THE COALITION FOR HEMOPHILIA B

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION WINTER 2021

WE CAN'T WAIT TO SEE YOU AT SYMPOSIUM 2022!

REMEMBERING VAL BIAS

INHIBITORS IN HEMOPHILIA

KEEPING THE FLAME WHILE ENDURING THE STORM

CONTENTS

MISSION

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

SYMPOSIUM 2021:

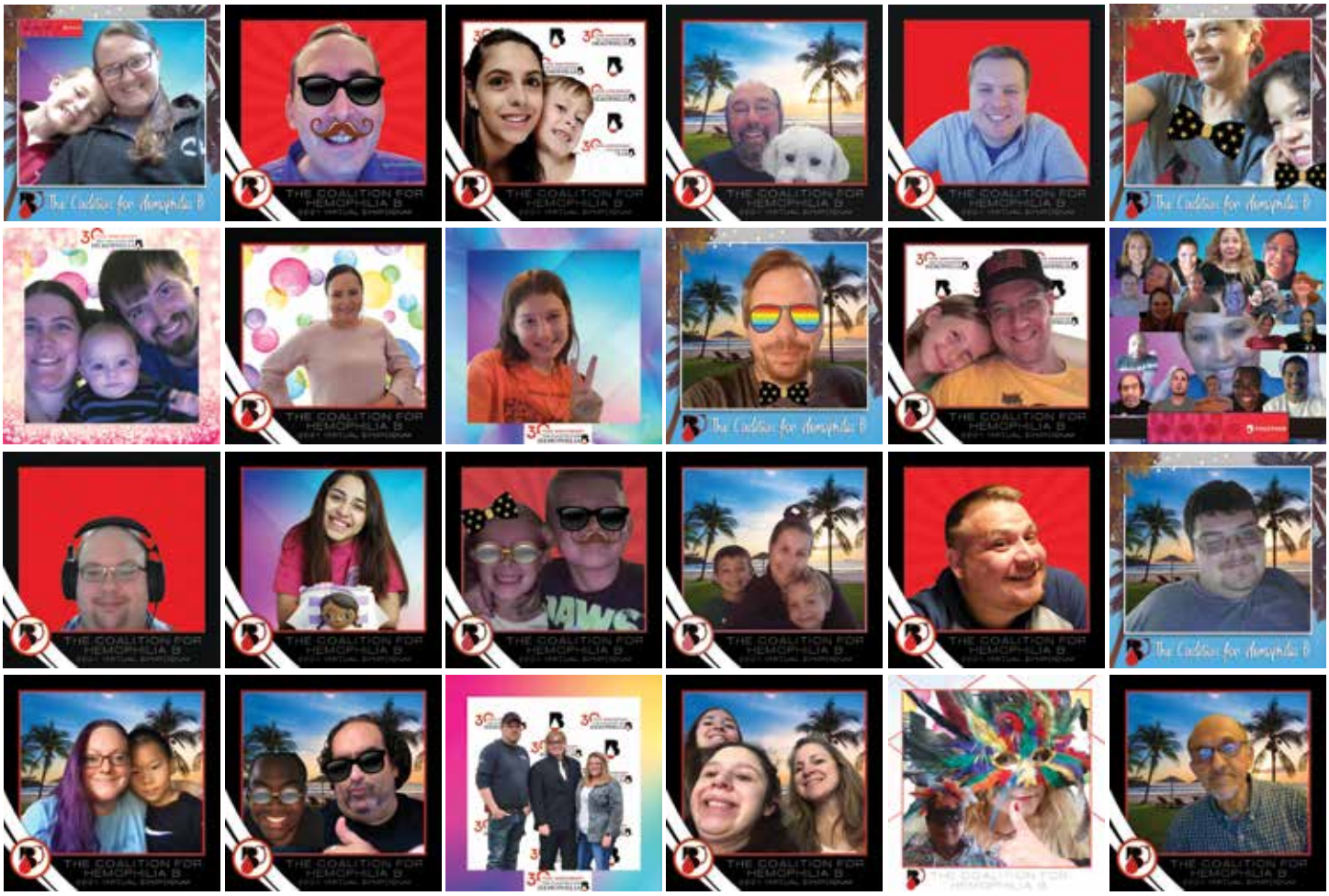
ANOTHER GREAT SUCCESS!

BY GLENN MONES

On the weekend of September 24-26, 2021, community members returned to their computers, tablets and smartphones from living rooms, bedrooms, home offices and kitchen tables across the country for another amazing virtual Symposium by The Coalition for Hemophilia B.

Although our original hope was to return to an in-person event this year, our leadership, in consultation with multiple respected medical authorities, determined the all-virtual option was still the best choice for the health and safety of our members, staff and exhibitors.

Fortunately, we had amassed a great deal of knowledge and experience in hosting such events, not only through our first successful effort last year but also through attending and studying a variety of other events in the community and beyond. In addition, we had the benefit of upgraded technology which has developed exponentially in just the past year. As a result, we were able to bring the program to a new level of engagement and interactivity, and the feedback we received from the participants bears this out. Among the most popular sessions



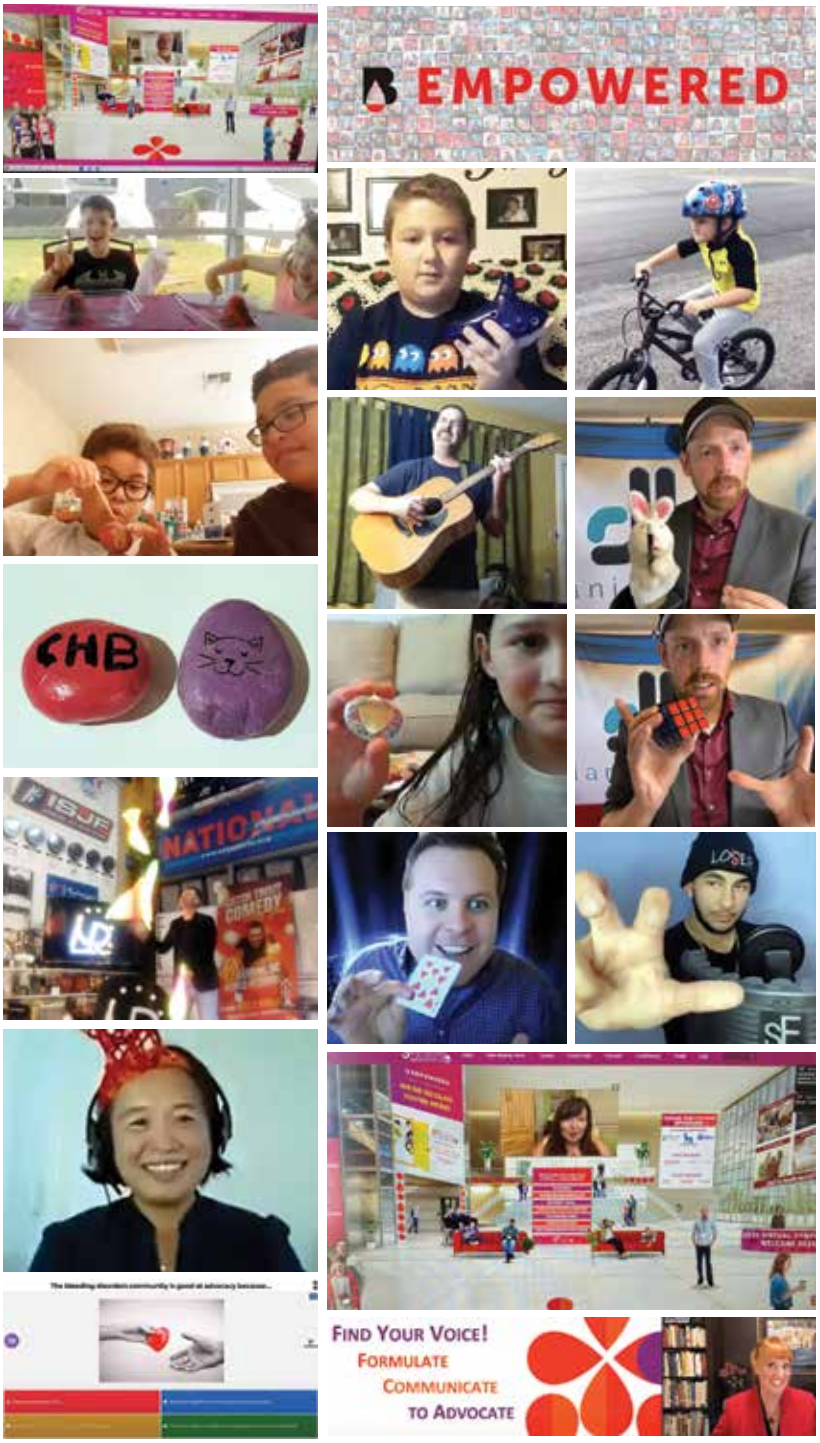
provided participants with up-to-date information on treatment and related medical and scientific topics led by a range of familiar speakers. These included a keynote presentation by Dr. Christopher Walsh who directs the Mount Sinai Hemophilia Treatment Center in New York City. Dr. Walsh gave a comprehensive overview of the status of treatment for hemophilia B.

Another well-attended session was a panel on emerging therapies led by Coalition Chair Dr. David Clark and with the participation of experts from several treatment manufacturers. Several sessions focused on advances in the use of gene therapy to treat hemophilia B including one from the perspective of trial participant Brian O'Mahony, a patient and community leader from Ireland.

Yet other engaging topics included the activity of factor IX outside the bloodstream, the "anatomy of bleeds" with physical therapist Angela Forsyth, and the use of cannabis and CBD to treat pain in hemophilia B with social worker Ellen Kachalsky.

The Symposium also featured sessions designed to empower community members with tools and skills to help them advocate for themselves, their families and their own wellbeing. Topics included several favorites including "Lego Serious Play" with Lee Kim, "What's So Funny" with Robert Friedman, Tai Chi with Rick and Cassandra Starks, kinesiotaping with Dr. Mike Zolotnitsky, art therapy with Jessica Carlisle and Alicia Gattis, being the "best patient advocate" with Donnie Akers, Esq., the B Voice Advocacy with Glenn Mones, "Living the Abundant life" with Matthew Barkdull and "Finding Your Voice" with Natalie Sayer.

Attendees also heard an inspirational



presentation called “Brave is in Our Blood” with community member and mentor Jeron Hill.

The Symposium had “something for everyone,” with multiple programs geared toward specific audiences within the community. These included sessions for women affected by a bleeding disorder, sessions for men, sessions for parents and many others.

Not to be left out, children, teens and young adults also had many programs geared to their age groups and interests. These included a healthy amount of education combined with a variety of creative activities led by the Coalition’s own Rocky Williams, storyteller Milinda DiGiovanni, and others.

Perhaps most important, the Symposium featured multiple opportunities for participants to share their strength and experience while making new connections. The feedback we have received from participants has been stellar, with many commenting on how well we were able to replicate virtually so much of the in-person experience.

The Coalition for Hemophilia B is grateful to our corporate sponsors for having made this very special event possible.

Platinum Sponsors	Gold Sponsors	Silver Sponsors
MEDEXUS PHARMA	CSL Behring	HEMA Biologics
novo nordisk	sanofi	Takeda
Pfizer		TREMEAU PHARMACEUTICALS

We also want to thank our many exhibitors, speakers, facilitators, staff, and of course, all the families and individuals who participated. See you in 2022!

A **ONCE-WEEKLY** TREATMENT OPTION FOR HEMOPHILIA B.

To find out about a prescription option, talk to your doctor or visit

OnceWeeklyForHemophiliaB.com

Pfizer

Help Us Re-Introduce and Advance the Access to Marketplace Insurance Act

The Coalition for Hemophilia B is working with an organization called *United for Charitable Assistance* to ensure that receiving third-party assistance with the costs of treatment is not an obstacle to accessing affordable care. The issue is being addressed in this case through the reintroduction of federal legislation called the *Access to Marketplace Insurance Act*.

The legislation would allow people living with rare and chronic illnesses to access nonprofit patient assistance programs without health insurance providers prohibiting the acceptance of premium assistance or cost-sharing assistance on their behalf. The legislation modifies a current CMS Rule that allows health insurance providers to prohibit accepting charitable assistance. This prohibition is viewed by many as a stealthy way to undercut ACA (Obamacare) reforms that ended insurers' pre-existing condition exclusions.

YOU can assist us in getting this legislation passed. Right now, advocates are focusing on ensuring that the legislation has bipartisan support. As a part of that effort, we are targeting specific legislators as cosponsors based on their support for this legislation in an earlier Congress. **PLEASE CHECK THE LIST BELOW.** If you see someone listed who represents your state (Senate), or your district (HOUSE), **now is the time to reach out to them**. Their email addresses are listed and so is the address for their lead staff who should be copied. You can always look up the contact information of any member of Congress here: https://www.congress.gov/members

Then, customize the sample letter that appears after the list to contact the legislator and staff member. Please send The Coalition for Hemophilia B a copy as well.

If none of your elected members of Congress appear on the list, please think about what friends you may have in these districts. You can also use this opportunity to educate yourself and others about this important issue and prepare for additional actions in the future.

Thank you for your help and let us know if you have any questions.

