THE COALITION FOR HEMOPHILIA B

HEMOPHILIA B NEWS

WINTER 2020

30 YEAR ANNIVERSARY
THE COALITION FOR HEMOPHILIA B

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

30 YEAR ANNIVERSARY THE COALITION FOR HEMOPHILIA B

MEET CHRIS: LIGHTING UP THE WORLD

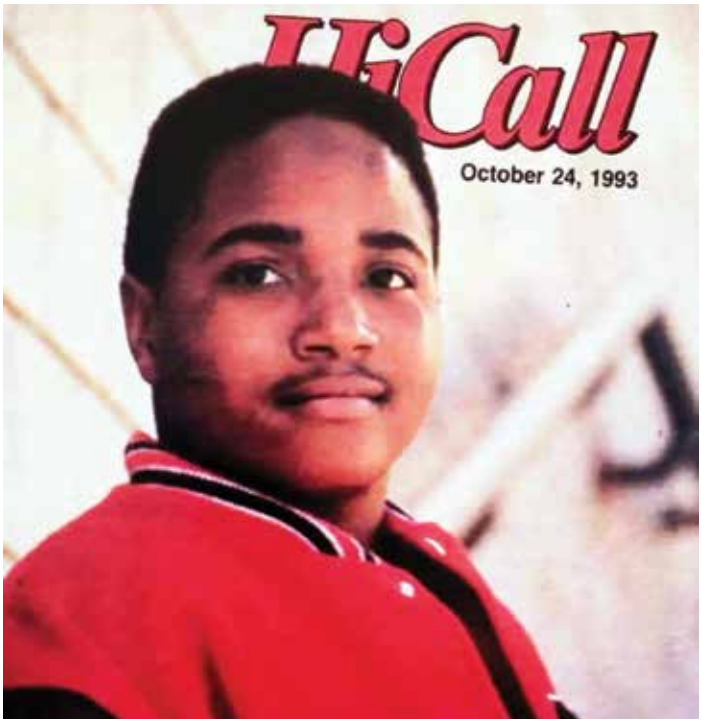
FAMILY IS KEY

BY DR. APRIL WILLIS AND RENAE BAKER

Let's talk about joy. The kind of pure, unfiltered delight that illuminates the darkness around us and creates a sense of gladness. Now, let's talk about Chris. Chris is a pillar in the hemophilia B community and is an individual who radiates joy.

In fact, Kim Phelan, COO of The Coalition commented, "Chris is one of the most loving and caring individuals I have ever met. He has a smile that will melt your hearts and exudes love to all. He really cares about everyone and will do whatever he can, whenever he can to advocate for his brothers and sisters in the community."

Chris has a way of spinning the not-so-great situations into opportunities to learn, grow, and recognize all he has to be thankful for. From his incredible younger brother who also has hemophilia, to his biggest cheerleader (his mother), to the ability that he is still able to travel despite his affliction, to the amazing connections he has built with the hemophilia community, Chris persists to revel in appreciation. He believes, "Having a positive outlook is the best thing for any people with hemophilia to have. Always keep yourself in positive energy no matter what happens, because then light always shines on things you've never seen before."



Back in the 1960s,his mother, Jacqueline, had a brother with hemophilia B. She watched over him closely with each hospitalization. Unfortunately he died from a severe head bleed. Due to the racial issues at that time,Jacqueline told Chris and his brother to be vigilant and advocate for themselves, and she taught them how to do that. When they were very young, she told them what they needed to do when they had a bleed, and how to talk to the doctors. To this day, she gives them the 'superpowers' of knowing what to do. She also gives them the spiritual essence of who they are as a family. That's where his strong passion to advocate comes from!

Still today, Chris can get angry with the care at the ER, "They still are not listening; not taking us seriously. And there's no sense of urgency that if they don't treat you right away, you'll end up with a worse situation. When we go to the hospital, we know what we need and it's frustrating to not get factor in a timely manner." This is all too common in the hemophilia community.

Chris also talks about some of the special aspects of having a brother with the same bleeding disorder. "We get to spend a lot of time connecting to each other. We have a unique bond that's different than in most families. We get comfort and happiness from being able to connect with and understand each other." Although they are three years apart, Chris explains that they have twin sensibilities as though they were truly twins. "When something is wrong with one of us, the other knows right away." This is an aspect of Chris's hemophilia family that he cherishes.

As a person not only with hemophilia, but with an inhibitor that often counteracts his treatments, Chris can be out of commission for extended periods of time as his body heals. "I was an active kid, trying not to be aware of

my hemophilia. I wanted to go out and do things, but hemophilia B with an inhibitor was stopping me."

Chris explained that his target joints are his knees. "Eventually, they were bone-on-bone. I had a total knee replacement." Having an inhibitor made it much more difficult for doctors to treat him. The surgery, recovery, and hospital stays were made longer because of it. "I graduated high school while I was in the hospital. I learned that having a great medical team that knows the cadence of my health is important." However, he is not one for complaining. Actually, he posts a daily positive attitude blog to provide inspiration to others! He is also one of the greatest advocates in the hemophilia community due to his experiences as a person of color championing for treatment options.

You will have good and bad days but remember to use your resources such as calling a friend or having a telehealth therapy session. We are lucky we have devices that keep us connected. Be gentle with yourself on those hard days and take it easy. Being a Coalition member gives us an added benefit of being that spark of light for someone else and that in itself gives us comfort. We are there for each other.

Chris says hemophilia has been good and bad for him. He enjoys the opportunities given to him through The Coalition for Hemophilia B. Chris became involved in the Coalition a few years ago when attending a men's retreat. "They've been a blessing to me. They've taught my brother and me that we all have stories to share and they have given us other avenues to tell our stories. The Coalition is family. We have a familial bond, and they show it in many ways."

When it comes to his connections to others in the hemophilia community, Chris is driven to create authentic relationships and to impact lives. At Coalition events, he is known to volunteer his time supporting the team and learning from those whose experiences have been somewhat different from his. "When I'm at a men's retreat, *The Beats* program or taking part in the conferences, I try to be a helper to the staff. I want to shine a light on them because they've done so much for me." According to Chris, "The Coalition is family. We have a familial bond, and they show it in many ways."

Coalition President Wayne Cook said, "Chris is an amazing person and all around nice guy. We are lucky to have him in the community. I was blown away when Chris showed up at our first annual *The Beats* music program to learn vocals and joined drumming. Never having played before, he was just fantastic!"

Chris wrapped up by saying, "The team is like another set of parents to me! Wayne, Kim, Chris and Rocky all teach us things that help us grow and realize how we really are giving to the community in such a way that others, down the road, who are growing up can better themselves. That's what The Coalition for Hemophilia B is all about. They help you understand your value and expand your horizons. The bonding within the Coalition is wonderful! I have learned so many things that I didn't know. This is how the family bonds are formed, and family is key."

When was the last time you met someone who was lighting up the world? If it's been awhile, I encourage you to meet Chris. He might be the light you are looking for.

"Being in the Coalition gives us the added benefit of being that spark of light for someone else, and that, in itself, can give us comfort."

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

Together, they're the Heroines of Hemophilia B.

Share your stories at

HeroinesofHemophiliaB.com

MEDEXUS PHARMA

YOUR STORY IS YOUR ADVOCACY

BY NATALIE SAYER

Every time you tell your story, you are an advocate. But what does that really mean? Well, the dictionary describes an advocate is a "person who publicly supports or recommends a particular cause or policy." Who better to be an advocate, than someone with first-hand experience. The most powerful advocates move our hearts, connect our minds and spur us to action. Many of you may have attended the Symposium session "Pen It! Prep It! Perform It!" in October. This article is meant to be a follow-up guide for you to write and deliver your own advocate stories.

Why do you write your story?

In addition to helping you clarify your thoughts; a written story helps you create impact when you tell it. You have seconds to grab people's attention and minutes to tell your story. Wouldn't you want every word to count?

Do you get nervous when speaking in front of people, especially strangers? Written words will keep you focused when the nerves or emotions creep in. Telling the same story in the same way, will also combat nervous energy. We will get to practice a little later.

Who is your audience and why should they care?

We all have many different stories we could tell. Knowing your audience is important to identify the right story and words to create impact for them. If you are speaking with a class at school or legislators in government, the topic might be similar, but the content will be very different. What you want from each audience will be different, too. Writing with your audience in mind enables you to tailor your message and impact specifically to them.

What do you want them to remember?

We tell our stories because we want to move our audiences to action, to empathy, or to support. We want them to remember, long after we have spoken, a key message. If you were at the Symposium, you may remember "Let Go to Grow" or "Life is hard. Life's not fair. What are you going to do about it?"

When you can build your story around a key message, and then repeat that message multiple times; you have a better chance that they will remember your point. When you connect emotionally, you reinforce your message. You get to choose how you want them to feel through your word choices. Do you want them to laugh or cry or cheer?

Where do you find your story?

When you know your audience and what you want them to remember, then you can review your life for moments that represent your message. Often when working with people to write and perform their stories, I hear, "I don't have anything interesting. I don't have stories." But with a little probing and some key questions, we find them. Is there

a moment or memory of a time that demonstrates your message? Do you want to share “a day in the life”? Were you part of something bigger than yourself, like when the song Silver Linings was written and performed at the 2019 Beats program? Trust that you have stories.

Who are the characters?

