the correct percentage, and everyone else can guess whether they thought the correct percentage was higher or lower. Through this, everyone got to learn a lot about each other through shared stories while also having a great time!

Thank you,Medexus, for sponsoring our event. It was great to spend time with the other teens. We learned a lot and had a fantastic time!


**COMMENTS:**

"I think the event was fun. Muhammad listened to what we had to say and was very involved."

"The event was well planned! The speakers were very engaging, and it's a great opportunity to bring the community together."

"We got to know each other better as we hung out and talked about what we were talented at."

"My favorite part was talking to everyone and just sharing things about our lives."

"It was an amazing experience and great opportunity to play a wonderful game together."

MEDEXUS PHARMA


# EVERYBODY'S ON THE SAME TEAM AT *GETTIN' IN THE GAME* JUNIOR NATIONAL CHAMPIONSHIP!

**BY SHELLY FISHER**

Participants from all over the United States converged in Henderson, Nevada, from October 6th to October 8th this year for the 21st Annual *Gettin' in the Game* (GIG) Junior National Championship (JNC), developed by CSL Behring. Created specifically for children ages 7 to 18 with bleeding disorders, the program provides opportunities to compete in baseball, basketball, golf, or swimming, and engage in both physical fitness education sessions and information-sharing meetings for attending families.

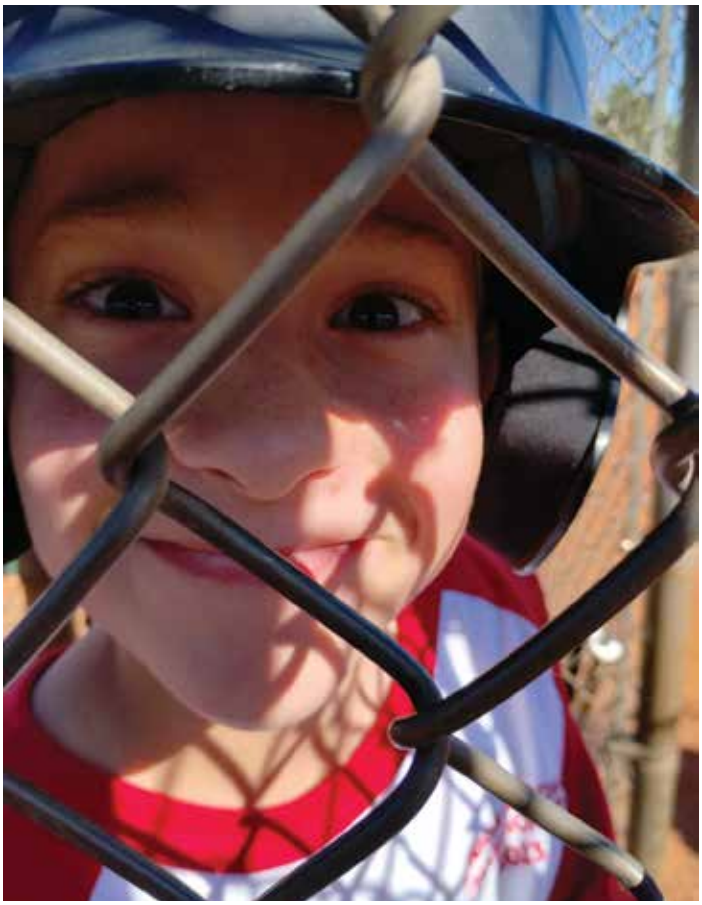
This year, the Coalition for Hemophilia B (CHB) selected two winners from among the many hopeful submissions. Ben's essay on baseball and Hannah's essay on golf earned them both a spot at this year's JNC, and the CHB couldn't have been happier to have them represent us in the annual event.

A second baseman and right fielder at heart, Ben said he likes the complicated side of baseball because it requires him to think fast. He jumped at the chance to go, and as soon as they arrived and got settled into their room, Ben hit the clinics and worked on his hitting, fielding, and pitching with coaches who gave him some tips on his overall game. A highlight for the 12-year-old was learning to throw a "two-seam fastball" that he clocked around 43 miles per hour and then was able to get his baseball signed!

Ben would encourage other kids to submit an essay to attend GIG. Though his favorite memory from GIG

is making friends, he's no stranger to sports injuries and understands the importance of the educational piece well. After getting his nose broken due to a ball taking a bad bounce on the field, he credits his mom for encouraging him to keep playing. After a quick recovery, he finished the championship game with his team, who took second place that season. Way to go, Ben! He also shared that at the GIG's JNC, "you can make new friends, improve baseball skills, or any sport you're doing, and learn more about your bleeding disorder."

With a drive of around 100 yards, 11-year-old Hannah



told me she loves the slow pace of golf and the fact that you don't have to be great at it to have fun playing it with friends and family. How did she get started? She watched her dad play and thought she might give it a try. After playing with her dad's clubs, she was happy to get some of her own size, and the rest is history. She shared that her dad gets to give her some input on the clubs she uses, but she has final say and makes her own selection before each shot. Hannah told me she enjoys getting a treat with her dad after each round.

Hannah and her family were at a golfing event for the hemophilia community when someone mentioned Gettin' in the Game, and she said that she was instantly interested and got busy writing her essay. When she found out that she had been selected, she said she was "so excited!" Her next thought was, "How big is this event and how many people are going to be there?"

After her family arrived and checked into their hotel, they were driven over to the course, where Hannah practiced pitching, putting, and driving. Her favorite memory of her time at GIG is getting help from Coach Betsy because "she was really kind." Her second favorite thing was the food. "I had six cupcakes in one day." Giant checkers and Jenga also made her favorites list before shooting a 59 at her 9-hole tournament. Hannah shared, "It's nice to play with other kids like me with hemophilia and ignore that we could get hurt easily and just play."

When asked if she would recommend Gettin' in the Game to others, she quickly answered, "Yes, it was super fun. There, you know that everyone is on the same team with goals of living a normal life."

Well said, Hannah! You just got a hole in one!

We would like to give a giant thank you to CSL Behring for making such a powerful and impactful event possible.

# B inspired!

*Stories and artwork from teens in the Hemophilia B Community*

FALL 2023

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## 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!