LIST OF TARGETED POTENTIAL SPONSORS AND LEAD STAFF

Senate:

◊ ***Senator Kyrsten Sinema (D-AZ)***
- Frank Smith: Frank_Smith@sinema.senate.gov

◊ ***Senator Mark Kelly (D-AZ)***
- Katherine Phillips: Katherine_Phillips@kelly.senate.gov

House of Representatives:

◊ ***Congressman Seth Moulton*** (D-MA-6th; Salem, Ipswich, Gloucester)
- John Chambliss: John.Chambliss@mail.house.gov

◊ ***Congressman Emmanuel Cleaver*** (D-MO-5th; Kansas City, Independence, Higginsville)
- Devin Kelsey: Devin.Kelsey@mail.house.gov

◊ ***Congressman Darren Soto*** (D-FL-9th; Kissimmee, Orland, Winter Haven)
- Nicole McLaren: Nicole.McLaren@mail.house.gov

◊ ***Congressman Bobby Rush*** (D-IL-1st; South West Chicago)
- Lauren Citron: Lauren.Citron@mail.house.gov

◊ ***Congresswoman Chellie Pingree*** (D-ME-1st; Portland, Augusta, Waterville)
- Evan Johnston: Evan.Johnston@mail.house.gov

◊ ***Congresswoman Suzan DelBene*** (D-WA-1st; Kirkland, Mount Vernon)
- Kyle Hill: kyle.hill@mail.house.gov

◊ ***Congressman John Garamendi*** (D-CA-3rd; Davis, Fairfield)
- Jacob Jernigan: jacob.jernigan@mail.house.gov

◊ ***Congresswoman Brenda Lawrence*** (D-MI-14th; Detroit, Southfield)
- Zachary Weber: Zachary.Weber@mail.house.gov

◊ ***Congresswoman Terri Sewell*** (D-AL-7th; Birmingham, Montgomery, Selma, Tuscaloosa)
- Earl Flood: earl.flood1@mail.house.gov

SAMPLE LETTER (Customize)

Dear (Include Legislator AND Staff Person's Name):

I am reaching out to you today as a constituent from (Insert City & State). I am a person affected by hemophilia B and a member of the Coalition for Hemophilia B. I am also an advocate for all Americans affected by rare conditions. Continued and unrestricted access to lifesaving, life-sustaining, and life-improving treatments is of paramount importance to the rare disease community. Patient assistance programs offered by nonprofit organizations help many patients maintain coverage and access. Please consider becoming an initial cosponsor of the Access to Marketplace Insurance Act to facilitate bipartisan reintroduction of this important legislation and to provide the patient community with the opportunity to advocate for the bill and raise awareness of barriers to charitable assistance.

[If you received assistance with the cost of treatment from an outside organization, please include something about your experience if you are comfortable.]

The Access to Marketplace Insurance Act would allow people living with rare and chronic illnesses to access nonprofit patient assistance programs without health insurance providers prohibiting the acceptance of premium assistance or cost-sharing assistance on my behalf. The legislation modifies a current CMS Rule that allows health insurance providers to prohibit accepting charitable assistance. This prohibition is viewed by many as a stealthy way to undercut ACA reforms that ended insurers' pre-existing condition exclusions. This bill attracted over 170 bipartisan cosponsors previously, including your office.

Thank you for considering this request. Senator Cramer (Grace Bruno, Health LA) and Congressman Buddy Carter (Nick Lisowski, Health LA) are prepared to drop the bill and are simply awaiting initial cosponsors and continued bipartisan support. On behalf of the local rare disease community, we hope we can continue to count on your office for this support. Please let me know if you have any questions. I look forward to hearing from your office in this regard.

Sincerely,

YOUR NAME
YOUR ADDRESS

CALL TO ACTION:

On February 10, 2022, the CDC proposed new guidelines for prescribing opioids offering more flexibility to clinicians treating pain. We need your support in submitting a formal comment by 04/11/22. Learn more: http://ow.ly/FTcr50HTg6q

MAKING DECISIONS UNDER STRESS: KEEPING THE FLAME WHILE ENDURING THE STORM

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

We live in a society of change and speed—high-speed internet, fast food, express lanes, instant messaging, the list goes on. Life's stressors can also come upon us both fast and unexpected. We may be, one minute, basking in comfort and security but one hiccup can shake up our lives, throwing us into a state of fear and uncertainty. As we dwell in a world of the unexpected, we must prepare to make critical yet objective decisions in the midst of a change, a crisis, or even a crucible.

One can easily categorize a crisis within the struggle that accompanies dealing with bleeding disorders—whether as patients, parents, or caregivers. Many understandably fear that if poor decisions are made, the consequences can be immense whether physically, emotionally, or even financially. Therefore, a question begs to be answered for those caught up in these circumstances: how can I make such decisions when I face this flood of fear and stress? I wish to offer three pieces of advice that may be helpful before significant decisions are made that can ease stress and increase your sense of control.

PRINCIPLE 1: YOU CAN ONLY CRITICALLY THINK OF ONE THING AT A TIME.

Some people pride themselves on multitasking in high-pressured environments. Yes, they may do well with juggling several tasks…but juggling is different than critical thinking. In short, human beings are more often terrible multitaskers, ESPECIALLY under stress. Detail is lost, consequences are considered but not weighed, financial details may be ignored, repercussions ill-thought-out, and other factors can be missed. So, what's the solution? I call it, "Weeding the Garden". Church and statesman LeGrand Richards once prosed:

For every worry under the sun
There is a remedy or there is none.
If there is a remedy, hurry and find it.
If there is none, never mind it.

Consider this analogy. When gardening, there are plants that can produce a yield, offering the grower a bounty of fruits and vegetables. In the same garden, however, there are other plants that offer nothing of value such as weeds and thorns that can choke and destroy a crop. Like a garden, we must clear pressing influences from our minds that serve no purpose. These can come in the forms of petty distractions, saying yes to too many requests, appeasing the social milieu, and other issues that should be put on hold or eradicated altogether.

I find it helpful to draw a line down a blank page, labeling one side "control/important" and the other "no control/not important". Write out your worries, concerns, and issues you're facing and sort them into these respective categories. You'll be amazed what falls under the "no control/not important" category. Focus and act upon that which you have control and is most important.

PRINCIPLE 2: YOU CAN'T DO IT ALONE.

Have you ever cooked with charcoal or briquettes? Nothing beats cooking up vegetables, shish kabobs, fist, hamburgers, steak, or chicken over a charcoaled grill. Once the coals are all fired up after lighter-fluid application, they eventually turn from ebony black to a glowing red and are coated with a light-gray ash. When they get to this point, the fluid and fire that originally set them ablaze have long since died out. So, what's keeping them burning?

Grabbing a pair of tongs, remove a single coal from the grill, and let it sit out in the air for a while. What happens to it? It eventually burns out. Why? While a single piece of coal can sustain its own heat for a while, it won't burn nearly as long as others in the grill. The agent that keeps coals' heat sustained is not just its own internal burning mechanism—it's one another. Try this with a liquid. Set out a brimming mug of hot beverage and then another beverage mug at the same temperature but only half full. The lower-volume mug will cool off much faster than the full mug.

So what's the moral? Simply, humans cannot sustain their own "heat" very long. Although some may possess the attributes of grit and endurance, "burnout" is still more likely to occur without the benefit of support, guidance, and love. We must understand this principle. Trying to make stressful and even life-changing decisions void of support is like trying to keep a match lit in a hurricane. It just doesn't work. Invite others to rally around you (e.g. doctors, family, friends, groups, co-workers, clergy, etc.) in the crucible so you can feel empowered by their heat and support.

All too often, people complain that they have no support system. Let me divulge a little secret that people find hard to believe but actually works. Open your mouth! Try it. Get to know your neighbor, express your concerns with a doctor, or get counseling. When somebody you trust asks how you're doing, tell them the truth. You're struggling. You don't have to go into an enormous amount of detail but express how you're feeling. It may surprise you that others may be going through something very similar. You may have just found your new best friend!

PRINCIPLE 3: YOU CAN'T RIDE A DEAD HORSE.

As life begins to change we must adapt and conform to our new circumstances. With every new major milestone and every crisis, life takes on a different dynamic, necessitating a change in course. A biblical text expounds upon this principle, "When I was a child, I spake as a child, I understood as a child, I thought as a child: but when I became a man, I put away childish things" (1 Corinthians 13:11). As much as we wish that our present circumstances would revert back to what was familiar and comfortable, our present reality demands a different approach.

Say, for example, you inherited a beautiful stallion. From a foal, the horse seemed perfect in every way. It responded to its training, learned commands, and respected your authority. In time, it seemed you recognized its behaviors and cues well enough to anticipate any problems and respond to its needs.

Over many years, the horse gradually declines and dies. Fortunately, the horse left behind offspring and you begin to train one particular horse that seems promising. You feel like you're now an old pro, so you implement the training formula that worked so well from your previous experience. To your shock, the horse is not as responsive and is even downright stubborn! Demanding respect, you keep implementing your "proven" principles of horse training but to no avail. Mounting the horse and attempting to train from the saddle is as easy as canoeing the Colorado River upstream. Finally, you throw in the towel and give up. The horse is sold as a lemon for only a few hundred dollars.

To an extent, all of us attempt to ride a "dead horse", In other words, we assume that one method of practice that once worked governs and applies to all situations. "Johnny was so easy but that Billy! He's as stubborn as a mule! What did I do wrong?" Mental health professionals are a great resource to help us objectively break old patterns that don't work and define new ways of thinking through and navigating the changing tides. It is a liberating feeling when old habits are broken and new, effective actions are taken that help achieve better solutions.

As a closing thought, it's good for us to remember that anxiety and stress are specters that all of us will face. A lack of stress in life is more the exception and hardly the rule. To an extent, stress can be highly beneficial as it can serve as a barometer of how much work we need to put forth to accomplish a goal. However, too much stress skews our ability to "see things as they really are"; thus, diminishing our ability to objectively work towards effective solutions. If the degree of stress you're enduring is making it difficult to make critical decisions, please know that you're in good company. In fact, if you're not experiencing some stress, I'd be worried about you.

If you can keep these three principles in mind as you're making decisions, I feel confident that you will be given the mental and physical energy to be successful.

CHRONIC PAIN: THE OTHER HALF OF THE STORY

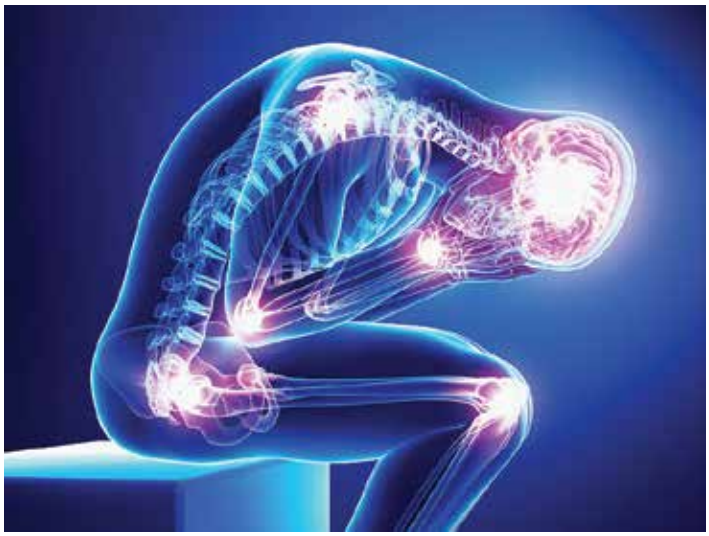
BY DR. DAVID CLARK

Treating chronic pain is one of the most challenging areas in modern medicine. It is also one of the most prevalent problems, affecting an estimated one in five people worldwide. It is a huge problem in hemophilia with many patients experiencing chronic pain as a result of joint damage. Even in people whose bleeding is well-controlled, chronic pain can lead to decreases in quality of life and limitations for employment and participation in physical activity. In spite of its prevalence and importance, we still do not have adequate tools for controlling chronic pain.

Chronic pain is defined as pain that lasts longer than three to six months and has become independent of the injury or illness that caused it. Acute or short-term pain is a natural result of an injury like a joint bleed. It's the body telling us that there is a problem. Once you treat the problem, the pain should go away, but sometimes it lingers and becomes a problem on its own, independent of the original injury.

You might think that your chronic pain is the result of your bones crunching together when you use a damaged joint. That is partly true but often is not the complete story. Studies suggest that chronic pain is due to a dysfunction in the nervous system pathways that carry the pain signals to the brain. Instead of responding to the injury that originally caused them, the pathways seem to get stuck in the "ON" position, transmitting pain signals that are no longer necessary. Why that happens is unknown, but recent research is giving us some clues about where to look.

The nerves that carry pain signals are primarily made up of two kinds of cells, neural cells (neurons) and glial cells (glia). When first discovered, in the mid-1800s, glia were thought to just be the scaffolding that carried the neurons. The neurons were the cells that did all the work. Later discoveries suggested that the glia were the nerve's support staff, feeding the neurons and removing their waste products. In the 1990s, we found out that glia also help the neurons communicate with each other. Now we are discovering that the glia have much more significant functions. This is a continuing story in medicine and biology. Things that were previously considered to have no real function or to just be support structures have later often been found to actually have important roles.



It turns out that when neurons transmit a pain signal from the site of an injury to the spinal cord and brain, they encounter a complex of glial cells that regulate the signal that the brain receives. The glial cell complexes amplify or diminish the intensity the signal, but they can also malfunction. A malfunction can set up an endless loop that permanently sends a pain signal to the brain. We don't know what triggers this malfunction.

Unfortunately, glia are hard to study. We're finding out that they may be just as important as neurons, but they operate in complex systems where it is difficult to pinpoint what is actually happening. We also don't have drugs that target glia – all of the current pain medications were developed to target neurons. One theory is that the pathways in glia have a lot of redundancy. They have so many ways to transmit pain signals that if a treatment blocks one pathway, another will take over. These alternate pathways may be where the malfunction originates, but even if they are not, they make it more difficult to develop treatments. We may need to target many glial pathways in order to stop the pain signal. The body apparently wants to make sure we don't miss a pain signal.

While it may take much more time to really discover how to treat chronic pain, all this research is at least showing that it is a real physical problem. It's not just in the patients' heads (at least not in their imaginations). This recognition is often an important first step – just ask a woman with hemophilia. Now that the hemophilia community is focusing more on women with bleeding problems, there are more and more doctors who understand that women can actually have hemophilia.

[New York Times, The Quiet Scientific Revolution That May Solve Chronic Pain, 11/9/21. The NYT article is easy to read, but if you want to really delve deeply into the science, a recent review article is: Donnelly CR et al., Central Nervous System Targets: Glial Cell Mechanisms in Chronic Pain, Neurotherapeutics, 17:846-860, 2020, DOI:10.1007/s13311-020-00905-7]

TAKE CONTROL TO A HIGH LEVEL WITH REBINYN®

IN HEMOPHILIA B

Clayton, 34 years old, is a pilot and enjoys hiking and camping in his spare time. Clayton lives with hemophilia B.

Rebinyn® elevates factor levels above your normal levels[a]

+94% Factor IX (FIX) levels achieved after an infusion[b]

83-hr (3.5 day) average half-life in adults[a]

With a single dose of Rebinyn® 40 IU/kg in adults with ≤2% FIX levels[a]

Achieve higher factor levels for longer

Compared with Alprolix®[c], Rebinyn® provides

4x greater factor coverage

6x higher factor levels at 7 days

Image of hemophilia patient shown is for illustrative purposes only.