While your story is your story, every story has lead and supporting characters. Each of the characters has a purpose. When you think of stories or movies that have stuck with you, who were the characters? What role did they play? Who was the “hero”? Who was the villain? Who was the wise one? Who provided comedy? Who was kind? Your story has characters. How can each help bring your point to life?

What is the plot?

Every story has a plot or a story arc, which takes the audience on an emotional journey. Think about the classic movies. The hero has a challenge, faces setbacks, has help to overcome them, and learns from the experience. Star Wars, Harry Potter, Forest Gump and many more follow this “hero’s journey” model. While it isn’t the only model, it is a good place to start. Remembering the audience and point you are trying to make, what is the plot of your story? How do you hook them in the beginning? How do you create a strong call to action at the end?

Write, re-write and write again.

So you have written your first draft, congratulations!Now the real work begins. Creating a powerful story requires finesse. It isn’t uncommon to write four or five drafts before you are happy with your work. As you read each draft, identify what emotions you want your audience to experience with each paragraph. Do your words create that emotion? Have you repeated your key phrase throughout the story? Can you simplify or delete words?

One of my favorite editing tools in Word is the Spelling & Grammar check, especially the Readability Statistics. If you can make your point and emotional connection, while still writing at an eighth-grade level, you are golden!

Who is in your circle?

The best way to ensure your story will land with your audience is to test it out on people, who you trust to give you real feedback. You may have a few people read your draft and suggest edits. Or you may tell your story and ask your trusted audience key questions like “What was the point?”, “What did you feel?”, “What did you learn?”, “What was confusing?” or any other question that ensure your story is making the connection you want it to make. Use that feedback as you refine each draft.

Practice. Practice. Practice.

When an actor receives a script, it is only the beginning. It is through rehearsal that they bring the words to life for the audience. It is also the actor or storyteller’s job to create the experience every single time they perform the work. We call it “first day freshness”. Rehearsal is a process. You start by learning the words. If you are telling your story live on a stage, you may decide to move at certain points, always moving with intention, to enhance the story. Ultimately, you want to rehearse exactly how you are going to perform.

If you are telling your story to a class, then you will rehearse standing up. If you are testifying to a committee at the legislature, then you may rehearse sitting at a table. Rehearsal is also where you decide what to do with your voice and physical presence to bring your words to life.

If your story is very emotional for you to tell, rehearsal is also where you learn to express the emotion with control. You want your audience to feel the emotion, yet if you are breaking down uncontrollably, you may lose them. The more you tell your story, the easier it will be to control your delivery. Practice matters.

Your stories. Your advocacy.

Regardless of who or how large your audience is, your stories matter. When you tell your stories, you have the opportunity to raise awareness, garner support, or even change someone’s world. Your stories matter. Your experiences matter. Your advocacy matters.

ADVOCACY UPDATE

BY GLENN MONES

During the last quarter, we learned that insurance companies could still employ accumulator adjusters. These programs, designed to allow the companies to make more money, can prevent a patient from counting third-party co-pay assistance towards their out-of-pocket maximum. Essentially, this means the companies who do this are “double dipping.” We were advised for a time that these programs would not be applied to hemophilia, but that turned out not to be the case. We are now looking at whether a legislative fix might be necessary.

In an end-of-year victory, Congress voted to approve the end-of-year omnibus federal spending and economic relief package which included the provisions of the Hemophilia SNF Access Act. This legislation mandates that patients covered by Medicare will have access to clotting factor during a stay in a skilled nursing facility (SNF). This includes rehabilitation facilities that could be required after a surgery or other hospitalization. This change will also improve overall patient access to these facilities, since many have refused to accept hemophilia patients in the past because of fears that clotting factor would not be adequately reimbursed during a stay.

The bill is awaiting the president’s signature, which is expected by next week. This issue has long been on the advocacy agenda of the hemophilia community. It moved forward in January 2020 with the introduction of the bipartisan legislation and was prioritized during the National Hemophilia Foundation’s Washington Days advocacy event in February. The Coalition for Hemophilia B is proud of the role our members played in helping to bring this issue to the forefront. We applaud the entire hemophilia community and the legislation’s sponsors for achieving this important victory.

Washington will see substantial changes in 2021 which could positively impact our ability to advance healthcare advocacy goals. We must be ready to take full advantage of these opportunities. In 2021, our B Voice advocacy program will preserve and expand the best aspects of what we have developed to date, empowering our community with more information, education, knowledge and “tools you can use,” while also addressing the lack of health equity that many in our community face. Areas of focus will include:

- Elimination of co-pay adjustor programs
- Elimination of surprise billing
- Expansion of Medicaid and other affordable coverage options
- Increased focus on health equity within the hemophilia community and beyond
- Ensured inclusion on insurance formularies of all approved therapeutic options as prescribed by doctors in consultation with their patients and caregivers
- Support for adequate funding models for gene therapy and other novel treatments
- Addressing the lack of health equity for women and other underserved populations in our community
- …and many more.

We have already begun our efforts with the new administration by joining a sign-on letter to President Biden about the importance of health equity and other priorities for healthcare advocates. With your help, we can make a difference for people with hemophilia and all Americans dealing with chronic conditions this year. Join our efforts!

women bleeders

A NEW FEATURE SECTION

ARTICLES TO SUPPORT WOMEN WITH HEMOPHILIA B

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.

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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

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novo nordisk®

No Longer Alone

BY RENAE BAKER

“How do you explain what the heck you’re going through when you’ve never been told what to expect, and no one else knows what you’re going through either?”

Emili expresses her gratitude for the extraordinary programs, care, education, and fellowship that The Coalition for Hemophilia B provides. The sheer volume of information and resources her family receives about hemophilia B through the Coalition has improved Emili’s and her family’s lives tremendously. She heaps praise on the team for their care and tireless work enriching the lives of its members. But she went through many years that she would describe as “not fun” before she found the Coalition for Hemophilia B.

I met with Emili, via zoom, as her daughter was attending her first day of kindergarten, in a mask-optional school. We could have had a whole conversation around a parent’s worries about sending her child to an enclosed building during a pandemic to start her elementary school years, but to Emili, it was simply an additional concern to add to a 30-year stew of life-threatening challenges.

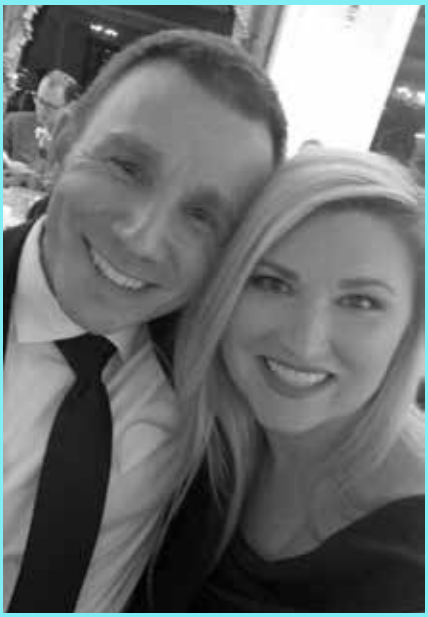
Emili recalled her own elementary school experience. “I was very athletic.” She said of her childhood days in a small town in Arkansas. “I played a ton of sports.”

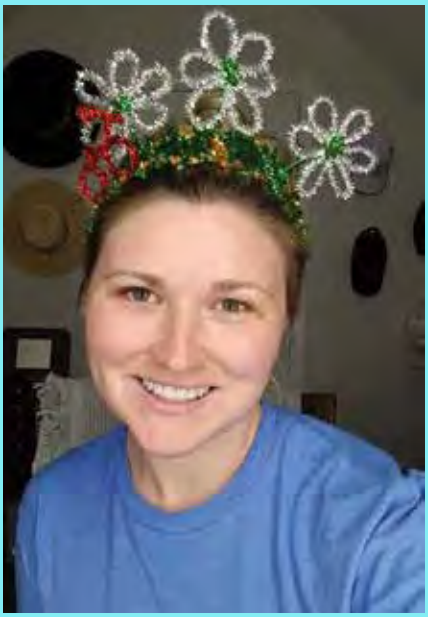
Emili knew there was hemophilia in her family. At least one of her grandmother’s brothers had it and she knew that her father had it.“In fact, I knew my dad had hemophilia B,” Emili said, but because the family had not been educated about it, they didn’t understand the level of care he needed. “He only infused if he was physically weeping out of his body or dying in the emergency room.” Emili’s father had also been athletic. He was a baseball pitcher. “As a child, he’d have terrible swelling and unknown bleeds, and Grandpa would just tell him to ‘walk it off.’”

The lack of knowledge both in her family and her local medical community has caused long-term damage in both her father and Emili. Still a young woman, Emili has lost some range of motion and has already had six surgeries on her right knee. “The first joint bleed I remember having was my left ankle - I was probably 9. I was playing basketball, and I did a lay up and twisted it. It swelled massively. Everyone thought it was a sprain, but it wasn’t getting better, so we went to an ortho and had an MRI. They just said, ‘There’s no fracture. It’s just a bad sprain.’”

When she was 12-years old, Emili’s parents took her to a hospital in Little Rock to see if she was a carrier. “My parents didn’t know of anyone who specialized in hematology only, so we saw my dad’s doctor, a Hem-Onc, and learned that I am, indeed, an obligate carrier. But he told us that there was no way women could have Hemophilia, and if I did, by some chance have it, I would be 1 in 250,000 hemophiliacs, and – of course – there aren’t that many hemophiliacs in the world.” So, with some relief, Emili and her parents believed that she couldn’t have hemophilia. Yet, she was having severe nose and mouth bleeds. Tooth extractions brought on relentless bleeding and would cause her to miss an alarming number of school days. “The blood loss was terrible! But the dentist just told my mom, ‘Put a tea bag on it!’”