[a]In a phase 3 study of adults, single dose pharmacokinetics were tested during the first Rebinyn® 40 IU/kg dose in 6 adults.

[b]Based upon a 2.34% increase in factor levels per IU/kg infused in adults.

[c]Based upon a phase 1 study comparing a single 50 IU/kg dose of Rebinyn® to a single 50 IU/kg dose of extended half-life rFIXFc in 15 adults. To allow for direct comparison between products, all patients received the Alprolix standard 50 IU/kg dose.

INDICATIONS AND USAGE

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

Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat and control bleeding in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for routine prophylaxis or for immune tolerance therapy.

IMPORTANT SAFETY INFORMATION

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

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

Who should not use Rebinyn®?

Do not use Rebinyn® if you:

- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my health care provider before using Rebinyn®?

Tell your health care provider if you:

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

How should I use Rebinyn®?

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

What are the possible side effects of Rebinyn®?

- **Common side effects include** swelling, pain, rash or redness at the location of the infusion, and itching.
- **Call your healthcare provider right away or get emergency treatment right away if you get any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- **Tell your healthcare provider about any side effect that bothers you or that does not go away.**
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

Please see Brief Summary of Prescribing Information on the following page.

Rebinyn® is a prescription medication.

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

Learn more at rebinyn.com and connect with your local HCL

Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536 U.S.A.

 Printed in the U.S.A. US19REB00028 July 2019

novo nordisk®

rebinyn®
Coagulation Factor IX (Recombinant), GlycoPEGylated

rebinyn®

Coagulation Factor IX (Recombinant), GlycoPEGylated

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

Rx Only

This information is not comprehensive.

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

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

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

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

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

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

What is REBINYN®?

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

REBINYN® is used to treat and control bleeding in people with hemophilia B.

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

REBINYN® is not used for routine prophylaxis or for immune tolerance therapy.

Who should not use REBINYN®?

You should not use REBINYN® if you

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

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

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

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

You should tell your healthcare provider if you

- Have or have had any medical conditions.
- Take any medicines, including non-prescription medicines and dietary supplements.
- Are nursing.
- Are pregnant or planning to become pregnant.
- Have been told that you have inhibitors to Factor IX.

How should I use REBINYN®?

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

REBINYN® is given as an infusion into the vein.

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

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

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

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

Use in children

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

If you forget to use REBINYN®

If you forget a dose, infuse the missed dose when you discover the mistake. Do not infuse a double dose to make up for a forgotten dose. Proceed with the next infusions as scheduled and continue as advised by your healthcare provider.

If you stop using REBINYN®

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

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

What if I take too much REBINYN®?

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

What are the possible side effects of REBINYN®?

Common Side Effects Include:

- swelling, pain, rash or redness at the location of infusion
- itching

Other Possible Side Effects:

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

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

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

Animals given repeat doses of REBINYN® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

More detailed information is available upon request.

Available by prescription only.

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

Revised: 11/2017

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

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

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

For information about REBINYN® contact:
Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA

USA17BIO03951 12/2017

novo nordisk®

INHIBITORS IN HEMOPHILIA B

BY DR. DAVID CLARK

In hemophilia, an inhibitor is an antibody that the immune system produces that interferes with or neutralizes the effects of infused clotting factor, factor VIII for hemophilia A or factor IX for hemophilia B. Development of inhibitors is a widespread problem in hemophilia A, where up to 35% of patients may develop an inhibitor. In hemophilia B, only about 5% of patients develop inhibitors to factor IX, but those that do face a much more difficult problem.

No one knows for sure why some patients develop inhibitors. The risk does seem to be higher in patients with nonsense gene mutations or with large gene deletions. Those patients produce either no factor IX or a highly distorted form of factor IX. Therefore, their immune system will have never seen anything that looks like normal factor IX, so it thinks that the infused factor IX must be a foreign protein that could cause trouble. Because of the small number of hemophilia B inhibitor patients, there has never been enough to determine whether the type of factor product or prophylactic treatment increases the risk of inhibitor development.

The level of an inhibitor is measured using a Bethesda assay with the results expressed in Bethesda units (BUs). A sample of the patient's plasma is mixed with a sample of normal (non-hemophilic) plasma and assayed (tested) for factor IX. Simultaneously, another sample of the normal plasma without any patient plasma is also assayed, and the results are compared. If there is a drop in the factor IX level between the two, that indicates that there is an inhibitor in the patient's plasma that is neutralizing the factor IX in the normal plasma. The amount of the drop in factor IX activity indicates how high the inhibitor level is.

Inhibitor levels are called "titers", and they are divided into two categories, which tend to indicate how the inhibitor should be treated. If the patient has a "low

titer", which is less than 5 BU, he or she can often be treated with a higher factor IX dose, enough to overwhelm the inhibitor. That is, the patient's inhibitor will neutralize part of the infused factor IX, but there will still be enough left over to treat the patient's bleeding. However, if the patient has a "high titer" inhibitor, above 5 BU, you can't realistically give him or her enough factor IX to overwhelm the inhibitor. You have to treat the patient by another method.

Some patients develop low titer inhibitors that stay low or even disappear over time. Others with low titer inhibitors see their titer gradually increase over time and become high titer. Some even start out with high titer inhibitors. The high titer inhibitors are the ones that are harder to treat. We don't know what causes the difference.

There is a treatment for inhibitors that has a pretty good success rate for hemophilia A patients with inhibitors to factor VIII called immune tolerance induction (ITI). In ITI, you give the patient frequent high doses of factor over a period of time. This may take a year or more, but in about 70% of hemophilia A inhibitor patients, ITI eventually tolerizes the patient to the infused factor VIII so that his or her immune system quits making the inhibitor antibody. We don't know exactly why this works, but there is a lot of research going on to try to figure it out. One byproduct of the COVID pandemic is that we are learning a lot more about the immune system.

Unfortunately, ITI has a much lower success rate in hemophilia B, with less than half of patients responding. Again, we don't know why. There are also some additional concerns in hemophilia B. First, B inhibitor patients sometimes develop allergies to factor IX. These allergies can be severe and even lead to anaphylaxis, a serious allergic condition that can be fatal. Patients with allergies to factor IX cannot be treated with any factor IX and therefore can't undergo ITI. Another risk is that factor IX inhibitor patients undergoing ITI can develop nephrotic syndrome, a kidney disorder that is not always reversible.

Thus, many hemophilia B inhibitor patients just have to live with their inhibitor, much like all hemophilia patients did before the development of clotting factor products. They face frequent bleeds and chronic joint damage. Fortunately, there are some treatments that can restore clotting in inhibitor patients, but they're only partial improvements. They don't completely restore coagulation, they're a larger burden on quality of life than simple clotting factor products, and they're expensive, but they do help. They're called bypassing agents because they bypass the step in the clotting system that depends on factor VIII and factor IX. They consist of activated clotting factors that trigger other parts of the clotting system to promote the clotting process.

There are three bypassing agents currently available in the U.S. FEIBA is a plasma-derived product that contains several activated clotting factors. NovoSeven and Sevenfact are recombinant versions of activated factor VII. Since FEIBA includes factor IX, it can't be used in inhibitor patients who develop allergies to factor IX, but it is used by some hemophilia B patients without allergies. NovoSeven and Sevenfact are very similar, but some patients may do better on one or the other of them.

Most inhibitors develop in patients with severe hemophilia. Mild and moderate hemophilia A patients can develop inhibitors, but it is rare in mild or moderate Bs. Anyone can develop an inhibitor at any time, but most inhibitors develop within the first 50 exposure days. An exposure day is a day when you receive a factor infusion. The presence of an inhibitor is suspected anytime a patient fails to respond to clotting factor infusions.

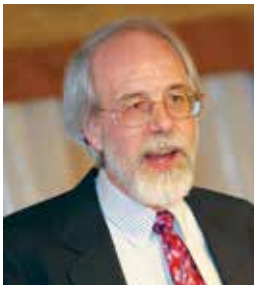
According to the World Federation of Hemophilia (WFH), children should be screened for inhibitors every five exposure days for the first 20 exposure days. Then, every ten exposure days between 21 and 50 exposure days. Then at least twice a year until 150 exposure days. Inhibitors can also develop when a patient experiences intensive factor exposure, for instance in surgery or heavy bleeding. The National Hemophilia Foundation (NHF) recommends that all hemophilia patients be tested annually for inhibitor development. The U.S. Hemophilia Treatment Center (HTC) network provides free annual inhibitor screening to all patients receiving care at HTCs.

Fortunately, there are a number of treatments under development that may improve the quality of life for hemophilia B inhibitor patients. These include improved bypassing agents that have longer half-lives and are given by subcutaneous instead of intravenous injection. They also include rebalancing agents that restore the ability of the clotting system to function without factors VIII or IX. Gene therapy is a question. It's not known whether it will work for inhibitor patients. It could be dangerous if the treatment starts cranking out factor IX in a patient who is allergic to it. Once gene therapy is started, there is currently no way to stop it. On the other hand, there is some animal data that suggests that continuous factor production might tolerize the patient to the new factor.

Hemophilia B patients with inhibitors probably have the hardest time of any hemophilia patients. They suffer frequent bleeds and chronic joint damage. Studies have shown that their quality of life is lower than that of other people with hemophilia. Hopefully, some of the new treatments under development will change that in the near future.

WHERE IS THE FACTOR IX AND *WHAT* DOES IT DO THERE?

BY DR. DAVID CLARK



If you've played our "Are You Smarter Than Your Hemophilia?" game at one of our meetings, you might remember the two questions about what happens to the factor IX that you infuse. First, "After an infusion, how much of the factor IX ends up in the bloodstream?" The answer is: "About half." The next question is: "Where does most of the rest of the factor IX end up?" The answer is: "It sticks to the walls of the blood vessels."

That was about the extent of our knowledge until recently. The best guess for why that happens was that it provides a reservoir for excess factor IX that might be needed for clotting. If too much of the factor IX that is circulating in the blood gets used up in forming clots, more would be available from the blood vessel walls. That's probably still true, but it may only be a part of the picture.

Dr. Darrell Stafford of the University of North Carolina (UNC) and his collaborators have been looking at this wall-bound factor IX and have come up with a lot of interesting results. First, it doesn't just hang on the inside walls of the blood vessel. It actually penetrates through the layer of cells lining the blood vessel and binds to the collagen underneath that is the structural framework of the blood vessel. The cells lining the inside of the blood vessels are called endothelial cells. In most blood vessels, they form a tight layer that keeps components in the blood from leaking out of the vessel. They also have a non-coagulating surface that keeps the blood in contact with the cells from clotting when the vessel is intact.

When an injury breaks a blood vessel, it also breaks open some of the endothelial cells. On the inside wall of the endothelial cells is a protein called tissue factor. Normally the blood doesn't see the tissue factor because it is inside the cells, but when the cells are broken open, the tissue factor comes in contact with the blood. The exposed tissue factor binds to factor VII in the blood, which starts the clotting process.

When the blood vessel breaks open, it also exposes its collagen framework to the blood. The collagen reacts with factor XII and several other proteins to trigger another part of the clotting process. Although there is still some question about exactly how this all occurs, the general idea is that the tissue factor-factor VII pathway gets clotting started quickly, while the collagen-factor XII pathway amplifies the signal to provide more clotting activity than could be obtained from the tissue factor-factor VII pathway alone.

Collagen is a strong flexible protein that forms the framework for many of the organs in the body. Skin and bones are also made of collagen - in bones, it is in a complex with calcium to give the bones their rigidity. These structural frameworks were originally thought to be the main role of collagen, but we've learned that it also has other biological duties. Although medicine and biology have a bad habit of assuming that some things in the body do not have any use, my experience is that almost everything in the body actually has more than one use.

The collagen framework of the blood vessels includes a special type of collagen called collagen IV. It turns out that factor IX binds to collagen IV, but we don't know what it does there. That's the focus of Dr. Stafford's work. At our symposium last fall, Dr. Sidonio presented an overview of some of Dr. Stafford's results. As Dr. Sidonio pointed out there is essentially nothing that goes on in the body that doesn't happen for a reason, probably including factor IX binding to collagen IV.

So what is factor IX doing hanging off of collagen IV molecules in the middle of the blood vessel wall? We know that factor IX is important for clotting. There is some evidence that it is also involved in wound healing beyond just forming the initial clot, and it may have a role in bone health, neither of which is well understood. It could also have other functions that we haven't discovered yet.

Dr. Stafford's group has produced an impressive amount of research that suggests that the collagen-bound factor IX may be involved in clotting. In fact, it appears that the collagen-bound factor in the blood vessel walls might actually be more important for clotting than the free factor IX dissolved in the blood.

However, note that I've used the word "suggests" in the above paragraph. None of this is considered scientifically proven, but the work has provided a number of significant clues as to what may be going on – and it might be a quite different scenario than we have previously considered.

This work may have implications for the extended half-life (EHL) factor IX products. We know that they all behave differently in terms of how readily they move into the vessel walls. Some have about the same distribution between the wall and the bloodstream as standard half-life factor IX, while others remain mostly in the bloodstream without entering the vessel walls. The ones that don't enter the walls give a higher recovery after an infusion. That could be seen as an advantage – you get a higher factor level in your blood that could give you better protection against bleeds.

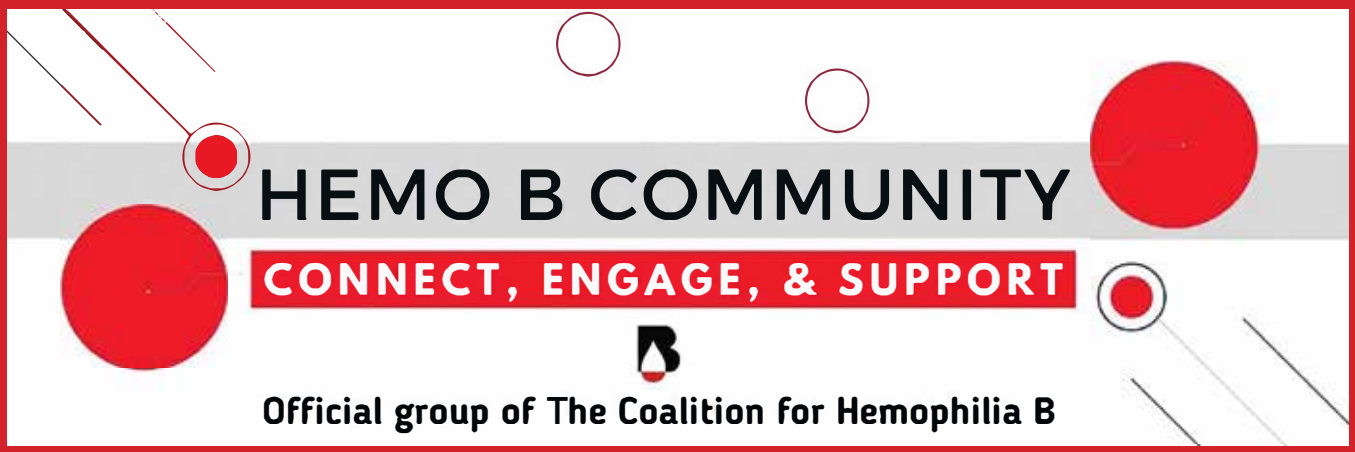
On the other hand, the products that move more easily into the vessel walls could be better if it is the factor IX in the walls that is important. At this point, we don't know which is better, or if it makes a difference. Everyone has an opinion, but we need more actual studies before anything is proven.

This may also help explain some of the differences we see from patient to patient with the EHL products. Most patients do well on any of the products, but we see some patients who have significant factor levels in their blood but still don't clot well. We see others with undetectable factor levels at their trough, who don't bleed. Are these differences due to variations in how the patient's bodies handle the factor IX, or could they be due to other differences like genetically different amounts of other clotting factors or anticoagulants? We don't know, but this field is getting a lot of attention lately.

As often happens in science, new questions arise faster than answers. That's actually good because it means we may be discovering important things. The human body is amazingly complex, and there is so much we don't know. Don't despair. We know that factor IX works, whether we know exactly how or not. The new findings may help us design products that work even better, and help us find solutions to difficult challenges like treating inhibitors. This is an exciting time in hemophilia research.

[Mann DW et al., Haemophilia, 27(3), 332-339, 2021, is a good review of the Stafford group's research. Unfortunately, it is not available for free online.]

COALITION FACEBOOK GROUP!



Join Our Facebook Group! You may already know about The Coalition For Hemophilia B Facebook PAGE, but we have now created a private Hemophilia B GROUP as an opportunity for families to connect, engage, and support each other. We encourage you to share photos from special events, celebrate milestones, and most importantly, build genuine relationships. Please join our GROUP today by searching for "Hemo B Community" on Facebook.

Emerging Therapies

BY DR. DAVID CLARK

Several of the studies reported below were presented at the recent American Society of Hematology (ASH) annual meeting, December 11– 4, 2021 in Atlanta. Copies of the abstracts (summaries) can be read or downloaded for free at: https://ashpublications.org/blood/issue/134/Supplement_1?_ga=2.132024207.1642633.1591024830-1859730944.1554736446.

As Long-Term Acetaminophen Use May Increase Blood Pressure

2/8/22 Acetaminophen (also called paracetamol or Tylenol) is often used by people with hemophilia because unlike many other NSAIDs, it does not cause gastrointestinal bleeding and has been assumed to not cause an increase in blood pressure. However, a recent study suggests that with long-term use acetaminophen may, in fact, cause an increase in blood pressure. For the study, long-term use wasdefined as 4 g of acetaminophen per day for two weeks.

The study was a double-blind (neither the subjects or the physicians knew whether they were getting acetaminophen or the placebo) placebo-controlled (patients got either acetaminophen or a fake tablet) crossover (for the first two weeks half the patients got acetaminophen and half got the placebo; then after a two-week washout, the subjects switched, and the first half got the placebo while the other half got acetaminophen) in 110 patients. In the 103 subjects who completed the study, they found a highly-significant increase of about 5 mm Hg in the subjects receiving acetaminophen.

There are still many unknowns. Even a small increase in blood pressure is usually considered to increase the risk of cardiovascular (heart and blood vessel) disease, but that outcome was not specifically studied. In addition, it is not known what happens with longer-term use. Note that people with hemophilia tend to have an increased risk of high blood pressure already. If you are a regular acetaminophen user, don't panic, but do talk to your doctor about the risk. You should at least have your blood pressure measured periodically. [MacIntyre IM et al., Circulation, 145(6), 416-423, 2022; DOI: 10.1161/CIRCULATIONAHA.121.056015]

Aging with Hemophilia

12/17/21 A research report on aging with hemophilia was recently released from a study funded by a National Hemophilia Foundation Innovative Investigator Research Award (NHF-IIRA) grant. This was the first ever NHF-IIRA awarded to a social worker, Tam E. Perry of Wayne State University in Michigan. Along with co-investigator and social worker Sara Schwartz of the University of Southern California (USC) and other collaborators, they interviewed 27 older people with hemophilia (aged 50 or older) along with eight professionals from various caregiving sectors.

A news release from USC begins with the quote, "The first generation of people with hemophilia to live past 50 are aging in a world that doesn't know what to do with them." This is a free easy-to-read report that contains the texts of interviews with many of the subjects. The interviewees are de-identified, but many of you know Bobby Wiseman, who was quoted in the news release: "I wasn't expecting to be alive past 12, then 15, then 35-ish, and now I'm a fluffy 50 and having to deal with a system that's not ready for me. We weren't supposed to get old." The report gives several recommendations to meet the needs of this cohort of our elders. [USC news release at https://dworakpeck.usc.edu/news/aging-against-all-odds-usc-social-work-professor-investigates-forgotten-hiv-population, which also gives a link to download the report.]

Novo Provides Updates on Concizumab Development

12/12/21 Novo Nordisk is developing concizumab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI). Lowering TFPI activity appears to rebalance the clotting system to help restore clotting in people with hemophilia A or B, with or without inhibitors. Concizumab is a daily subcutaneous injection. At the ASH meeting, they presented two papers on results from their clinical studies. One paper

reported on surgeries in patients enrolled in their Phase II studies. In a total of 50 various surgeries in 20 patients, there was only one post-surgical bleed classified as severe with 13 bleeds classified as mild/moderate. Most procedures were dental surgeries – the one severe bleed was during a wisdom tooth extraction. The second paper was a study on health-related quality of life in subjects in the Phase II studies. Most patients showed improvements in most categories. Concizumab is currently in Phase III studies. [ASH abstracts 345 and 1041]

Sanofi Reports Updates for Fitusiran Development

12/12-14/21 Sanofi is developing fitusiran, a drug that reduces the amount of the anticoagulant antithrombin being made by the body. Lowering antithrombin levels appears to rebalance the clotting system to help restore clotting in people with hemophilia A or B, with or without inhibitors. Fitusiran is a monthly subcutaneous injection. At the ASH meeting, they presented three papers on results from their clinical studies. In their Phase III study of inhibitor patients, they were able to reduce the annualized bleed rate (ABR) from 18.07 in patients treated on demand with bypassing agents (16 As; 3 Bs) to 1.67 in fitusiran-treated patients (29As; 9Bs). In the parallel study of hemophilia patients without inhibitors they saw an ABR reduction from 31.0 in patients treated on demand with factor to 3.1 in the fitusiran-treated subjects. In the third study, they showed improvements in health-related quality of life in both types of patients. [ASH abstracts 4, 3197 and LBA-3]

GENE THERAPY

Amarna Is Developing a Gene Therapy for Hemophilia B Using SV40 Viral Vectors

12/3/21 Amarna Therapeutics, a Dutch company, is developing a gene therapy for hemophilia B using SV40 viral vectors instead of the more common AAV viral vectors. SV40 (simian virus 40) is a monkey virus that can evade detection by the immune system and is thus thought to be a good candidate for use in gene therapy. Amarna was originally developing an SV40-based gene therapy to produce factor VIIa to treat hemophilia A and B, with or without inhibitors. They have abandoned that project to focus on gene therapies to produce factors VIII or IX for hemophilia A or B, respectively. The hemophilia B treatment is called AMA005. They recently announced a round of new investment in the company that they expect to allow them to begin Phase I/IIa clinical studies in about two years. [Amarna press release 12/3/21]

Belief Biomed Completes Dosing of First Gene Therapy Patient

1/3/22 Belief Biomed Group, a Chinese company, is developing BBM-H901, an AAV-based gene therapy for hemophilia B. They announced that they have recently dosed their first patient in a registrational clinical study (a study aimed at product licensure). [Belief Biomed press release 1/3/22]

Community Hematologist's Perceptions of Gene Therapy

12/12/21 Although many of us in the hemophilia community have become familiar with the idea of gene therapy, that does not appear to be true of the general medical profession. A group from Cardinal Health and SUNY Upstate Medical School performed a survey of U.S. community-based hematologists and oncologists to evaluate their perceptions of gene therapy and barriers to its adoption into general practice. The results were presented at the ASH meeting.

About half of respondents were not aware of recent results from gene therapy studies. Fifty-three percent stated that they expect gene therapies to be administered and managed by academic centers (presumably including many HTCs) to which they would refer their patients, and that the therapies would not directly affect their practices. Many cited cost as a major barrier to adoption, specifically cost limitations by payers (49% of respondents), cost to patients (46%) and prohibitive costs to hospitals and practices (37%). Responders also cited concerns about real-world outcomes (18%) and long-term complications (13%). Despite these concerns, most responders reported a moderate (39%) or high (43%) comfort level with prescribing gene therapy, if it were reimbursed. [ASH abstract 2986]

Freeline Presents Long-Term Follow-Up Data from Phase I/II Study

12/13/21 Freeline Therapeutics is developing FLT180a, a gene therapy for hemophilia B. At ASH, they presented results from ten patients in their Phase I/IIa study receiving a range of AAV doses of FLT180a for up to 3.5 years. Except for one patient who lost factor IX expression after an episode of liver inflammation, they found a dose-dependent relationship (the higher the dose, the more factor IX produced) of factor IX production up to 280% of normal in the high-dose cohort. They have selected a mid-level dose for the next phase of studies that is expected to give levels in the normal range (50 – 150%). They found no spontaneous bleeds in any patients with greater than 50% factor IX levels, but one patient had a traumatic bleed even with a level of 57%. Other patients also experienced short-

term liver inflammation which was successfully treated with steroids. Short term steroid treatment will now be standard in all additional patients. Freeline is now beginning a Phase IIb dose confirmation study with the selected dose. [ASH abstract 3967 and Freeline press release 12/13/21]

Pfizer Presents Five-Year Results and Schedule Update

12/13/21 Pfizer is developing fidanacogene elaparvovec, a gene therapy for hemophilia B. At ASH, they presented follow-up results from fifteen patients in their Phase I/II studies for up to five years. None of the patients developed inhibitors or any other serious adverse events, and the treatments were generally well-tolerated. Three patients had required steroid treatment due to liver inflammation in the initial study. Average factor IX levels ranged from 22.8% the first year to 19.8% in year five. Four patients underwent six surgeries successfully – two significant surgeries, an appendectomy and a lumbar discectomy, were performed without additional factor IX infusions. Pfizer is currently conducting a Phase III study with results expected in early 2023. [ASH abstract 12/13/21 and Pfizer press releases]

CSL/uniQure Present Phase III Gene Therapy Results

2/4/22 CSL Behring and uniQure are developing etranacogene dezaparvovec (EtranaDez), a gene therapy for hemophilia B. At the annual meeting of the European Association of Haemophilia and Allied Disorders (EAHAD), they presented the final analysis of data from their Phase III study of EtranaDez. In the 54 subjects, who were all males with severe or moderately severe hemophilia B, they found average factor IX levels of 39.0% at six months after treatment and 36.9% at 18 months. They found a reduction in the average annualized bleed rate (ABR) from 3.65 during the six-month lead-in study to 0.83 at month 18. Ninety-eight percent of subjects were able to discontinue prophylaxis, and the total annualized factor IX consumption was reduced from 257,339 IU/subject/yr to 8487 IU/subject/yr. The treatment was effective in subjects with pre-existing antibodies to AAV.

The treatment was generally well-tolerated with 80.4% of adverse events considered mild. One 77-year old patient died from causes unrelated to the treatment. One patient developed liver cancer, but that was also deemed unrelated to the treatment after extensive study. There were no signs of inhibitor development. CSL plans to submit U.S. and European license applications by June 2022. [CSL Behring press release 2/4/22 and uniQure press release 12/9/21]

In Remembrance

Fred Murray Blood

October 5, 1935 - December 5, 2021

Fred Blood, a Coalition community member, passed away on December 5, 2021, at the age of 86 in Woodstock, Vermont. Fred enjoyed volunteering with the Vermont Association for the Blind, working as the parking meter enforcement for the local police department, serving with the American Legion, delivering for Meals on Wheels, and serving with the Masonic Lodge. He and his wife Stella Eileen, are the parents of three children – David, and Kenneth Blood, and hemophilia B community member Pamela Williams.

Two pieces of advice he left to his children were: “Everyone is only a stranger until you meet them; then they become a friend,” and “You are never truly lost. All roads lead somewhere, and if you come to a dead-end, you just go back the way you came and try a different path.” Fred will be deeply missed by everyone who knew him.

In Remembrance

Val Biaz

March 20, 1958 – December 30, 2021

"Val was one of the greatest leaders and advocates for the bleeding disorders community. He worked tirelessly to advocate for safer products, along with a better standard of care for all patients. He was not only a true leader but a true inspiration to everyone he met, he was a friend who will be missed but his legacy will live on."
— Wayne Cook, President, The Coalition for Hemophilia B

The Coalition for Hemophilia B joins the entire bleeding disorders community in mourning the loss of outstanding activist and leader, Val Bias 63.

Among his many roles, Val served as the long-tenured CEO of the National Hemophilia Foundation where among his numerous accomplishments was the transformation of NHF's nationwide chapter network. Val also significantly advanced the community's legislative advocacy efforts through the creation of *Washington Days,* which continues to bring hundreds of people affected by bleeding disorders to educate and influence elected legislators every year. His efforts were critical in lifting lifetime health insurance caps through the *Affordable Care Act (Obamacare)*. In his younger days, Val was also instrumental in achieving the passage of the *Ricky Ray Hemophilia Relief Fund Act,* which provided compassionate payments to people with bleeding disorders who contracted HIV/AIDS during the height of the tainted blood crisis.