“And then, dear Lord Jesus, my menstruation got here! And that was a whole ‘nother ball game!” It only took a couple of incredulous looks from her high school girl friends for Emili to realize that her adolescent experience was not something to which they could relate. “They





looked at me like I was crazy. It was scary, so I always dealt with it by myself. I never thought to share or ask my mom about the heavy bleeding. I thought, 'It's just me. I'm weird. Deal with it.'"

Because Emili's doctor told her she couldn't have hemophilia and there was nothing wrong with her, her teachers and coaches remained unaware of any of the problems she was having. Being the athlete she was, Emili was determined to not let her periods get in the way of her sports, in spite of the fact that she was bleeding heavily 10-14 days a month. "I was using the super-sized tampons and two maxi pads at a time, and I'd still bleed through my clothes. I always had to bring extra clothes with me to school and sporting events. I often felt like I was going to pass out while playing. One day during track practice I actually did pass out and fell down four or five stairs which left me with a scar on my left ankle."

The older she got, the worse it got. "After I had my children, it got exponentially terrible," Emili says with the kind of laugh Victorian authors depict at gravesides.

Now, married to Henry and living in Dawsonville, Georgia, Emili became pregnant with their first child, daughter Gensen. The gestation period was relatively uneventful. Bruises from bumps were more notable, and her factor levels went down. Her hematologist checked her factor levels every three months and to his surprise, her levels always came back 13-28%. He felt he should, but couldn't, diagnose her while she was pregnant, because hormone levels can affect factor levels.

In his 26-years of practice, he'd never treated a woman with hemophilia. He didn't know what to do beyond notating it on her chart for delivery and advised her to have a special consultation with the delivery

anesthesiologist, which she did. Emili describes Gensen's delivery as "great," and she experienced the normal postpartum bleeding for only three or four weeks and then went back to her pre-pregnancy bleeding.

A couple of years later, Emili became pregnant with their son, Xander. Her factor IX level went up, but her von Willebrand's level went down again. Her hematologist wanted Emili and Henry to be more prepared for this pregnancy, because they were having a boy. This wonderful hematologist gave them his on-call number and instructed them to make sure her other doctors had it.

Emili visited with him on December 7th, at which time he said, "I'm officially going say you have von Willebrand's type 1 and mild hemophilia B. Emili left the appointment, went home, and her labor began a few hours later on December 8th.

In spite of the extra precautions and

consultations they had taken to make sure the hospital was prepared for Emili's bleeding disorders, the next few hours would be, as Emili says, "extremely terrible." "We knew things were going be bad from the get-go when the anesthesiologist walked in and asked me what hemophilia was. He said, 'It looks like you might have a bleeding problem.'"

Emili had planned to have an epidural for this delivery, as she had for Gensen's birth. An epidural provides anesthesia that creates a band of numbness from the bellybutton to the upper legs. It allows the mother to be awake and alert throughout labor as well as to feel pressure, which helps the mother push when it's time to give birth.

The anesthesiologist proceeded to botch the first and second epidural. He then decided to resort to an intrathecal epidural. This kind of epidural is more invasive as it's inserted deeper into the spinal column and there is a higher chance of experiencing spinal headache."I was numb, but I could feel that I had started bleeding from my back. Then he told us that we'd have to have it in for a minimum of 12 hours because it can cause spinal bleeding, so I thought, 'This is fantastic!'" with her jolly brand of sarcasm. "You should havtold me this BEFORE you did it!"

Unbelievably, a short while after the intrathecal epidural was inserted and doing its job, the pump which controls the drip of the numbing agent broke. "So I had this terrible epidural in my back that I was bleeding from, and I could feel everything, because it wasn't working!"
Henry was furious at this point and left the room to find the head anesthesiologist. Thankfully, it was the same physician who performed the epidural procedure for Gensen's birth.

"He changed out everything and applied a nerve block like you'd have with a C-section. So, I was numb from the chest down and Xander was born. He was born with an ankle bleed.

After he was checked out and determined to be fine, Emili immediately started losing "loads and loads" of blood. "I'm lying there numb, helpless and bleeding. I didn't know what was happening. My husband was in full freak-out mode and was saying, 'What is going on? Someone needs to treat her. Stop worrying about my son; he's fine. He's crying. He's breathing!'"

The cause of the bleeding turned out to be that Emili's placenta had torn during the delivery. Her blood pressure dropped. She was losing so much blood that they tilted her bed to get her feet higher than her head.

"Imagine the worst crime scene you've ever seen on tv," Henry illustrates, "Xander's birth was worse than that!" "If Xander had been born first, we wouldn't have had a second child." Emili states emphatically.

There is so much more to this story that Emili would like to relay to anyone who wants to contact her, including the tales of the hospital not replacing her blood and sending her home for the weekend, the spinal headache, the spinal patch, and the postpartum bleeding that continued for so long that strong medication was needed.

Emili replaced her OBGYN after this delivery. Her new doctor recommended a procedure that would help her stop having periods for several years. The procedure, an ablation, sounded good to Emili and Henry, and she went in for it. During the procedure, the doctor unknowingly nicked her cervix, which was discovered when Emili sat up in the recovery room and it started bleeding profusely. She was then immediately whisked away for a second surgery.

After all of that, (and more,) the years of menstruation cessation of which the doctor spoke turned out to be a mere six months. To make matters worse, her periods now came with excruciating pain akin to labor. Her doctor recommended a prescription of progesterone that would mimic her being constantly pregnant. Emili said, "Listen lady, you can put me on an IV drip as long as I'm not bleeding any more!"

That was May of 2019. Since then, Emili has had no vaginal bleeds. In between her pregnancies

and progesterone prescription, Emili has had the typical joint bleeds, knee surgeries, hematomas drained, and her gall bladder removed.

Emili doesn't tell you all of this to scare you, but to let you know that you can find women, through The Coalition for Hemophilia B, who will be able to answer your questions, share their experiences, and give you advice that can fortify you against experiences like this.

Emili became aware of The Coalition for Hemophilia B through a specialty nurse. "About a month after Xander was born, Lindsay messaged me and told me about the Coalition and that they were about to have a huge event within driving distance from us." Outside of her father, Emili had never met another person with hemophilia, let alone hemophilia B.

On their drive to the symposium, Henry wondered how many people might be there. Emili hoped for 50. Emili, a self-professed non-cryer, gets very emotional when she recalls that there were over 300 people there! After 30 years of "walking it off," thinking she was "weird," and navigating her way through one personal horror show after another with no one who could relate to her experience, she found herself walking into a room full of new brothers and sisters who embraced her family and had answers to every question she had and more! Here was a beautiful pool of untapped knowledge to access to become educated, healthier, happier, proactive, and better advocates for themselves. "I said to Henry, 'Dad is never going believe this!' so I took 82 photos and sent every one of them to him!"

"We learned that we weren't alone. We learned that we aren't the only ones struggling with huge medical bills over this. We learned that women can, indeed, be bleeders. There were other women! Some even had horrible births! I wasn't alone in that anymore. I met women who had known about their bleeding disorder since they were three. (I wanted to know where they lived and learned all of that!) We learned about insurance and prophylaxis and the different types of bleeds."

Emili sent all of this information to her dad, including the fact that people with mild hemophilia B can be on prophylaxis. "That was huge for him! Now he infuses once a week and sends me a photo for accountability."
Emili and her family are so grateful to the Coalition for Hemophilia B and the teaching and training they provide. "They bring in actual hematologists, not just hem-oncs. They helped me advocate for myself, my son, my dad and my grandma."

She explained that her grandmother has afib and is almost 80 years old. "Her doctor wanted to do all sorts of things to explore, and I was able to say, 'NO! You are not sticking my granny until you check her factor levels.' If I hadn't been taught, by the Coalition, to do that - who knows what could have happened?"

"Ever since Xander was 3 months old, we've been to every symposium and attend every event we can. It grows bigger every year. It's so great! I have a family of hemophilia B moms and sisters. I can message them and say, 'This is happening,' and they'll message back, 'Don't worry about it. This is what you do.'" Emili says with an inhalation of gratitude and an exhalation of relief.

Words of advice Emili has for new Coalition for Hemophilia B members: "Don't think you or your child has to live in a bubble - if you have questions, ask anyone within this community and they'll have the answer."

Women With Hemophilia

BY DR. DAVID CLARK

11/19/20 The World Federation of Hemophilia (WFH) is starting a new initiative on women with bleeding disorders. WFH held a virtual Global Summit on Women and Girls with Inherited Bleeding Disorders on November 19 – 20, 2020. They have also added additional information to the eLearning section of their website on *Carriers and Women with Hemophilia*.

For too long, the needs of women with bleeding disorders have been ignored, mainly because of ignorance of the actual genetics of hemophilia. We are finally starting to understand that many women are affected by hemophilia and need treatment. Both NHF and HFA, as well as The Coalition for Hemophilia B, have focused more and more on women's needs over the years, and this worldwide attention from WFH (wfh.org) is very welcome. Note also the Foundation for Women and Girls with Bleeding Disorders (fwgbd.org) that does a wonderful job helping women find treatment.