At the request of Val's wife Robin Bratton-Bias, who survives him along with their son Langston, and many relatives and dear friends, the National AIDS Memorial has established a *Val Bias Memorial Fund* in his honor. Those wishing to contribute to the fund can visit https://aidsmemorial.networkforgood.com/projects/150606-val-bias-hemophilia-memorial-fund.

Thoughts from Bobby Wiseman:

Shortly before learning Val had transitioned, I learned of the passing of the Episcopal Assistant to the Presiding Prelate of TFAM (The Fellowship of Affirming Ministries). At the time, I was on a cross-country road trip with my brother/road dog and his kiddos (five boys ages 5–12) in the process of checking in a hotel to escape a freak winter storm.

I returned a couple of phone calls relating to both transitions. A few folks I spoke with from the community stated something to the effect of "Everyone is worried about you." My reply was, "I am fine." Yes, I had a moment of sadness and sorrow balanced by comfort. Many know I have known Val since I was 5-years-old. Yes, I met him at summer camp – Camp Hemotion. Yes, as I progressed in summer camp, both myself and Craig G. were the Junior Counselor for the 5-year-old boy cabin and Val was the Camp Director with Todd Smith.

As I grew up, Val was there both at camp and outside of camp in many, many various activities – bleeding disorders and non-bleeding disorder events. He was keen on education, keen on speaking with both those who agreed and disagreed with a viewpoint or stand. In many conversations with Val, the key takeaway was that whatever challenge you may have, do not let it define nor confine you. Do not be "so stuck" in the thought or practice that there is only one way to do something.

He was a husband, father, brother, cousin, godparent. He was a director, organizer, developer. He knew he could not do all things, so he delegated. I had no clue how to even start some of his delegations. When I would ask the "how-to" questions, he would maybe offer one piece of information to "assist" and gave me a deadline to report back on progress. In the process of reporting, the organized delegator asked key questions forcing me to think differently,

to look at situations from the context of a wide variety of folks, and provided me with safe personal and professional development. Oh yes, he did have that voice of chastisement balanced by love, admiration, respect and trust.

I have many memories of meetings and phone calls where it took a long, long time to reach an agreement or consensus. I can remember many late-night conversations at camp or retreats where the language was ummm, interesting and funny as all get out. I can remember, despite joint-related issues, Val would drop to the floor and under a chair when "the bat" would fly about in the dining hall during our nightly meeting. I can recall campfires, HFNC Board meetings, cookouts at the Bias home. I can recall many, many things about Val.

He was steadfast in "getting the work done." He was steadfast in making trailblazing decisions and actions for the local community (Northern California), California, and the national and international bleeding disorders community. In my opinion, he was not one to lavish in the limelight. He believed you do not need to be in the limelight to do the work. He propelled so many folks in private, one-on-one conversations. He loved music and living life. I can recall so many conversations regarding raising a family. I fondly remember the look on Robin and Val's faces when Langston came home. I recall many moments when I wanted to cuss him out. And yes, there were times we both did that! Not gonna lie about it! I can recall when there were some significant health issues where I was on a death bed, and he was there; not just for me, but also for my Mama. I can recall talking about food recipes, him telling me to be true to myself and not to let the opinions of others or even the opinions of myself limit or hamper me.

What has hit and settled in my brain and heart regarding the transition of Val:

If By Chance

If by chance, I have the opportunity to sit and talk with someone, I will.

If by chance, I can make an impact on community, I will.

If by chance, I can pour into others, I will.

If by chance, I can tell my truth, I will.

If by chance, I can be the best me, I will.

If by chance, I can be whatever I choose or want to be, I will.

If by chance, I can develop a program or service that all do not see value in, I will

If by chance, I can enjoy life to its fullest while doing no harm, I will

If by chance, I can be a student, I will.

If by chance, I can be a teacher, I will.

If by chance, I can learn from my mistakes, I will.

If by chance, I can provide an opportunity for learning, I will.

If by chance, I can find commonality in the midst of chaos, I will.

If by chance, I can give and show love, I will.

If by chance, I can understand the real heart of what is being talked about, I will.

If by chance, I can be accountable and responsible, I will.

If by chance, I can be a person of faithful relationship, I will.

If by chance, I can try my best and say where I was wrong, I will.

If by chance, I do not know the answer or solution, I can and will tell the folks I don't know what the next steps are, I will.

If by chance, I need to apologize, I will.

If by chance, I want to live my best life as I define it, I will.

If by chance, I can make a difference in the life of one's self, I will.

If by chance, I can smile in the midst of sadness, I will.

If by chance, I can laugh, I will.

If by chance, I can dance like there is no tomorrow, I will.

If by chance, I can live out my desires and dreams, I will.

If by chance, I want to make an impact, I will.

I had the chance to experience and witness awesome friendship, love, and professionalism of Val. If by chance, I can continue actively living out the vision that has been poured into me, I will. Yes, there is a loss with Val no longer physically present with us. However, the seeds have been planted for so many people to live out their "if I can" not just from Val, yet many, many people.

Rest well social-justice warrior. Rest well knowing what you have built and built upon will continue. Rest well and know that "if by chance" will continue!

women & girls with hemophilia

articles to support, educate, and empower

#morethanaperiod

Bridging the Gap Between Diagnosing and Treating Women – Stormy's Story

BY RENAE BAKER

"I knew my family had something wrong with their blood, but I didn't know what. Everybody bruised and had bleeding issues, and they just called themselves *free bleeders*," Stormy says with the kind of laugh you might hear at a big family dinner. She didn't have any idea her frequent pain and swollen ankles had anything more to do than with her childhood play. "We lived in the country, so I was always falling into holes. I remember limping around a lot, but it was just like, 'Whatever!'"

Stormy grew up in North Florida. She was one of seven daughters. Their mother hemorrhaged after every birth and ended up back in the hospital. Most of the daughters had horrible periods and early hysterectomies. Stormy had her hysterectomy at 33. "When I look back, I can remember, every time I would lose a molar, I'd wake up, and my hair would be plastered to the pillow from the dried blood. I thought, 'That's just what happens.' I remember my very first period," she laughs. "I was at Disney. I was exhausted, not feeling good, and we had one of those old-fashioned photos taken. I was just like, [she makes a miserable face which does not reflect the "magic" of Disney.]"

Stormy goes on to reveal that her periods were always bad. Even on hormone pills, she never bled for less than ten days each month. Forty-five-day stretches of bleeding were not uncommon. As she looks back at the extensive periods, pain, and gum bleeding, and continually being told that these were "just female things" and that "some women just bleed like that," Stormy finds herself infuriated. Her severe endometriosis led to a laparoscopy and blood transfusion at the age of 18. After a few more of these procedures, her doctor put her on male hormone injections and a two-year course of a contraception pill that would put her into a state of menopause by the age of 21. "Back then, I was so desperate and so young that I didn't ask questions. If they said, 'Do it,' I said 'OKAY!'"

Stormy married Craig, and they moved to Germany, where he was stationed in the US Army. While still newlyweds and living overseas, their doctor advised them that, because of Stormy's endometriosis, they would have to act quickly if they wanted to have children. That wasn't their plan, but they followed the doctor's orders. After two miscarriages, their daughter, Whitney, was born. Five years and a third miscarriage later, their son, Collin, was born in 1999. After yet another miscarriage and enduring almost non-stop bleeding and excruciating pain, she and Craig decided they were done trying for more children, and Stormy had her hysterectomy. "I always said I wanted six kids, and I got six; just not all here on earth with me."

Once Collin was diagnosed, at age three, with mild hemophilia B, Stormy became very involved in the bleeding disorder community. She homeschooled Whitney and Collin and was a very busy mom. During this time, she noticed her ankle swelling to an alarming

size. She saw her doctor about it but was told, "You're a busy mom. In light of your son's hemophilia, you're obviously not taking care of yourself."

Stormy proceeded to consult an orthopedic doctor. He showed concern over her ankle, but Stormy felt that the extent of his concern was the too-familiar "Whatever." Because of other joint pain she was having, the doctor sent her to a rheumatologist. This specialist told her that all she needed was Prozac; that there was nothing wrong with her. Stormy knew that this was not a result of clinical depression, and she knew she was not merely imagining this pain, but the doctors were not taking her seriously. One day, a nurse friend looked down and said, "Look at your ankle!" Stormy said, "It's just always like that." "Does it hurt?" her friend asked. "Yeah!" Stormy exclaimed. It was this friend who validated Stormy's pain by telling her that it was not normal. She recommended that Stormy see a podiatrist whom her husband had just seen. Stormy saw this doctor and learned that, for three years, she had been walking around with three tears in her tendon.

When Collin started going to camp, Stormy started volunteering on a leadership team in the Georgia bleeding disorder community and at camp. "I always liked working with the girls," Stormy said, "and one year, I remember telling them, 'You've got to start taking care of yourself. You've got to start learning to infuse,' and somebody said to me, 'Well, have you ever been tested?' I said, 'No! I'm a carrier!'" Spurred on by this question, Stormy went to see a private hematologist at Emory University Hospital. This doctor told her that her levels were around 42. Stormy said to her, "Oh, so I have hemophilia!" The doctor said, "I don't know!" Stormy said, "Don't worry about it. I'll take care of it," and called the HTC. She was seen at the HTC and was diagnosed with mild hemophilia. Stormy remembers her excitement over finally being taken seriously. "I thought to myself, 'Yes! They didn't say I'm a carrier!'"

But that excitement would soon turn to frustration and hurt as the doctors refused to prescribe factor for her to have on hand when she needed it. "I said, 'Look, y'all recommend that my son have some on hand. I need some on hand just in case, too.' When something would happen to me that I knew I would infuse my son for, I would call them and they would say, 'It's not traumatic. You don't need to worry about it.'"

One day, Stormy suffered a bad ankle twist. The doctor had her infuse, and she noticed that her hip and neck felt better. She called her doctor with the news and asked if she could try infusing a few times to see how it might help. The doctor dismissed her by saying "It's the placebo effect. There's no reason other parts of you should feel better." Stormy grows visibly frustrating, recalling that moment. "That pissed me off so bad! I said, 'Don't tell me it's the placebo effect! If it feels better, it feels better. I know!'"

That incident lit a fire under Stormy. She thought, "This is wrong! If this is happening to me, how many other women is it happening to?" Stormy asked to see a different doctor. The new doctor told Stormy that she was just getting older and that it was probably arthritis, and she shouldn't be infusing. Stormy asked, "Does factor help arthritis?" The doctor said, "No." "Then it's not arthritis!" Stormy insisted. "I'm sure I have arthritis, but this is a different pain, and when I infuse, it helps!" In a conversation with an HTC nurse, Stormy asked, "Could I be having microbleeds in my hip?" The nurse answered, "To have a microbleed, you would've had to have an injury." Stormy said, "Well, I've had two babies. That was pretty traumatic on my hips!" Stormy continues, "It got to the point where instead of calling them beforehand, I would infuse when I thought I needed to and then let them know."

After a couple of surgeries, for which Stormy was allowed to infuse, she found her third doctor. "I was really excited because she agreed to let me start doing prophy before I went hiking or did stuff I knew aggravated my hip or ankle." But once again, Stormy's excitement was short-lived as the prophy was never prescribed. Stormy called and was told by the nurse, "Well, your level was 60 this time. She's not giving you factor." Stormy challenged the nurse, "So last year, I had a genetic condition, but this year I don't?" "Well, your levels weren't high enough. You shouldn't be having any bleeding." "But I am!" Stormy persisted. "I know the difference." The lack of respect Stormy was feeling was creating a mounting sense of indignation. She started calling private doctors until she found one who would see her. It is a sad commentary on the state of women's health care at the institutions which specialize in bleeding disorders, but Stormy has left the HTC and is now being treated by a private doctor. "I realize that this doctor probably doesn't know enough about hemophilia, but I do, and I feel confident that, as long as he'll give me factor, I can take care of myself. I'm on prophy now, and it's amazing!"

Stormy's mission is to raise awareness of the unfair treatment, (make that 'non-treatment') that women are

receiving simply because they are not males.

The incongruity is made even clearer when Stormy remembers back to her days volunteering at camp. If she would twist her ankle, the pediatric doctors on hand at these programs would examine her and tell her that she needed to infuse right away. "I would infuse, and it would help, but my adult doctors at the HTC wouldn't let me infuse."

Stormy recently decided to check back in with the HTC, during one of Collin's visits, to let them know her progress now that she was on prophy. The doctor said to her, "I hear you're on prophy now, so why are you here? What do you want?" Stormy responded, "The same as my son gets comprehensive care, I think I deserve comprehensive care." The doctor looked her in the eyes and said, "Well, I can tell you right now that no 'milds' get prophy from this office. So, if you have a doctor doing that for you, I don't know why you're here." Stormy started to say, "Do you want to talk about it?" when he cut her off with, "I will give you a prescription for PRN (as the situation demands,) but I will not give you a prescription for prophy." Then he proceeded to talk about taking her levels, but this time she cut him off – "There's no need for me to be seen here." He said, "Well, we'll be here for you if something major happens or if you need guidance." She stood up and said, "There is no need for me to be at this office anymore. And by the way, my son, who is mild, is sitting in the room next to me, and he's on prophy. He has been allowed, his whole life, to infuse however he wants to. If he wants to infuse every day of his life, he's never been told 'no.'"

This recent exchange took place only a week after a leading hematologist had encouraged Stormy by telling her that a larger number of hematologists are showing more curiosity about female bleeders and educating themselves about it. He didn't think she would have a problem getting the prescription from her local HTC. But he was wrong. "It was like a gut punch," Stormy admitted. "It's hurtful. You feel like you don't matter as much as a man. "

Another aspect Stormy would like hematologists to consider: "If it is the placebo effect – so what? It's not their insurance. They aren't paying a dime for it. In fact, they're making money off of me." She realizes doctors feel obligated to caution that clots are an increasing danger especially as women get older. Stormy sought out the opinion of a well-respected former hematologist who shared her expertise with her. "Yes, as you get older, the chances of a clot may go up, but we have watched women in labor, and their levels will go up to 200-250, and they're not clotting. So even if we put you at 100%, you should be okay." Stormy let her know that when she infuses, she feels better all over. The doctor said, "We've been hearing that, especially with older men. The bottom line is that we don't know what all factor does in your body." So maybe factor is helping more than we know.