11/24/20 We have often recommended Shelleye Horowitz's articles on the issues women have in dealing with hemophilia. She writes frequently on *Hemophilia News Today*, a free hemophilia email and web-based news service. She is a woman with mild hemophilia A who can trace her family's disorder back through at least six generations.

Her November 24 column described her journey through six different HTCs as she moved around the country. The six centers all diagnosed and treated her differently. Some only labeled her as a "carrier" or symptomatic carrier," which turned out to be a barrier to receiving adequate treatment.

She was admonished to "not waste expensive factor" with the implication that the men who had "real" hemophilia needed it more. Even after she finally obtained a correct diagnosis and treatment from one center, the next would often ignore that and start over, often taking her backward. As she wrote "It was confusing, frustrating, and showed a flaw in the current system for treating hemophilia."

Because of her experience, coupled with the experiences of many other women, Shelleye is advocating for a consistent *Standard of Care* for women with hemophilia. In what other field would someone with a serious medical issue not be taken seriously, be ignored and be denied needed treatment? (Actually, there are probably some other medical fields that don't take women seriously, but that's not right either!) We applaud Shelleye and her efforts and wish all women with bleeding disorders better outcomes in the future.

Hemophilia Health News

BY DR. DAVID CLARK

Some of the following reports come from studies that were presented at the 62nd Annual Meeting of the American Society of Hematology (ASH), December 5 – 8, 2020. Copies of the abstracts (summaries) are available on the ASH website at hematology.org.

NOT ALL PATIENTS BENEFIT FROM EXTENDED HALF-LIFE PRODUCTS

12/5/20 Not all patients benefit from switching to extended half-life (EHL) factor products according to a Canadian/ Dutch study presented at ASH. Small studies have shown that EHL products are beneficial in increasing trough levels and decreasing infusion frequency for groups of patients, but this is the first study to look at half-lives in individual patients in a large group. The Web-Accessible Population Pharmacokinetics Service (WAPPS) is used by many treaters to analyze pharmacokinetic (PK) data from their patients. WAPPS maintains a database of PK data for patients with hemophilia, and the researchers were able to collect results on 649 patients who had used both standard half-life (SHL) and EHL products.

About 11% of the subjects had hemophilia B. As a group, they showed a 3.1-fold (310%) median increase in half-life with EHL products compared to SHL products. Individually, however, some patients only showed half-lives increased by 25%. The relative increases were similar for both children and adults. This suggests that some patients may not benefit significantly from switching. The authors recommend individualized PK studies for patients considering switching. [ASH abstract 234]

MANUAL THERAPY HELPS HEMOPHILIA JOINTS

10/23/20 Physical therapy is an important aspect of hemophilia treatment for those with bad joints. A group in Spain wondered whether the addition of manual therapy to passive stretching would be beneficial for joint health. Manual therapy involves a therapist using their hands to manipulate parts of the body, while passive stretching is stretching by holding one position for a length of time. The researchers looked at 28 hemophilia patients (24 As; 4 Bs) with target knee joints. Half received manual therapy with passive stretching in two hour-long sessions per week for 12 weeks. The other half followed their normal routine as a control group. The groups were assessed before and after the 12-week treatment period and then followed up another 12 weeks later.

The results were striking. In the control group, the average frequency of knee bleeds was 0.71 after the first 12-week period and 2.29 at follow-up. In the group treated with manual therapy and passive stretching the frequency of knee bleeds was zero after the first 12 weeks and 0.43 at follow-up. Pain in the treated group decreased from an average of 5.21 (on a 10-point scale) pre-treatment, to 2.0 after the first 12 weeks, to 1.71 at follow-up. Pain scores for the control group were essentially unchanged. The treated group also showed improvements in joint health and range of motion, while the control group saw no benefit.

There have always been concerns that physical therapy could increase bleeding in hemophilia patients, however, this study suggests just the opposite. The authors recommend future studies with larger groups and additional joint-health measurements to confirm their findings. [Cuesta-Barriuso R et al., Haemophilia, online ahead of print 10/23/20]

TREATMENT COMPLIANCE/ADHERENCE

11/20/20 Complying with your hemophilia treatment regimen is an important part of maintaining your health. Compliance includes things like keeping your regular physician appointments and keeping up with your infusions. A new study from Brazil looked at 138 adult

hemophilia patients (104 As; 34 Bs) for a period of one year, with a long-term follow-up three years later to evaluate mortality.

They found that non-compliance was high; only 25.4% of patients were fully compliant in all aspects of their treatment. Looking just at clotting factor regimen compliance, the rate was better, but still low at 65.9%. The only endpoint they measured was mortality (death), and all ten of the deaths during the four-year study were in the group that was least compliant. [Soares BMD et al., Patient Preference and Adherence, 14 2279-2285, 2020]

12/6/20 A paper at ASH also looked at patient compliance. A group from the UK analyzed data from the "Cost of Severe hemophilia across the US: A Socioeconomic Survey" (CHESS US+) study that was based on a patient-completed questionnaire. The 356 subjects with severe hemophilia (260 As; 96 Bs) were divided into three groups (low, moderate and full adherence) based on self-reported compliance. They found that self-reported pain increased as you go down in compliance – the low adherence group had the most and most severe pain, the moderate group was intermediate, and the full compliance group had the least pain.

When they looked at employment status, though, the results were reversed. The low adherence group had the highest percentage of full- or part-time work, and the full compliance group had the lowest. Unemployment and being retired were also highest in the full adherence group. These results were consistent with the results of the B-HERO-S study performed a few years ago. It appears that there may be a trade-off between good clinical outcomes (as measured by lower pain) and employment. The authors state, "Patients achieving optimal adherence were less likely to be in full-time employment, and more likely to be part-time or unemployed." The reasons are not understood. [ASH abstract 2541]

Editorial Comment: Along these lines, a question was raised recently in a meeting on gene therapy. In the uniQure study, it was noted that prior to receiving gene therapy 70% of the 54 study participants had had a total of 123 bleeds in past six months while on prophylaxis. The question was: "Why are these people bleeding?" The whole idea of prophylaxis is to minimize bleeding to protect your joints. Was it a question of non-compliance with their prophy regimen, or that the regimen itself wasn't sufficient, or something else? People shouldn't have to bleed. If you find yourself in this situation, please talk to your physician or HTC.

AGING WITH HEMOPHILIA

Because of the impact of the AIDS crisis on the hemophilia community, we know very little about health and socioeconomic status of older patients – we don't have very many to study! Several recent studies aim to fill part of that gap.

12/6/20 An international group of researchers analyzed results from the "Patient-Reported Outcomes, Burdens and Experiences" (PROBE) study to determine the effect of aging on the health status of hemophilia patients. The PROBE study compiled results for 1157 people with hemophilia (PWH) compared with a control group of 690 people without a bleeding disorder (PWoBD). Of those, 8% of PWH were 65 years of age or older, compared with 12% of PWoBD. On questionnaires rating health status and health-related quality of life, they found that the scores for PWoBD did not change with age, while the scores for PWH got worse. [ASH abstract 2529]

12/6/20 The HUGS VII study examined the cost and burden of hemophilia, including health-related quality of life, arthropathy (joint damage) and economic impacts, in hemophilia A and B patients 40 year of age and older who receive care at HTCs. They divided the subjects into a group 40 – 49 years of age (25 subjects) and one of 50 years and older (45 subjects) and looked for differences in a number of attributes. Between the groups, they found that fewer of the older group were employed (33.3% vs 72.0%); the older group was more likely to be married or have a partner (71.1% vs 56%); and the older group was more likely to be mild or moderate rather than severe (68.9% vs 40%). The older group was less likely to be on prophylaxis (38.6% vs 62.5%), but the prophy rate for those with severe disease was the same (85.7% vs 86.7%). Joint pain and range of motion limitations were about the same for both groups. The younger group was more likely to be on physical therapy (41.7% vs 15.9%). [ASH abstract 2490]

12/7/20 Another U.S. group looked at cardiovascular disease (CVD) outcomes in hemophilia patients. Using a nationwide database of hospital discharges, the investigators looked at results for 2007 compared with 2017. One of the interesting findings is that in 2017, the average age of hemophilia patients admitted to the hospital was 44.3 years, while in 2007, the average age was 30.9 years. Does that reflect improvements in hemophilia therapy over the ten-year period? For comparison, the average ages of the patients without hemophilia was relatively unchanged from 47.2 years in

2007 to 49.6 years in 2017.

The other interesting finding is that in both years, hemophilia patients scored better on almost all CVD risk factors and outcomes than the general population. Does this suggest that hemophilia patients have somewhat better cardiovascular health, or that hemophilia patients get better healthcare because of the HTC network? We don't know. Sometimes studies raise as many new questions as they answer old ones.

Don't let these results go to your head, though. The authors also note that from 2007 to 2017 the rates for all CVD risk factors and outcomes generally rose for hemophilia patients. CVD still remains a significant risk for the hemophilia population and needs more study. [ASH abstract 2686]

RISK OF HIGH BLOOD PRESSURE IN INHIBITOR PATIENTS AND IN HEMOPHILIA B PATIENTS

12/5/20 Hemophilia patients appear to be at a higher risk of hypertension (high blood pressure: HBP) than the general population. HBP also develops in younger patients with hemophilia, so it is important to keep track of your blood pressure. A 2018 study suggested that inhibitor patients might have a higher risk of CVD than non-inhibitor patients, but the study included too few inhibitor patients to be certain. A new study looked at 691 hemophilia patients including 44 with inhibitors at four U.S. and Canadian HTCs. This included 533 subjects with hemophilia A and 157 with hemophilia B. For A and B combined, 419 patients were HCV-positive and 128 were HIV-positive.

They found that subjects with inhibitors, or with a history of inhibitors, had no increased risk of HBP. However, after looking at subsets of the data, they found that hemophilia B patients had a greatly increased risk of HBP compared with hemophilia A patients. Bs actually had a lower risk than As until their early 30s but then the risk started increasing markedly, peaking in the late 40s/early 50s and decreasing slightly after that. At age 50, Bs had a probability of having HBP of about 90% compared to about 40% in As. By age 70, both were at about 80%. They also found that subjects who were HCV- or HIV-positive, or who had a history of those infections, had an increased risk of HBP.

There are still many unanswered questions about this, but it points out that hemophilia B patients should keep a close eye on their blood pressure. [ASH abstract 1633]

ARTIFICIAL INTELLIGENCE TAKES ON PROTEIN FOLDING

11/30/20 This is not a piece on hemophilia treatment per se, but it is about a huge advancement in protein science overall that may have a large impact on much of medical science. For decades, researchers have been trying to predict the structures of proteins. Proteins consist of long strings of molecules called amino acids. They are like beads on a string, with each bead representing one amino acid.

Factor IX, for instance, has 461 amino acids (beads) when it is first made in a liver cell. Forty-six of those are lost when it is secreted into the bloodstream, but that still leaves 415 amino acids in the final protein. Factor IX is actually a medium-size protein; factor VIII, the protein missing in hemophilia A, has 2332 amino acids (beads). Each amino acid is made up of a number of individual atoms.

The unique thing about proteins is that the string of beads curls up into a specific 3D shape, and that shape gives the protein many of its properties. Predicting that 3D shape, called the protein's configuration, and thus the protein's properties, has always been a puzzle. Now, an artificial intelligence (AI) offshoot of Google has developed a method. The offshoot is called DeepMind, and the AI program is called AlphaFold.

The amino acid sequence, that is the sequence of the beads on the string, has been postulated to completely determine the three-dimensional configuration of proteins. Based on that assumption, researchers have been using supercomputers to calculate protein configurations from first principles, that is, from the energy distribution around each atom in the protein. That can actually be done but the calculations are extremely complex and take tremendous computer power. One problem is that whenever another atom is added, it changes the energy distribution slightly, so you have to go back and re-calculate all the others. It's an iterative process that can take months for even a small protein. It's basically an impossible feat for real-world proteins and today's computers.

Now, DeepMind has another approach using AI. AI doesn't worry about why a certain amino acid sequence is shaped like a loop or a spiral or a flat sheet. It just looks at examples of proteins and invents its own rules that predict the shapes they have. AlphaFold is not yet perfect, but it has been very successful in matching configurations that have been determined experimentally by methods like X-ray crystallography and electron microscopy.

These experimental methods are good, but they are also laborious and expensive. AlphaFold can give scientists a head start using these methods. According to the DeepMind scientists, "AI will make it possible to study living things in new ways."

This is brand new and only starting to be used, but its influence on medical science may be profound. [Nature news article 11/30/20]

Five Self-Care Practices: Your Emergency Kit for COVID-19

BY MATTHEW BARKDULL, MENTAL HEALTH PROFESSIONAL

It's not difficult to recognize how the COVID-19 pandemic has sucker-punched the global community. Not only has it threatened and impacted our health, but its tentacles have also disrupted our social wellbeing, our mental and emotional stamina, our financial security, and the list goes on.

As reasonable yet inconvenient safety measures are required to maintain safety, we cannot thrive by maintaining a defensive strategy. As any sports enthusiast can tell you, the best defense is a good offense. Before George Washington died in 1799, he was quoted as saying "…offensive operations…is the surest, if not the only, means of defense." How can we begin to reclaim our lives through an offensive strategy? The answer is simpler than one might think—self-care!

Self-care is the gateway to recovery. It is akin to priming the pump of our momentum. As a licensed mental health professional, I wish to strongly recommend the following self-care strategies that have been shown to fill our individual gas tanks despite the uncertainties around us.

Self-Care Strategy #1 – Find Time to Laugh: A Jewish proverb states "as soap is to the body, so laughter is to the soul." Laughter increases tolerance to pain, shifts our perspective, increases our heart rate producing more oxygen flow through our bodies, helps fight upper respiratory disease, and decreases stress hormones. Amazing, huh? Stream a funny show, watch a YouTube video, enjoy a favorite sitcom, or find someone (in person or virtually) to just spend some time to relax and laugh a little.

Self-Care Strategy #2 – Move: Notice I didn't say exercise (although that would be excellent if possible). Movement creates motivation. Take a moment to stretch (as tolerable based on your own individual circumstances). Movement can also involve deep breathing. Follow this train of thought: (1) movement increases oxygen in our cells; (2) cells use oxygen to break down sugar; (3) breaking down sugar produces energy our bodies need. Find a movement or exercise strategy that works for you and consult with a recommended physical therapist. You'll be glad you did!

Self-Care Strategy #3 – Create Rituals: We all need something to look forward to each day—it doesn't have to be gargantuan or pricey. A ritual is simply a positive action that is performed at predictable times. Here are a few examples of common rituals—reading a book while taking a bath, playing games, going for walks, attending the gym, calling a friend, going for a drive, meeting up with friends before or after work (in person or virtually), etc. Rituals aren't only meant to be carried out during the holidays. They can be put in place daily. Give it a try!

Self-Care Strategy #4 – Socialize: Among the many fallouts of the pandemic, our ability to socialize has been rudely compromised. Studies have made it abundantly clear that the lack of social connection and support lead to increased stress and increased mental health problems. Even though we're compromised somewhat, here are a few ideas: create or participate in a virtual environment where people can just open up and socialize; play virtual games; based on local restrictions, meet up masked and socially distanced with a friend or two; serve someone you may know is struggling.

Self-Care Strategy #5 – Engage in Mindfulness: Mindfulness is simply being aware of your surroundings in an intentional, therapeutic manner. For example, being mindful of our thoughts can help us challenge thinking errors such as labeling, blaming, and catastrophizing. People engage in yoga, tai-chi, or other movement activities that promote connection with body, soul, and mind. Other activities include keeping a gratitude journal, deep breathing, progressive muscle relaxation, removing shoes and feeling the ground beneath one's feet, nature bathing (sometimes called forest bathing), and other techniques.

By following these offensive strategies, you will likely fill your body, mind, and spirit tanks to help navigate the current world circumstances. The time will soon come that this pandemic will be history and we can gather together once again—with more gratitude for our each other and our associations.

Matthew Barkdull, as a severe hemophilia B patient himself, is happy to offer his coaching/counseling services to any in the Coalition community. Feel free to reach out to him at 385-434-8136 or barkdullmd@paramountcounselingsolutions.com.

ARE YOU READY TO TELL YOUR STORY?

Whether you have an incredible career, an extraordinary family, or a tale of triumph, we want to hear from YOU! We will have you will collaborate with an in-house writer to help communicate your story in a compelling and meaningful way. The best part is that no previous writing experience is necessary. So, what's holding you back? For more information on being featured in the CHB newsletter, please contact us at contact@hemob.org. We can't wait to read all about you!

IDELVION®, Coagulation Factor IX (Recombinant), Albumin Fusion Protein
Initial U.S. Approval: 2016

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

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

Who should not use IDELVION?
You should not use IDELVION if you have had life-threatening hypersensitivity reactions to IDELVION, or are allergic to:
- hamster proteins
- any ingredient of IDELVION

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

What should I tell my healthcare provider before using IDELVION?
Discuss the following with your healthcare provider:
- Your general health, including any medical condition you have or have had, including pregnancy, and any medical problems you may be having
- Any medicines you are taking, both prescription and non-prescription, and including any vitamins, supplements, or herbal remedies
- Allergies you might have, including allergies to hamster proteins
- Known inhibitors to Factor IX that you've experienced or been told
- you have (because IDELVION might not work for you)

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

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

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

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

A common side effect of IDELVION is headache. This is not the only side effect possible. Tell your healthcare provider about any side effect that bothers you or does not go away.

Based on October 2019 revision

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

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

Emerging Therapies

BY DR. DAVID CLARK

Many of the following reports come from studies that were presented at the 62nd Annual Meeting of the American Society of Hematology (ASH), December 5 – 8, 2020. Copies of the abstracts (summaries) are available on the ASH website at hematology.org.

Catalyst Receives FDA Fast Track Designation for MarzAA

12/2/20 Catalyst Biosciences has received Fast Track Designation for marzeptacog alfa (activated) (MarzAA) from FDA. This gives them greater access to FDA during product development and an expedited review for licensure. MarzAA is a variant factor VIIa for treatment of hemophilia A and B patients with inhibitors. It has a higher activity, longer half-life and subcutaneous dosing for on-demand treatment of bleeds. [Catalyst press release 12/2/20]

12/6/20 At ASH, Catalyst presented the design for their Phase III study of MarzAA, which is scheduled to start in December. The trial will include approximately 60 A or B inhibitor patients enrolled at 54 sites in 19 countries. It will be a crossover study in which 30 patients receive MarzAA and 30 patients receive a current factor VIIa product for the first half of the study. Then, at the mid-point, they switch and the MarzAA group receives factor VIIa and vice versa. For more information look on clinicaltrials.gov at Study NCT04489537. [Catalyst press release 12/7/20 and ASH abstract 1795]

HEMA Biologics Reports on Clinical Study for Sevenfact

12/6 and 12/7/20 At ASH, HEMA Biologics reported on the clinical studies for their Sevenfact, variant factor VIIa product that was recently licensed by FDA. Sevenfact is a bypassing agent for treatment of hemophilia A or B patients with inhibitors.