Stormy echoes a phenomenon heard from other women in the community. "We go through cycles of health. Over time, we go, go, go to doctors, and it feels like we're getting nowhere. We're told it's all in our heads; that they can't find anything wrong with us, that we should just exercise more. And then we just stop going because we're tired. They're not doing anything for us, so why go?" Stormy has been keenly aware that she has joint problems which would only get worse without proper treatment. The stonewalling of her treatment infuriated her. "Then it pissed me off that other women aren't getting treated. And then I started to think about other young girls, my grandbabies maybe. What's going to happen to them?"

The more involved she has become in advocating for female bleeders, the more grateful Stormy is to the men who went through the bad blood hell. Because of them, her son is getting better care. Likewise, "If I have to go through hell so that my grandchildren or these young girls don't have to live in the same pain and deal with what I've had to deal with, then that is what my goal is. That's why I join every committee I can. That's why, even if it's not for me, I'm learning to say to doctors, 'This is what I'm seeing in the community. These are things that need attention.'" Stormy continues, "As hard as it is, I'm beginning to tell national program leaders that, even though they say that they have programs to support women, the reality is that women walk away disappointed."

It's hard, but Stormy realized that this is what she must do. "You've gotta do hard things, or you'll never see change. Even if I wasn't getting treatment, I'd still be doing what I'm doing, because I don't ever want anyone to have to hurt as I've hurt. Just because a woman is diagnosed, doesn't mean she is being treated properly."

Stormy started the hashtag #morethanaperiod because it seems all that the doctors want to talk to women

bleeders about is their period. “A lot of us don’t even have our periods anymore, because our bleeding disorder forced us to have a hysterectomy!” Stormy is calling for doctors to stop talking only about girls’ periods all the time. She believes that the girls who are being treated are only being treated for their periods. “We have joint bleeds. We have muscle bleeds. We have spontaneous bleeds. Stop talking about our levels and start treating our symptoms.”

Women in the community go from hopeful to frustrated to hear certain doctors encouraging prophylaxis for female bleeders, only to hear their doctors give them a hard “No.” There are women who have considered flying halfway across America to get the care they need. Why should that be in this day and age? “It would be different if it were a disease where there were only specialists here or there,” Stormy states, “but every state has a treatment center. There are some women who have told me they take their son’s factor in the hospital parking lot before a surgery, because they are afraid they’re going to die! Let that sink in.”

Stormy became aware of The Coalition for Hemophilia B when Collin was around 15. “As a woman, just being able to know that there are people who believe us, who listen, who support us, who give us opportunities to get involved and to be heard is life-changing,” Stormy says. “I look at myself as this small, country girl. I never in a million years dreamed that I’d be standing on a stage, giving a presentation and an award to a doctor!” She is referring to last March’s Eternal Spirit Award, which she was honored to present to Dr. Sedonia at the Coalition’s annual dinner. Although she has never considered public speaking one of her gifts, (she says it makes her so nervous that she nearly throws up,) her drive to help others is garnering her more and more opportunities. Those nerves, at these speaking engagements, might be considered part of the “hell” through which she is willing to go to help enhance the treatment women with hemophilia receive.

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.

New Hemophilia B Classifications for Women

BY DR. DAVID CLARK

Now that we are recognizing women can also have hemophilia, it is important to define diagnostic criteria that apply to them. This is needed for insurance coverage of their treatment, but also for their own recognition and self-respect. Imagine if you had to limp around on your damaged joints from doctor to doctor to try to get one to take you seriously. Too many women in our community have had just that experience. Now, we can give names to their problems.

An international group of twelve hemophilia treaters and patient advocates has taken on this project under the Scientific and Standardization Committee (SSC) of the International Society on Thrombosis and Haemostasis (ISTH). The project was mainly supported by the Coalition, NHF and HFA from the U.S., as well as other hemophilia organizations around the world. There was no commercial support. The results were published in an article in the Journal of Thrombosis and Haemostasis on July 31, 2021. [See the complete citation at the end of this article.]

The results are shown in the table below for hemophilia B. (Hemophilia A uses the same categories.) For factor IX levels up to 40%, women receive exactly the same diagnoses as their male counterparts. They are classified as severe/moderate/mild based on their factor levels. Above 40%, the tables are turned. Men with factor levels above 40% are not considered to have hemophilia; however, women who are carriers can still have a bleeding diagnosis.

The first thing to recognize is that the term "carrier" is now being returned to its proper definition. Carrier is a genetic description – it does not define a bleeding disorder. A woman is a carrier because she carries a mutated factor IX gene on her X chromosome that she can pass on to her offspring. She may or may not have a bleeding disorder. Carriers can have normal levels of factor IX.

Next, we need to talk about the 40% upper limit for hemophilia. That is the international standard. In the U.S., we commonly use 50% as the upper limit for hemophilia and the lower limit for the normal range of factor levels. We recognize that men with levels up to 50% may still have mild hemophilia and may need treatment. In the rest of the world, men with levels of 40 – 50% don't have hemophilia.

This gets more complicated because we know that women can bleed even at levels up to 60%. We don't

Factor IX Level, % of normal	Diagnosis/Classification	
	Women	Men
Less than 1%	Severe hemophilia B	Severe hemophilia B
1% to 5%	Moderate hemophilia B	Moderate hemophilia B
More than 5% to less than 40%	Mild hemophilia B	Mild hemophilia B
40% or more	Symptomatic carrier of hemophilia B (If you are genetically a carrier and have bleeding symptoms.)	Normal
	Asymptomatic carrier of hemophilia B (If you are genetically a carrier but do not have bleeding symptoms.)	

know why they still bleed, but the study authors have recognized this and given women two more categories. If a carrier has a level above 40% but does not have bleeding symptoms, she is classified as an "asymptomatic carrier." However, if a carrier has a factor IX level over 40% (with no upper limit) but still has bleeding symptoms, she is classified as a "symptomatic carrier." This fuzziness in the over 40% levels could lead to situations where it is now the men who could have trouble getting treated. Going by the international classification, a man with a 50% factor IX level would not have mild hemophilia, even if he has bleeding symptoms. Yet, if he were a woman with 50%, she would be a symptomatic carrier who might have a better chance of being treated.

In addition, all of the categories are just approximations. It's the best we can do with our current state of knowledge. We know that up to about 15% of people (men and women) do not bleed according to their category, mild, moderate or severe, as determined by their factor level. For instance, some people who are classified as severe bleed like moderates. Some people who are classified as mild, bleed much more heavily.

Another term seen sometimes is "obligate carrier." This is also a genetic description, not a bleeding diagnosis. If you are genetically female (have two X chromosomes) and your father has/had hemophilia, you are an obligate carrier. That just means that you carry (have inherited) your father's mutated factor IX gene. That's just how the genetics works. You may or may not bleed. (Of course, the genetics can always mess up – that's how we get hemophilia in the first place. However, it is extremely unlikely that when your father passes his mutated factor IX gene, there is another mutation that actually fixes the gene.)

One interesting point in the article is the estimate that for every male with hemophilia there are 1.6 female carriers. Since many of these female carriers might have bleeding problems, there may actually be more women with hemophilia than men. Tell that to your doctor who says women don't get hemophilia!

This is all based on averages – no one is average! That's why you always have to talk to your doctor about your individual case. No one should bleed, no matter what their factor levels are.

[van Galen KPM et al., A new hemophilia carrier nomenclature to define hemophilia in women and girls: Communication from the SSC of the ISTH, Journal of Thrombosis and Haemostasis, 19(8), 1883-1887, 2021. DOI: 10.1111/jth.15397. The easiest way to access this on the internet is to type "DOI: 10.1111/jth.15397" into your browser. This is an open-access article – you can read or download a copy for free.]

FIRST TIME ATTENDEES SYMPOSIUM

THE WILLIAM N. DROHAN SCHOLARSHIP

SCHOLARSHIPS NOW OPEN

NAT LATHROP MUSIC SCHOLARSHIP

LET'S PLAY NINE SCHOLARSHIP

HEMOB.ORG

IXINITY®
coagulation factor IX
(recombinant)

Discover more about IXINITY®

Visit IXINITY.com

Scan me or visit
ixinity.com today!

MEDEXUS PHARMA

Sign the 9 is now online!

Now it's easier than ever to make a difference.

Help make IXINITY® available to individuals in the United States who can't afford to be without it. It's easy. Just **visit signthe9.com** and fill out a brief form. When you do, **9 IU of IXINITY will be donated in your name.** While you're there, build your own one-of-a-kind 9. Add your name, share your 9 on social media...and watch the support grow!

Scan me or visit
signthe9.com today!

MEDEXUS PHARMA

IXINITY®
coagulation factor IX
(recombinant)

EDUCATOR JEN FRAKER JOINS THE CHB TEAM

Jenifer Fraker, MA, has recently joined The Coalition for Hemophilia B as Director of Education. Jenifer is a tenured teacher with more than a decade of experience in learning and development. She is a certified instructional designer and holds a master's degree in education from the University of Tennessee.

"The Coalition for Hemophilia B has a long-standing reputation of providing high-quality educational programming for our community. I am honored to be a part of this important organization," shared Jenifer. Jenifer and her family have been actively involved with the hemophilia B community for several years, as two of her children have hemophilia B. Jenifer and her husband Jason live in Tennessee with their five boys: Parker, Isaac, Ben, Nathan, and Andy.

"Jenifer is a welcome addition to our team," stated Kim Phelan, COO of the Coalition. "With her passion, creativity and love of educating, coupled with a fantastic sense of humor, she was the perfect fit for this role, which will take on recreating all of our educational platforms. We are delighted to have her join us!"

EDUCATING TEENS WITH A MIX OF LEARNING AND FUN

BY ROCKY WILLIAMS

The Coalition for Hemophilia B takes seriously our responsibility to provide every segment of the community with the knowledge, tools and support they need to achieve the best possible healthcare outcomes and the highest quality of life. Meeting that primary obligation can be particularly important for the teens we serve with hemophilia B as they transition into adulthood and gradually take over their own self-care. Through several focus groups we learned the perfect recipe by combining education with dose of fun for a wonderful outcome that makes learning, support and education fun and interactive.

The Coalition has created a variety of programs that are presented to teens virtually over the course of the year. Each program combines expert speakers on a variety of key topics including things like basic hemophilia knowledge, the importance of adherence, treatment advances, ensuring healthy joints, mental health, managing finances, and many more. The fun components include knowledge-based quiz games, team building exercises and a variety of others. Individual teen leaders work with us in crafting the programs to ensure they will be engagingto others.

One of our recent programs, held in December, is a good example of our "magic formula." Part of the program featured a fun, team building and role-playing game in which a group of hemophilia B community teen members got together to travel back in time to a "Midnight Express" themed escape room. The teens were put to the test as they solved a variety of puzzles and worked as a team to complete their mission! This fun adventure was followed by a talk and discussion led by Matt Barkdull. Matthew, who has hemophilia himself, is a certified medical family therapist, licensed marriage and family therapist, certified wellness coach, and a licensed financial professional. Matt has worked with many Coalition groups providing life-skills, self-care guidance and much more.

Charlie, one of the teens who planned and co-hosted the December event, said ,"I think that this meeting was great because so many people joined in on the conversation. These events are super cool because we get to connect with many other teens and people in the hemophilia community."

The Coalition for Hemophilia B would like to express our deep gratitude to Sanofi for sponsoring our November and December teen events. None of these programs would be possible without the support of generous partners like Sanofi.

If you are a teen in the community or are the parent of a teen, please consider participating in a future program, or even help to plan one. We would love to have your involvement and hear your ideas!

WINTER WOMEN'S EDUCATION AND EMPOWERMENT RETREAT: WARMED HEARTS AND IGNITED A PASSION FOR LEARNING

BY JEN FRAKER

Held virtually, this year's winter women's retreat was held December 3-5, 2021. Even though we missed giving hugs and high fives, and all the other aspects of gathering in person, the familiar sense of community that only comes when the hemophilia B women get together radiated through our devices. The weekend was packed full of amazing speakers and opportunities for empowerment and growth.

Leading up to the retreat, all attendees had program kits shipped to them. The virtual retreat began on Friday evening when Kim Phelan welcomed and led the women in blowing up the balloons from their kits. Each time she called out life stressors, you had to blow into the balloon. Once all worries were exhaled into the balloon, they were fully inflated and released high up into the air. This release was a great reminder to attendees to "let go" of the things bothering them and focus on themselves and take the time to learn from top experts and bond with others for support. Perfectly timed, Robert Friedman's session, *Humor as a Tool for Good Health*, helped remind participants that finding humor in everyday things can help decrease blood pressure and reduce anxiety.

Also in the program kits, the women found paints and canvases for the next activity. Together, led by *Paint the Town Art Studio*, the women created their unique masterpieces and enjoyed sharing the experience, and discovered that they had some talented artists among them in their community. Art therapy has been proven to improve communication, concentration, and can help reduce feelings of isolation. Friday night ended with some laughter and the promise of more opportunities to learn and grow together.

Saturday morning began with Claire Louise Clifton's session *Mindful Movement and Dance*. Dance has many benefits, including lowering stress, releasing negative energy, and strengthening the mind-body connection. The music and movement that Claire shared were definitely a hit, and her playlist was requested by so many of the participants. Debbie de la Riva LPC, community member and founder of *Mental Health Matters Too*, talked about forming good habits and removing negative ones in an effort to strengthen mental health and increase capacity for personal growth.

After lunch, Dr. Roshni Kulkarni shared a wealth of information and answered many questions about women and girls with bleeding disorders. Dr. Roshni Kulkarni is the founding member of the Foundation for Women and Girls with Blood Disorders and is also on the World Federation of Hemophilia Women with Inherited Bleeding Disorders committee. Dr. Kulkarni is currently Professor Emerita, Pediatrics and Human Development at Michigan State University.

Later in the day, the women chose between two great sessions, *Fact or Fiction: Women and Hemophilia*, led by Carrie Koenig and Jessica Steed, or *Caregivers and Spouses: Raising Resilience*, facilitated by Rebecca Gorde and Danielle Kempker.

After those powerful sessions, Cassandra Starks led a *Tai Chi and Meditation* session. Tai chi is a low impact, slow-motion exercise best described as gentle meditation in motion. As you move, you breathe deeply and naturally. It also addresses key components of

fitness, strengthens your muscles, flexibility, and balance, as well as promotes relaxation and stress reduction through gentle, flowing movements.

Refreshed and ready to keep learning together, the women then had an opportunity to choose between two more great topics. Psychologist Dr. Joel Minden spoke on the benefits of *Cognitive Behavioral Therapy* (CBT) for anxiety and depression, and Dr. Amber Federizo led the session *Menstruation and Intimate Health*.

As the evening drew to a close, the women enjoyed the well-known *Chit Chat and Chocolate* session. One of the Coalition's most intense sessions, moderated by psychologist Dr. Mina Nguyen-Driver with Lori and Jenifer sharing their stories of resilience. Later, participants shared their own stories and created deep bonds over their common experiences living with hemophilia B.

Saturday night ended with some great games, led by Chris Villarreal, the world's best Bingo caller.

The last day of the retreat started with tai chi, a wonderful way to welcome the day, and then moved into a session led by Ellen Kachalsky, LMSW, ACSW. Ellen discussed timely and relevant topics for many in the community, navigating finances and disability issues. Afterward, the ladies had breakout sessions where they could choose from *Self Care* with Kelly Gonzalez or *Relationship Challenges* led by David Rushlow, LMSW. In the last session of the weekend, Catherine Canadeo, CHHC, AADP, guided the women on their journey to finding happiness by focusing on how their emotional wellbeing, proper self-care, and nutrition are tied to their overall physical well-being.

It was truly a wonderful weekend, and the attendees concluded the weekend more empowered and left with a stronger sense of community and support.

The participants shared their reflections about the positive experience after the retreat and here are some of the things they said:

"Thank you so much there were extremely knowledgeable and interesting presentations that explained the science and stress of living with hemophilia B; thoughtful diverse events that were not only educational but fun and relaxing..."

"This year's women's retreat was extra special for me. I found out the week before that not only is my middle child a carrier, but she HAS hemophilia B. I was shocked and confused so the retreat couldn't have come at a better time."

"The retreat was wonderful and a much-needed break from everyday crazy life. It provided time to connect, relax, and learn together. Very grateful for this opportunity and to CHB and sponsor for providing these valuable programs!"

"This was my first time attending as a spouse. I was a bit nervous as I did not know anyone, but I was welcomed with open arms and made new friends. The sessions were so informative I can't thank you enough"

A special thank you to Sanofi for their generous sponsorship of this event!

Winter Men's Education and Empowerment Retreat

BY GLENN MONES

During December 10-12, 2021, men from throughout the hemophilia B community gathered over Zoom for our Winter Men's Education and Empowerment Retreat, our final men's retreat for 2021. The program was made possible by a sponsorship from Pfizer.

Attendees look forward to these events all year long as they offer men in the community a special opportunity to share feelings and experiences, knowledge, information, and camaraderie. Although the event had to be held virtually again to ensure safety during the ongoing pandemic, CHB was able to use the expertise we have developed in offering highly engaging, interactive programs and activities.

The program opened Friday evening with welcoming remarks by CHB President Wayne Cook followed by rap sessions and icebreakers led by Wayne, fellow community member Bill Patsakos, and frequent CHB speaker Robert L. Friedman. Robert is a therapist, counselor, musician, and author who recently received his Ph.D. in Complementary Medicine. Robert then led a session entitled *Humor as a Tool for Good Health*, demonstrating interactively how humor can be used to improve both physical and mental health.

In the evening, participants enjoyed an interactive session on *Sketch Therapy* led by Chicago-based artist and illustrator Amanda Schwarz. All participants received a set of sketching pencils in advance and were taught how to use them. Everyone was then guided in a series of exercises that allowed them to express feelings, thoughts and ideas using only the sketching tools. Ultimately, all attendees learned and got to experience how creativity and art can be used for growth, transformation, relaxation, and healing while still being fun and engaging.

Saturday's program opened with a gentle start under the caring guidance of community member and CHB favorite Rick Starks. Rick led participants in a session of *Tai Chi and Meditation* designed to promote physical fitness and a sense of calm and mental well-being. This was followed by a session called *The Power of Cognitive Behavioral Therapy (CBT): Skills to Work Through Depression and Anxiety*. The session was led by Dr. Joel Minden, a licensed clinical psychologist and author of the book *Show Your Anxiety Who's Boss*. The session went overtime as the participants became very engaged and had many questions. Finally, after feeding mind and soul, it was time to feed the body in a fun brunch session led by Chef Mike Hargett who led the attendees in preparing, and then enjoying, their unique breakfast sandwiches. As an accomplished chef and the only person with hemophilia to have received a double organ transplant, Mike's positive attitude towards his own life and health has inspired many in the community to make their life changes.

Following brunch, the attendees broke into two groups with facilitation by patient liaisons arranged by our sponsor, Pfizer. The first, geared towards men with hemophilia B, was led by Joseph Schuch, who focused on Empowered Tools for Self-Advocacy. The second, which targeted fathers and spouses of individuals with hemophilia B, was led by Mike Sager. Mike focused on dealing with adversity through Strength and Resilience.

In the afternoon, participants attended a session called *Navigating Finances and Disability Issues* led by Ellen Kachalsky, LMSW, ACSW. Ellen is a social worker who has worked with many communities focused on patients with chronic health issues. Her topics included learning about the differences between SSDI and SSI and managing your finances when living with a disability and potentially limited income.

This was followed by a session on *The Mechanics of Physical Intimacy* led by Alice Anderson PT, DPT, MS, PCS, a physical therapist with long-term experience in the hemophilia community. Alice went over some of the challenges presented during physical intimacy for individuals with joint damage or chronic pain. She handled what many consider a very difficult subject with great openness and sensitivity.

The afternoon program ended with a session called *Relationship Challenges During the Daily Grind: Let's Talk About It* led by David Rushlow, LMSW, and Karen Boyd, LMSW. David and Karen are both social workers with extensive experience working in the hemophilia community. The session included exploring how effective communications skills can help to create authenticity in intimate relationships, with further discussion on how to navigate intimacy while coping with challenging times.

In the evening, Wayne Cook led another great rap session followed by some fund, interactive games led by CHB's own Rocky Williams. The men had multiple opportunities to relax, unwind, share jokes and stories, renew friendships and develop new ones.

Sunday's program opened at noon with another great *Tai Chi and Meditation* session led by Rick Starks. Immediately following, the participants were again broken into two groups. The first, led by attorney and popular CHB speaker Donnie Akers, Esq., focused on tools and techniques for maintaining crucial legal and government benefits at different life stages. The second session, called *Leadership: Leading the Intentional Life*, was led by Matthew Barkdull, MS, MBA, LMFT, MedF. Matt is a member of the hemophilia community and a. certified medical family therapist with more than twenty years of experience. Matt's presentation described how to focus on building intentioned habits that have the power to transform character; thus, increasing confidence by "living and leading in crescendo."

The program concluded with a session called *Finding Happiness with Stress Management Tools* led by Catherine Canadeo CHHC, AADP. Catherine is a Certified Holistic Health and Life Coach who helps promote healing behaviors and happy, healthy lifestyles.

The feedback we have been given from many of the participants has been extremely enthusiastic. These are just small samples of the many comments we have received:

- "You make every retreat the best experience. Even though we couldn't be in person you make it so interactive that we felt right in there."
- "The men's retreat was a wonderful program - lots of fun and great information that you can use in your everyday life."
- "My life couldn't be the same without Coalition and my brothers and sisters putting together these amazing events. This year's retreat was so inspiring to me as a dad, husband and friend."
- "I don't like to travel and this retreat offered virtually, gave me the opportunity to learn the most up-to-date medical information regarding hemophilia B."

The Coalition for Hemophilia B expresses our deep gratitude to Pfizer, the sole sponsor of this amazing program. We also send our thanks to the speakers, the staff and everyone who did so much to make the event possible. Many more programs are planned for the entire year so please check our website at www.hemob.org and start registering for the programs that are most meaningful to you and your family.
See you soon!

Pfizer

Gingerbread, Trivia and Fun!

On Saturday, December 18, 2021 right in time for the holidays, the Coalition hosted a Gingerbread House contest and Trivia Night over Zoom for families in the community. The participants showed a lot of creativity and there were prizes for the best house and for the trivia winners. Most importantly, families got to spend a fun evening together with lots of fun and laughter.

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

MEDEXUS PHARMA

PARAGON HEMOPHILIA


First Place Winners!


Second Place Winners!


Third Place Winners!










TREATMENT IS EXPANDING!

BY GLENN MONES

Years ago, treatment options for hemophilia B were very limited, with just a few approved products. That has all changed with the introduction of many new therapies, and the continued development of radically different options. These include subcutaneous products, gene therapy and many others. For patients, these products may mean less bleeding, easier administration, improved quality of life and more. However, with so many new products – and a whole new vocabulary - one of the challenges for patients is understanding what these products are, how they work and who they may be right for.

As part of our ongoing efforts to address this challenge, the Coalition for Hemophilia B hosted Emerging Therapies Roundtable over Zoom on December 8. The program featured Dr. David Clark, the Chairman of the Coalition. Dr. Clark has 35 years of experience in the development and manufacture of plasma and tissue products, including Factor IX concentrates. He has a Ph.D. in Chemical Engineering from Cornell University. Dr. Clark is a frequent speaker at Coalition events and the author of many articles in our newsletters and other publications. He has a reputation for explaining complex subjects in terms everyone can understand.

Dr. Clark took participants on a virtual "tour" of the many products that are either recently approved or now under development. In each case, he explained the technology behind the therapy, its current stage of development, and what patients can expect and consider when the opportunities to try something new are presented. The program also featured ample time for questions and discussion with participants demonstrating a lot of interest and engagement. This was just one of many recent and planned efforts by the Coalition for Hemophilia B to provide our members with the information they need to better understand treatment options as they are developed. Please check our website, newsletters, and social media for more opportunities to learn.

The Coalition expresses our gratitude to Sanofi for sponsoring this very special event.

sanofi

BOOTS ON THE GROUND

SERVING THE COMMUNITY

CALL TO ACTION

- Do you love to travel?
- Want to help run programs?
- Apply today: hemob.org/volunteer

FOR MORE INFORMATION, PLEASE CONTACT US AT CONTACT@HEMOB.ORG

LET'S GET MOVING!

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

SATURDAYS IN APRIL

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

BRING YOUR LAPTOP OUTDOORS

- Special Weekly Guest
- Raffle Prizes

APRIL 2, 9, 23, AND 30, 12PM-1PM EASTERN
JOIN US FOR AN HOUR OF HEALTH AND WELLNESS!

Register Today: hemob.org/upcoming-events/wellness

BROUGHT TO YOU BY The Coalition for Hemophilia B

GENEROUSLY SPONSORED BY sanofi

Let's Play IX

The wonderful and popular game of golf is one of the few sports those living with hemophilia can safely enjoy. Through your generous donations, we are able to provide golf access and education to children within our hemophilia community so they can experience this fun social activity and its many healthy benefits. Funds raised also benefit the B Cares patient assistance program.

Highlights:

- Clinic with professional golfer ***Perry Parker***
- 18 Hole Scramble!
- Contests! Closest to the Pin, Longest Drive, Hole in One
- Breakfast and Awards Luncheon
- Dry-FIT Polo Shirt!
- Goodie Bags!
- Raffle Prizes!

The Coalition for Hemophilia B

LET'S PLAY IX

"LET'S PLAY IX" GOLF OUTING

MAY 18, 2022

8:00 am Registration and Breakfast
9:00 am Clinic with Perry Parker
10:00 am Tee Off
Luncheon and Raffles

18-hole tournament to benefit
The Coalition for Hemophilia B programs

Join the fun and support a great cause!

Contact Kim Phelan
kimp@hemob.org
(212) 520-8272


THE COALITION FOR HEMOPHILIA B
SYMPOSIUM 2022
ORLANDO, FL
MAY 19-22
APPLICATIONS FOR TRAVEL AND
HOTEL GRANTS AVAILABLE:
HEMOB.ORG/ANNUAL-SYMPOSIUM

applygenerationixproject.com

MENTORSHIP

Delivered by GUTMONKEY

Brought you by THE COALITION FOR HEMOPHILIA

Sponsored by MEDEXUS PHARMA

Pfizer

MEN'S RETREAT

EDUCATION | EMPOWERMENT | CONNECTIONS

JUNE 9-12
PHOENIX, AZ

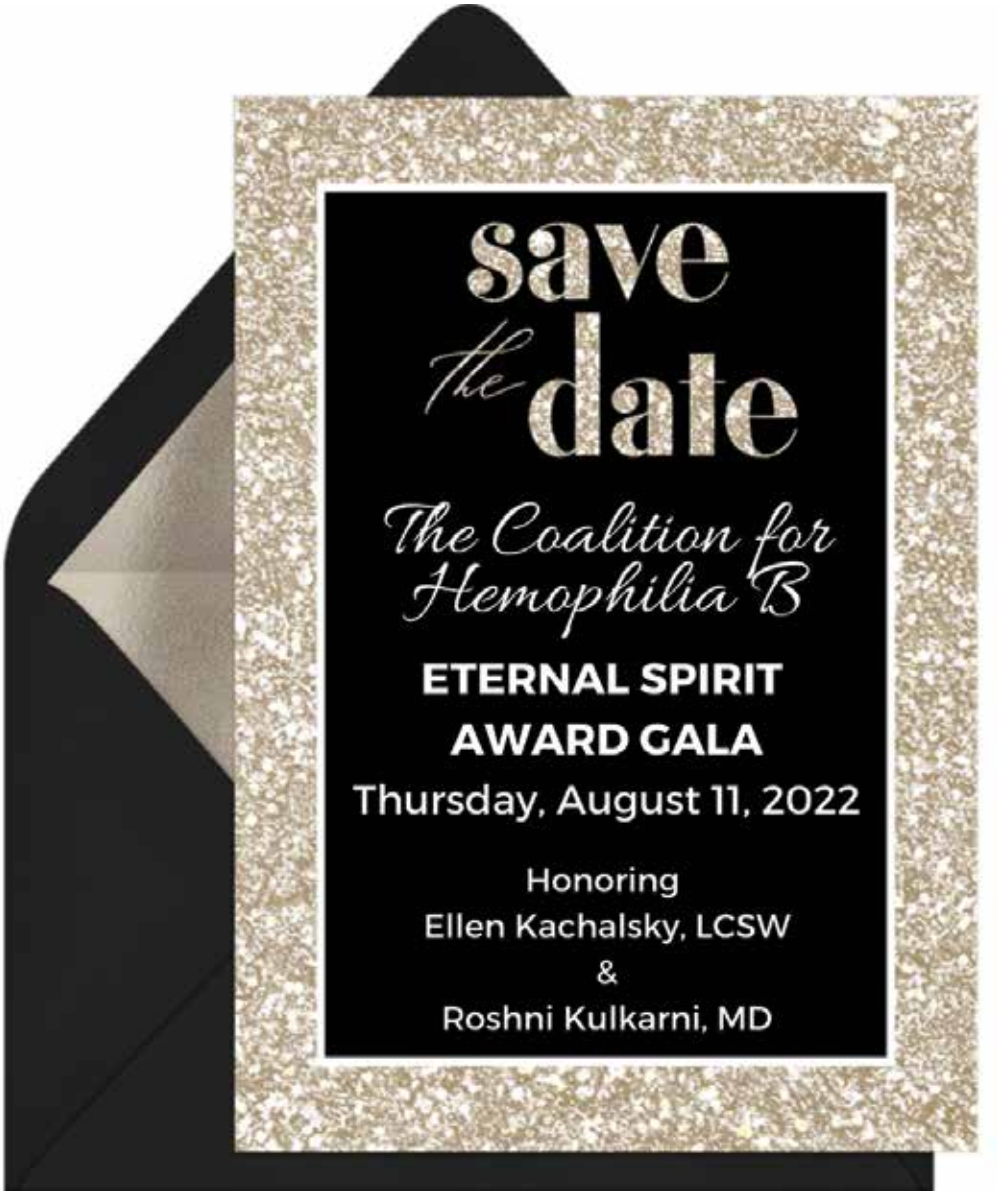
WOMEN'S RETREAT

Pfizer

EDUCATION | EMPOWERMENT | CONNECTIONS

JUNE 23-26
PHOENIX, AZ

CHB's only in-person women's retreat this year- our winter retreat will be virtual only