One study looked at 396 joint bleeds in 50 inhibitor patients from the Phase III licensure study. They looked at doses of 75 µg/kg every three hours compared with an initial dose of 225 µg/kg followed by doses of 75 µg/kg every three hours starting nine hours after the first dose. At 3 hours after infusion, they found that 26.2% of the bleeds had resolved (stopped) for the 75 µg/kg initial-dose subjects and 63.3% resolved with the 225 µg/kg initial-dose. This suggests a benefit of quicker resolution with a higher initial dose. At 24 hours, the results were similar at 94.4% and 94.5% of bleeds resolved, respectively.

They also conducted a Phase III study of twelve male severe hemophilia A subjects with inhibitors having various major or minor elective surgical procedures. These included several orthopedic procedures that generally involve a lot of bleeding. They looked at two different dose regimens and evaluated bleeding at various points during and after surgery. The results for bleeding control were excellent during the procedures and good or excellent 24 hours after surgery. Blood loss during surgery was less than that seen in patients without bleeding disorders undergoing the same procedures. Two orthopedic patients were considered failures. One hip-replacement patient required a blood transfusion due to anemia after surgery. A knee-replacement patient withdrew because of a post-surgery hematoma. There were no thrombotic events and no evidence of inhibitor formation. [ASH abstracts 1790 and 2699]

Tremeau Receives Funding for Rofecoxib Development

12/9/20 Tremeau Pharmaceuticals announced that they have received funding for their development of TRM-201, rofecoxib, for the treatment of pain in hemophilic arthropathy (joint damage). Rofecoxib is a COX-2 inhibitor that was previously marketed as VIOXX. In addition to excellent pain relief, it also had the advantage over other COX-2 inhibitors that it had a reduced risk of gastrointestinal bleeding.

VIOXX was discontinued in 2004 because of concerns about cardiovascular safety. Research since then has shown that all NSAIDs have cardiovascular safety issues that depend on dose and duration of treatment. Presumably, some people on VIOXX were using large amounts of the then seemingly-harmless drug, which led to the cardiovascular issues. Tremeau believes that they can safely bring rofecoxib back as TRM-201 when used in moderate doses.

Tremeau recently had an “End of Phase II” meeting with FDA, which approved their results and gave permission to begin a Phase III study aimed at licensure. They plan to begin the Phase III study in early 2021.

Pain from damaged joints can have a huge negative impact on quality of life for hemophilia patients. Many found great relief with VIOXX and were sadly disappointed when it was removed from the market. It was reportedly more effective than opioids, which have their own problems. Its potential return to the market will be welcomed. [Tremeau press release 12/9/20]

Anticoagulant-Inhibitor Therapies

A number of companies are developing products that inhibit an anticoagulant in the clotting system. A normal clotting system contains both clotting factors, which promote clotting, and anticoagulants, which retard clotting and help control the system. These are in balance so the blood clots when needed but resists thrombosis, which is too much or unwanted clotting. Thrombosis can cause serious complications and even death.

In hemophilia, the system is out of balance because of the missing clotting factors. This tilts the system from the neutral balance point toward having difficulty in developing clots at all. By inhibiting the anticoagulants (inhibiting the inhibitors), these new products are expected to re-balance the system to allow clots to form when needed. A number of different anticoagulants are being targeted including antithrombin, tissue factor pathway inhibitor (TFPI), protein C and protein S.

(Note that these inhibitors are not the same substances as the inhibitors that we normally talk about in hemophilia, which block the action of factor VIII or IX. There are many kinds of inhibitors in biology, and now that hemophilia research has come more into the mainstream, we may need to adopt a more specific name for clotting factor inhibitors. Here, we'll just call them factor-inhibitors.)

Inhibiting anticoagulants has worked out fairly well so far in clinical studies of the products being developed. A big advantage is that these methods work for all types of hemophilia, A or B, with or without factor-inhibitors. However, it appears that the re-balanced clotting systems are not very robust. Inhibiting the anticoagulants, which control the system, creates a delicate balance that is easily disrupted. There have been problems with thrombosis in several of the studies, a couple of which are described below.

The biggest issue seems to be in treating breakthrough bleeds, bleeds that occur in spite of the treatment. The tendency has been to treat the bleeds the same way that you would treat a bleed in a patient who was not on an anticoagulant-inhibitor therapy, with either factor products or bypassing agents. That appears to be too aggressive an approach in some patients. It pushes the balance too far back toward easy clotting, which can lead to thrombosis. We may need to use a lighter touch.

The reports below give an update on the studies for several of the anticoagulant-inhibitor products under development. The most important, though, are the studies on thrombin generation. We normally use coagulation factor assays in hemophilia treatment that measure the level of either factor VIII or factor IX. Those were fine when all we were doing was to give patients factor products to raise their levels. Now that we're trying to tweak the entire clotting system, those assays might not be good enough. We need an assay that looks at the whole system.

A number of researchers over the years have suggested that thrombin generation assays (TGAs) would be much better at looking at the whole system. TGAs measure the production of thrombin, which is the final active enzyme in the clotting cascade. Thrombin converts fibrinogen to fibrin, which is a protein that sticks to itself to form the actual clot, sometimes called a fibrin clot. The amount of thrombin produced, as well as the time course of the production, gives an overview of the whole clotting process, not just the factor VIII/IX step. Unfortunately, TGAs are not ready for prime time – they are OK in the research lab but still need work before they can be used for everyday testing in a commercial clinical lab.

A TGA takes into account the actions of all of the clotting factors and anticoagulants in the system. It might be a much better assay to use when treating a breakthrough bleed with these new products. It might show, for instance, that the patient with the bleed only needs a little extra clotting factor rather than a full dose that might send her/him into thrombosis. For instance, a TGA study by Novo for its concizumab product shows that only about half the usual amount of factor IX is needed to treat a bleed in a patient on concizumab.

Note that wider adoption of TGAs might also solve one of the mysteries of hemophilia – that patients don't always clot as suggested by their factor levels. For instance, we know that about 15% of severe hemophilia B patients (<1% of normal factor IX activity) actually clot more like mild or moderate patients. We've been learning that this is partly because they may have non-normal amounts of other clotting factors and/or anticoagulants. Too often, it is assumed that while a hemophilia B patient has a low factor IX level, all of her/his other clotting factor and anticoagulant levels are normal or average. That's a huge assumption that is not always true.

These anticoagulant-inhibitor treatments under development could turn out to be excellent products and a good alternative for patients who can't or won't have gene therapy. Most of the products are dosed by simple subcutaneous injections, sometimes weekly or even monthly. We have to learn to control them better, though. We can't just assume that when we remove part of the control system of the clotting cascade that everything will be OK. Nature designed the system to be robust and yet flexible, but we can't just throw anything at it and expect it to work. We have to think carefully about what we're doing. The studies show that companies are doing just that.

Novo Reports on Thrombotic Events in Concizumab Study

12/6/20 Novo Nordisk is developing concizumab, a subcutaneous monoclonal antibody that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant that helps control the clotting reactions. By inhibiting TFPI, they hope to restore the balance in the clotting system to permit normal clotting. After

results of Phase II studies showed that concizumab was well-tolerated and had a favorable safety profile, Novo has now taken the product into Phase III studies aimed at licensure. However, in March 2020, three subjects developed non-fatal thrombotic events. The three subjects all had hemophilia A or B with factor-inhibitors. All three had used bypassing agents either on the day of, or in the days immediately before, their concizumab injections. The study was halted temporarily and then re-started after Novo and FDA agreed on modifications to the study protocol, especially in regard to use of bypassing agents.

Although bypassing agents are the mainstay of inhibitor treatment, they are still not well understood. Throwing them into the mix with anticoagulant-inhibitors can have unexpected consequences, as we have seen. [ASH abstract 1796]

Novo Reports on Additional Studies in Support of Concizumab

12/7/20 Novo Nordisk also reported the results of three other studies of concizumab at ASH. In a continuing follow-up of patients who had received concizumab in their Phase II study for up to 76 weeks, they reported on safety and efficacy for 15 hemophilia A patients and ten hemophilia B patients, all with factor-inhibitors. This included eight patients in the control group who had received factor VIIa during the initial study but were switched to concizumab afterward. There were no adverse events leading to withdrawal from the study and no thrombotic events. Six patients developed antibodies against concizumab, but these were apparently non-inhibitory. [ASH abstract 2696]

Novo also reported on the pharmacokinetic (PK) model that they developed for dosing patients on concizumab. The model was used to select the doses for the Phase III study. [ASH abstract 2701]

They also reported on a laboratory study of coagulation in plasma from hemophilia A and B donors using a thrombin generation assay. They found an additive effect of concizumab that suggested, for instance, that only about half the normal factor IX dose would be needed to treat a breakthrough bleed in a patient on concizumab. [ASH abstract 1777]

Pfizer Updates on Marstacimab Development

11/23/20 Pfizer is developing marstacimab, also a TFPI inhibitor. They have completed their Phase II study and have now treated the first patient in a Phase III study aimed at licensure. The Phase II study showed a greater than 75% reduction in annualized bleeding rate (ABR) with no treatment-related serious adverse events and no thrombotic events in all 20 patients for up to 12 months. Marstacimab is a weekly subcutaneous product. [Pfizer press release 11/23/20]

12/6/20 At ASH, Pfizer also presented a study of thrombin generation and D-dimer levels in marstacimab patients from their Phase I and II studies. D-dimer levels are often used indicate thrombosis (unwanted clotting) in patients. D-dimer is a protein fragment formed when a clot breaks down. Although it might seem counter-intuitive, the body starts breaking down clots as soon as they are made. A high level of D-dimer indicates that there has been a lot of clotting activity.