THE COALITION FOR HEMOPHILIA

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

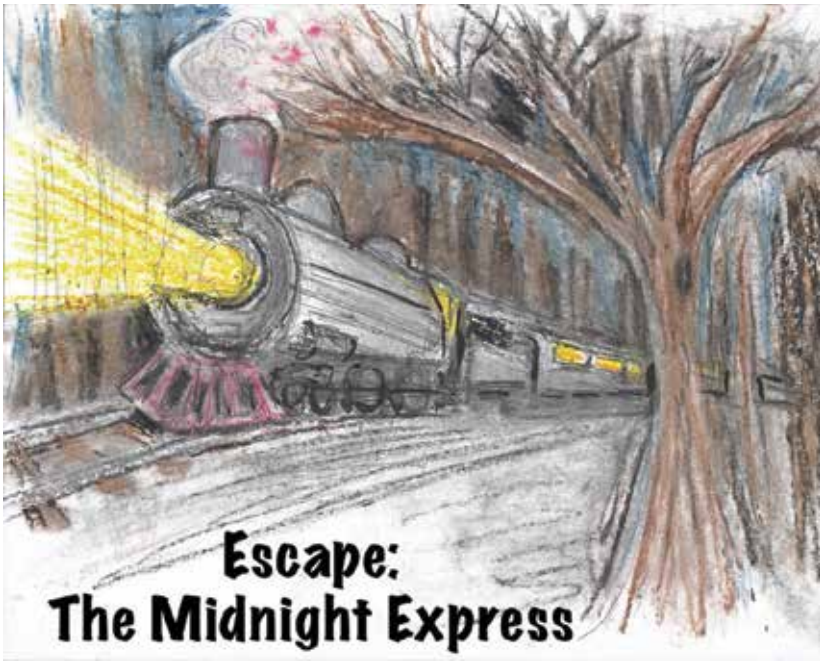
VISIT OUR SOCIAL MEDIA SITES:

Website: www.hemob.org
Facebook: www.facebook.com/HemophiliaB/
Twitter: https://twitter.com/coalitionhemob
Instagram: www.instagram.com/coalitionforhemophiliab
Linkedin: https://www.linkedin.com/company/coalition-for-hemophilia-b/

SCAN ME

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

Traveling Back in Time to Save the President



BY ROCKY WILLIAMS

Imagine you've gone back in time, and it's a chilly winter night in February 1861. You're on a train traveling from Pennsylvania to Maryland, and Abraham Lincoln, who is only a month away from being inaugurated as the 16th President of the United States, is on board. And, he's in trouble.

A group of hemophilia B community teen members got together to travel back in time and save the timeline with a *Midnight Express*-themed escape room! They got in the right mindset by learning about what it was like to live in 1861 with fun facts and true/false trivia.

TRUE OR FALSE? In 1861:

- Milk cost $1 a gallon
- Colorado was organized as a US territory
- Civil War soldiers had an unwritten code of honor to not shoot anyone while they had their back turned

Then we boarded the train! We were detectives sent back in time to sneak into the train car and find out what is threatening the timeline and the presidency of Abraham Lincoln. The teens' detective abilities were put to the test as they solved puzzles and worked as a team to save, protect, and restore the timeline.

After successfully escaping, the evening ended on an even higher note thanks to a dynamic talk hosted by Matt Barkdull, a facilitator and community member.

Belle, a teen who attended the event, said she really liked it. "I think it was fun and it was good to go for a hemophilia event but not just talking about hemophilia. It was fun to do the activity and I would like to do something like that again."

We'd like to express our gratitude to Sanofi for sponsoring this fantastic event, which we hosted online in December 2021. And if this event sounds too cool to have been planned entirely by adults, that's because it was! Seventeen-year-old hemophilia B community member Charlie cohosted the event with me and community member Cassandra.

"I think that this meeting was great because many of the people who joined participated and joined in on the conversations we were having," said Charlie. "The escape room was fun, and it made it much easier when everyone helped to solve the riddles. The process of creating the teen event was very interesting, and it was a lot of fun. If I had the opportunity, I would 100% be interested in creating another event."

Are you interested in learning how to cohost a Coalition event for teens? Reach out to us at rockyw@hemob.org!

Oh, and want to know what was true and false? Colorado was in fact organized as a US territory in 1861. But milk only cost a nickel, and the agreement among soldiers was actually not to shoot when someone was going to the bathroom!

Meet Future Songwriter: Gabriella

Hello! My name is Gabriella and I have hemophilia B. I'm currently 12 years old and I am from Ohio. I am connected to The Coalition for Hemophilia B community thanks to my mother, who has hemophilia B and is a carrier. My brother has it too. My grandfather on my mom's side has it as well.

I also have another bleeding disorder! It is called platelet dysfunction. While hemophilia B is an inherited disorder, platelet dysfunction may be hereditary or acquired out of the clear blue sky.

Having both of these bleeding disorders have no doubt impacted my life. For example, I've always wanted to play soccer. I've watched games and seen my friends play, but due to my hemophilia, I cannot play contact sports. There is too much risk! Unfortunately, most of the sports I have an interest in ARE contact sports. However, some non-contact sports I can play are cross country, track, and swimming. The Swim team is only for high school though, so I have to wait another year or two.

I like to stay positive, and there are a lot of other interests I have that my bleeding disorders do not impact. I think in the future, I will be a songwriter. I love to write songs. I write songs about a lot of my problems and sometimes about just random things. I love music in general. I began writing lyrics in 5th grade when I was 10 after experiencing a lot of bullying throughout elementary school. That was when I wrote my first song. I named it "That Day." It was pretty good for it being my first time if I do say so myself! I play piano/keyboard, acoustic and electric guitar, ukulele, and flute.

If I do become a songwriter or singer when I'm older, I may have to do performances. With that, I will have to be careful on stage. So far, I haven't really performed anywhere in public other than in my choir class. Though I did sing "That Day" on Zoom in front of my 5th-grade class!

Another career I might pursue is becoming a cosmetologist. I love makeup. I often practice on and with my friends, and enjoy watching tutorials on YouTube. I am only 12 and have many years ahead of me to discover my path.

Even at my younger age, I already am well aware that having alternatives in life are important. You can't just focus on one path, because if that doesn't work out,

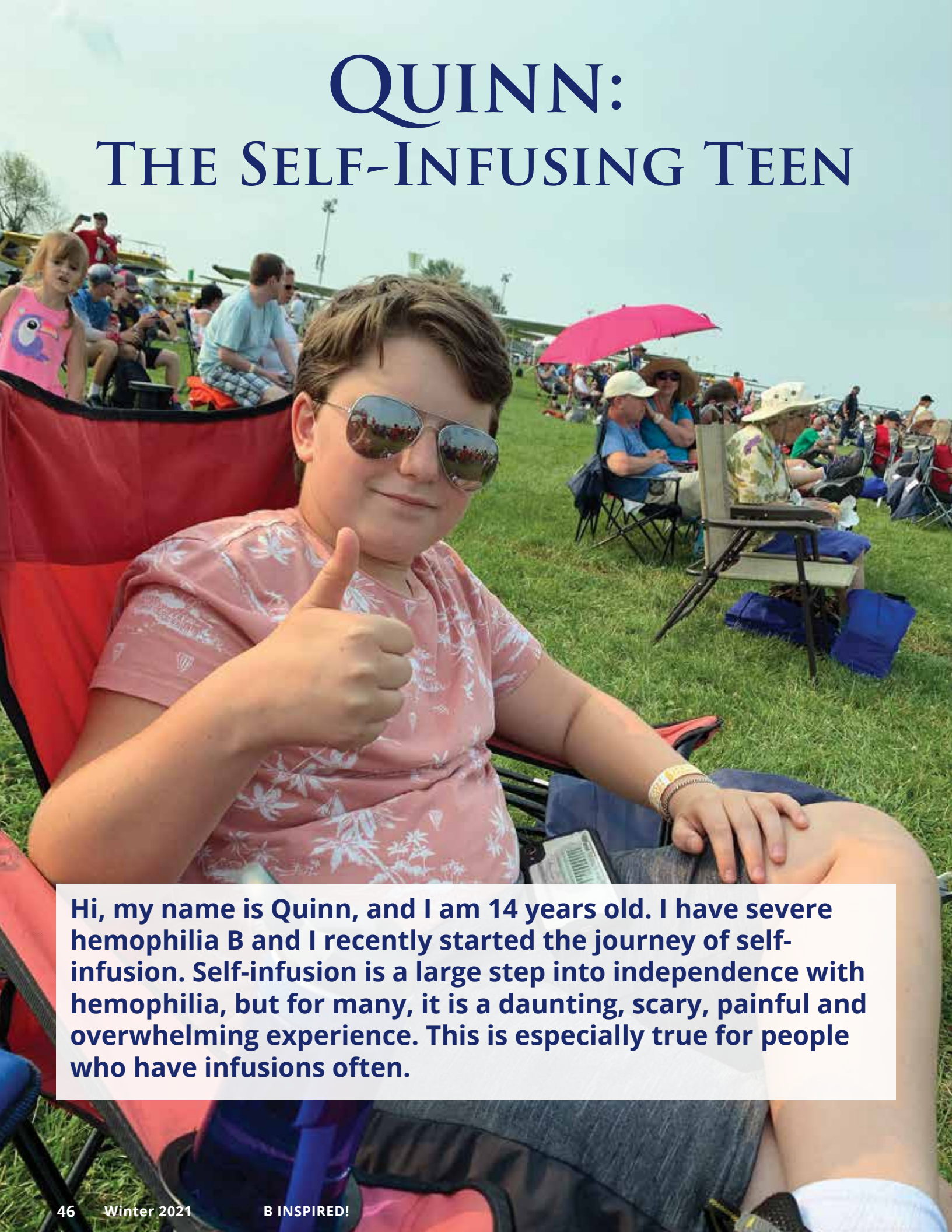
then what? Due to my bleeding disorder, I have to often find alternatives to things I want to do in order to be able to do them - even when it comes to activities with my friends.

The way I explain my condition to my friends is that it essentially is where people with hemophilia bleed and bruise easier when injured, more than the average person. I also tell them sometimes if someone with hemophilia is bleeding excessively, the person may need a factor, which is a medication to help control bleeding.

I am grateful to The Coalition for Hemophilia B because I met some people through the in-person Coalition event in Florida in 2019!

My advice to any other teens in my shoes is that you have to stay positive and try your best.
That's the key to success.

Quinn:
The Self-Infusing Teen



Hi, my name is Quinn, and I am 14 years old. I have severe hemophilia B and I recently started the journey of self-infusion. Self-infusion is a large step into independence with hemophilia, but for many, it is a daunting, scary, painful and overwhelming experience. This is especially true for people who have infusions often.

I wanted to learn how to self-infuse so I can take control of my hemophilia. It creates independence and would mean that I could go on trips without my parents and do infusions when and where I wanted.

The first time I stuck myself was at The Coalition For Hemophila B Symposium In Orlando with help from Nurse Hope. Some may call it beginners' luck (I would) but I got it on my first try! Once I got home from the trip, I was willing to try again on myself to see if I could still get it. I couldn't, and long story short, after about two months of missing, I stopped attempting to do this on my own.

I was very nervous at first because I knew how it felt to miss and have to do it over and so I thought that it would just be easier to have my parents continue to help me and get it on the first try instead of me trying and probably missing. I didn't try again for a whole year.

At the beginning of this school year, my parents and I went through the process of getting a home healthcare nurse to come when I had my prophy so he could help me learn techniques and strategies to guide me on the process of self-infusion.

The nurse at first helped me with the psychological aspect of it, basically getting me to try and stick myself and get over that hurdle. Tips the nurse shared with me after that included saving air in the syringe to use to flush out the last of the factor in the tube; using a pen to draw right on top of the vein and above where you want to poke so you have some sort of guide, and using the factor box or something like that to lean your hand over to pull back the skin which helps with the vein moving around.

Now after about a year of practice, I can pretty much do it all by myself but still have the nurse on call.

To anyone, especially the younger hemophilia generation, who has a goal to master self-infusing, I want to say self-infusion is a little difficult, but once you overcome the fear, the skills will come. Getting help with the process, if you are able to is a great way to learn.

B inspired!

Stories and artwork from teens in the Hemophilia B Community

WINTER 2021

IN THIS ISSUE:

QUINN: THE SELF-INFUSING TEEN

MEET FUTURE SONGWRITER: GABRIELLA

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!