Pfizer showed that marstacimab patients who developed breakthrough bleeds and were treated with factor VIIa, VIII or IX did not develop higher thrombin or D-dimer levels than patients who did not develop bleeds. Note that factor VIIa, but not FEIBA, was used for the factor-inhibitor patients who developed breakthrough bleeds. So far, marstacimab has not appeared to cause thrombosis in the studies. Whether that is because of the characteristics of marstacimab itself, the study design, the amounts of factor infused, the characteristics of the individual patients or something else is unknown. [ASH abstract 1789]

Sanofi's Fitusiran Study Placed on Hold and Then Restarted

12/11/20 Sanofi Genzyme is developing fitusiran, a drug that reduces the body's production of antithrombin. Antithrombin is an anticoagulant that inhibits clotting and is part of the control system for the clotting cascade. Reducing the amount of antithrombin anticoagulant activity helps to restore normal clotting in hemophilia patients. Fitusiran, an RNA interference drug, was originally developed by Alnylam before being bought by Sanofi about three years ago.

Fitusiran's Phase III study was voluntarily halted by Sanofi after some patients developed non-fatal thrombotic events. These were factor-inhibitor patients who had been given bypassing agents to treat breakthrough bleeds. After investigating the incidents and reviewing their data with FDA, Sanofi now is re-starting the studies with new dose and dosing guidelines. More details will be released shortly.

This follows a previous halt to their Phase II and III studies in 2017 after a non-factor-inhibitor hemophilia A patient died from a blood clot in his brain. Because he was originally diagnosed as having a brain bleed, he was given large doses of factor VIII, which might have led to formation of the clot. [Conversations with Sanofi 11/11 and 12/11/20]

Other Fitusiran Reports from ASH

12/5 and 12/7/20 Sanofi also presented five studies on fitusiran at ASH. Two were summaries of their Phase I and Phase II studies of fitusiran. A third showed that patients on fitusiran have an improved Health-Related Quality of Life. Two others looked at the behavior of fitusiran-treated patients' blood in coagulation assays.

One assay study shows that the low antithrombin levels in blood samples from fitusiran patients do not interfere with assays to determine their factor VIII or IX levels. The other is more interesting and might have some bearing on the thrombosis issues described above.

The investigators used thrombin generation assays (TGAs) to mimic what happens with breakthrough bleeds in fitusiran patients. First, they found that the thrombin-generation potential of subjects treated with fitusiran approaches that of untreated non-hemophilic subjects. Their results also suggested that TGA might be a valuable tool for monitoring patients on fitusiran. [ASH abstracts 511, 857, 862, 877 and 2693]

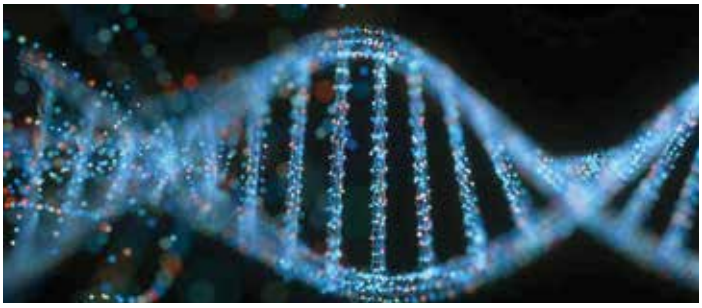
Silence Therapeutics Reports on Protein S Inhibitor

12/7/20 At ASH, Silence Therapeutics, a German company developing RNA interference (RNAi) drugs, presented the results of a study looking at inhibiting the production of the anticoagulant protein S to treat hemophilia. RNAi is a method for reducing or eliminating the production of proteins in the body. Fitusiran, above, is also an RNAi drug. Silence showed that the use of GalNAc-PS siRNA in a mouse model of hemophilia A reduced protein S levels and improved clotting times. They also showed that it significantly reduced bleeding and swelling in the knee joints of the mice. They have an ongoing project to further examine the use of protein S reduction in treating hemophilic arthropathy (joint damage). [ASH abstract 627]

GENE THERAPY

GeneVentiv, a Hemophilia Gene Therapy Company Spun Out from UNC-Chapel Hill

11/2/20 GeneVentiv Therapeutics, a new gene therapy company, has been spun out from the University of North Carolina at Chapel Hill (UNC). They have licensed a patent-pending technology from UNC developed by Chengwen Li, the company's co-founder and scientific adviser. Their GENV HEM is a factor Va gene therapy for all hemophilia types, A or B with or without factor-inhibitors. Factor Va is a co-factor that increases the activity of activated factor X. They believe that having a higher circulating level of factor Va will improve clotting, while the activated Protein C system will prevent thrombosis. Few other details are available. [GeneVentiv press release 11/2/20]



uniQure/CSL Announces Phase III Gene Therapy Data

11/19/20 uniQure presented interim data for their etranacogene dezaparvovec (aka AMT-061) gene therapy for hemophilia B. This is the first data to be reported from a Phase III gene therapy study for hemophilia B. The treatment consists of a high-activity Padua factor IX gene delivered with an AAV5 vector. The product has been purchased by CSL Behring in a deal worth up to $2 billion. uniQure will continue the clinical studies until licensure.

After 26 weeks of follow-up, the 54 subjects with severe or moderately-severe hemophilia B achieved an average factor IX level of 37.2% (range 1.0 – 97.1%). Thirty-eight of the patients (42.6%) had anti-AAV5 antibodies prior to the treatment. uniQure had previously shown that anti-AAV5 antibodies would in most cases not prevent the treatments from working, and so did not exclude those with antibodies from the study. Only the patient with the highest antibody level did not respond to the treatment – that's the patient with only a 1% post-treatment factor level in the above range. All but two of the patients were able to discontinue routine prophylaxis. In 39 patients (72.2%), there were zero bleeds in the first 26 weeks after treatment. In 15 patients, there were 21 bleeds after treatment.

There were a few mild adverse events, but nothing severe. Seven subjects experienced infusion-related reactions, and one subject only received a partial treatment because of a significant reaction. Liver inflammation was observed in nine subjects and was treated with steroids per the study protocol. The most frequent adverse events were headache and flu-like symptoms, each in seven patients. No inhibitor development was observed.

All patients will have concluded a one-year follow-up by the first quarter of 2021. [uniQure press release 11/19/20 and ASH Abstract 672]

uniQure Presents Five-Year Data for AMT-060

12/7/20 uniQure also presented five-year follow-up data for their AMT-060 gene therapy treatment. AMT-060 was their original gene therapy candidate, which used a normal (wild-type) factor IX gene. It was later replaced by AMT-061, which has a higher-activity Padua factor IX gene. uniQure has continued to follow the subjects treated with AMT-060, who have now reached up to five years post-treatment in a Phase I/II study. These include five subjects

who received a 5 x 1012 gc/kg dose and five others who received a higher 2 x 1013 gc/kg dose. ("gc" refers to "gene copies" and is essentially equivalent to the number of active AAV5 vector particles received.)
The low-dose group had average post-treatment factor IX levels of 4.4%, 6.8%, 7.3% and 5.1% at years one through four. The high-dose group had levels of 7.1%, 8.4%, 7.9%, 7.4% and 7.5% at years one through five. Levels for the low-dose group appear to have increased over time to a peak and now are lower. In contrast, levels for the high-dose group seem to have remained steadier. There were no significant safety concerns and no signs of inhibitor development. [ASH abstract 3373]

uniQure Reports on Gene Therapy Durability in Newborn Mice

12/6/20 The AAV vectors being used in all of the gene therapy clinical studies so far insert their new factor IX genes into the nucleus of liver cells where the genes remain as separate small entities called exosomes. The new genes do not insert themselves in with the cell's other genes on the chromosomes, that is, they do not "integrate into the genome." The genome is the collection of all the genes in an organism. Almost every cell in the organism contains a complete copy of the whole genome in its nucleus. The genes are mounted on chromosomes, which are scaffolds that hold and organize the genes in the nucleus of the cell.

Using non-integrating genes has been thought to be the safer approach because when a gene randomly inserts itself onto the chromosomes, it can disrupt other genes. The downside is that scientists believe that the separate small genes are lost when the cell divides. Young people have rapidly growing livers, which grow by cell division – one cell becomes two cells and so on. Thus, it has been assumed that AAV gene therapy will not work in younger patients.

uniQure's new study shows that this might not be a valid assumption, at least in mice. They dosed normal mice at various stages of life ranging from two days-old to six months (adult mice) with a human factor IX gene therapy. They found that all of the mice, even those dosed at two days old, retained all of their factor IX expression up to 18 months after dosing. uniQure is conducting ongoing studies to explore this further. [ASH abstract 2452]

Are AAV-Delivered Genes Truly Non-Integrating?

11/16/20 The other question going along with the above uniQure study is whether new genes delivered by gene therapy using AAV vectors really don't insert themselves in among the other genes on the chromosomes. In the report of a ten-year study of hemophilia A dogs treated with factor VIII gene therapy, a group of U.S. researchers found evidence that the genes may, in fact, integrate into the genome. In two of nine dogs treated with the gene therapy, the researchers observed an increase in factor VIII levels starting about four years after treatment. Analysis of liver cell biopsies from six of the dogs showed a small number of cases in which the new gene had integrated into the cell's genome. They also found cells that had apparently divided after receiving the new genes, and that both the new and original cells produced factor VIII. This is in line with what uniQure reported above – that liver cells could divide and not lose the new gene.

The concern is that the AAV-delivered genes did integrate into the genome in random places, and 44% of the integrated genes were inserted near cell growth genes. The problem is that disrupting cell growth genes could lead to cancer; cancer is uncontrolled cell growth. None of the dogs showed evidence of cancer or altered liver function, but the study results suggest that such developments might be possible. The authors emphasize the importance of long-term monitoring of gene therapy patients for genetic issues like these.

Both of these studies show that there is still a lot to learn about the actual effects of gene therapy on the body. [Nguyen GN et al., Nature Biotechnology, online ahead of print, 11/16/20]

FDA Puts Clinical Hold on uniQure Gene Therapy Study

12/21/20 uniQure announced that FDA has placed a clinical hold on their Phase III gene therapy study of AMT-061 after a patient in the study was given a preliminary diagnosis of hepatocellular carcinoma, a form of liver cancer. The investigators found a liver mass during a routine ultrasound examination and will perform a biopsy to determine whether it is actually cancer. The patient had multiple risk factors including a 25-year history of hepatitis B and C, evidence of non-alcoholic fatty liver disease and advanced age, all of which predispose him to liver cancer. The patient was treated with AMT-061 in October 2019. uniQure is currently performing the required investigations. [uniQure press release 12/21/20]

Patient Perspectives on Gene Therapy

11/7/20 A group of 20 Belgian hemophilia patients (17 As; 3 Bs) were interviewed concerning their perspectives on gene therapy. Most (80%) had severe hemophilia and 90% had moderate or severe joint damage. 75% were on prophylaxis and 65% had already discussed the possibility of gene therapy with their physician. Most participants had a positive attitude with 40% being very willing and 35% being willing to receive the treatment.

Five aspects turned out to be most important to the group: 1) effect on annual bleeding rate, 2) factor level after treatment, 3) uncertainty of long-term risks, 4) impact on daily life and 5) probability that prophylaxis could be stopped. The group was more concerned about long-term safety than about long-term factor levels. [van Overbeeke E et al., Haemophilia, online ahead of print 11/7/20]

RIXUBIS® [Coagulation Factor IX (Recombinant)] Important Information

What is RIXUBIS?

RIXUBIS is an injectable medicine used to replace clotting factor IX that is missing in adults and children with hemophilia B (also called congenital factor IX deficiency or Christmas disease).

RIXUBIS is used to control and prevent bleeding in people with hemophilia B. Your healthcare provider may give you RIXUBIS when you have surgery. RIXUBIS can reduce the number of bleeding episodes when used regularly (prophylaxis).

Detailed Important Risk Information for RIXUBIS® [Coagulation Factor IX (Recombinant)]

Who should not use RIXUBIS?

You should not use RIXUBIS if you

- are allergic to hamsters
- are allergic to any ingredients in RIXUBIS.

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

What should I tell my healthcare provider before using RIXUBIS?

You should tell your healthcare provider if you

- have or have had any medical problems
- take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies
- have any allergies, including allergies to hamsters

What should I tell my healthcare provider before using RIXUBIS? (cont'd)

- are breastfeeding. It is not known if RIXUBIS passes into your milk and if it can harm your baby
- are pregnant or planning to become pregnant. It is not known if RIXUBIS may harm your unborn baby
- have been told that you have inhibitors to factor IX (because RIXUBIS may not work for you).

What are the possible side effects of RIXUBIS?

Allergic reactions may occur with RIXUBIS. Call your healthcare provider or get emergency treatment right away if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.
Some common side effects of RIXUBIS were unusual taste in the mouth and limb pain.

Tell your healthcare provider about any side effects that bother you or do not go away.

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

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

Please see RIXUBIS Important Facts on the following page and discuss with your healthcare provider.

Takeda

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

Important facts about RIXUBIS®:

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

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

What is RIXUBIS used for?

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

Who should not use RIXUBIS?

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

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

What should I tell my healthcare provider before using RIXUBIS?

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

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

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

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

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

What are the possible side effects of RIXUBIS?

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

What else should I know about RIXUBIS?

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

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

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

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

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

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

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Takeda

WOMEN'S RETREAT FALL 2020

SPONSORED BY

Pfizer

BY CHRIS VILLARREAL AND DR. APRIL WILLIS

Our community entered a period of separation anxiety when the Coronavirus pandemic changed the world in so many ways. As we learned we would not be able to see each other in person, our Coalition adapted and learned how to recreate that sense of connection and belonging that our community was craving during these times through the land of Zoom!

With the generous support of our sponsor Pfizer, we held our Virtual Women's Retreat in the fall. The first day of the Retreat began with a program called *Chit, Chat, and Chocolate*, moderated by Dr. Mina Nguyen-Driver, a trained mental health expert in hemophilia. This has always been one of our most powerful sessions where the voices of women in the community from different walks of life and situations, openly share their story of struggles, passion, and triumphs. Through laughter, tears, and chocolate, the ladies connected and bonded during this powerful emotional experience. Afterward, they came together with professional artists that led them in a *Paint and Sip* session where they painted beautiful trees with different hues of flowers and leaves. Each a unique piece just as unique as each beautiful woman is.

Saturday started with meditation through dance, presented by Claire Louise Knifton from Ireland (see…Zoom can unite us worldwide!). The feedback on this practice of dance and meditation was very well received and welcomed by the women to help them relieve their stress. Afterward, Catherine Canadeo, an integrative nutritionist, talked about tapping into the intention to heal from within. It's not always what you're eating, but what might be eating you! Catherine's presentation delved into deep discussion and shared a multitude of tools ranging from food choices and balance to setting healthy boundaries for your overall well being. Incorporating a good regimen of daily habits is one of the best ways to create a happy healthy lifestyle for themselves and their families.

"I really enjoyed connecting with other moms and learning from others' experiences. Overall, the theme of self-care helped me realize that I can only be a true advocate for my family when I take a little time to reset myself as well. Thank you to the Coalition for a great weekend!" - S.S. from Wisconsin.

In the session on *Adversity, Resilience, and Strength*, part of Pfizer's program series was held with speakers Patty Eastin and Rachel Cooper-Leal. Women learned tools to help them cope with chronic illness in their family. These included tools to empower them in several areas including how to communicate with their healthcare team, financial considerations through life stages, and the importance of staying physically active, to name a few. Later that day, we were treated to a wonderful film documentary on the importance of connections featuring Lee Kim. Afterward, we went into breakout sessions to share our individual story about something we love. Each person had a chance to tell their story while another person would create it through pipe cleaner creations, later shared with the group.

Jillian Richardson, a certified coach who works with people one-on-one and in groups to help them establish deeper connections and relationships in their lives, joined us. Through her *Better Boundaries* workshop, led us on a journey of learning to say "no" to what we no longer will do and how to set those healthy boundaries. She equipped all with tools and proper response techniques to keep them from always saying "yes" which often leads to feelings of being overwhelmed and resentful. Saturday concluded with an exciting pajama party! This fun-filled night was chock-full of games that included tongue-twisting word games like *MadGabs*, a rambunctious game of *Pocketbook Bingo*, and our trivia gurus loved the online trivia game, *Kahoots!* With laughter, the women remembered that it is important to have a little fun once in a while because a smile is a curve that sometimes can set things straight!

On Sunday, attorney Donnie Akers presented very important legal tools for transitioning youth to adulthood, the different types of medical authorizations &consent to treatment, powers of attorney, and estate planning to prevent loss of Medicaid. Catherine led a closing vibrational healing session and shared the importance of telling your story to awaken your senses to feel good emotions, creating a sense of completion for all retreat attendees.

During the final closing chat, the women took time to reflect on the weekend retreat and all they learned. "The 2020 Hemophilia B Women's Retreat was an outstanding success despite being a virtual engagement."

The weekend was overflowing with exceptional programs,speakers, coaches, and motivators. It provided a unique time to reconnect, refresh, and be enlightened. We loved spending time with each of them and hearing from all of the beautiful ladies who connected, shared, and supported each other in so many ways. The strong bonds of this weekend will carry throughout the rest of their lives.

The world may be in mayhem but nothing should stop us from connecting! Connections are vital! We are very grateful to our sponsor Pfizer for making this possible and their continued support.

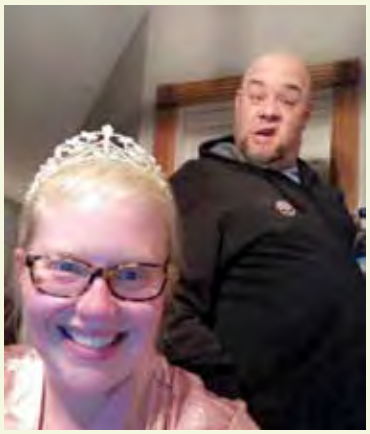
"No matter what we go through, we are blood sisters. Our connections are strong and I am so grateful for this program – it's priceless!" - E.D. from Illinois.

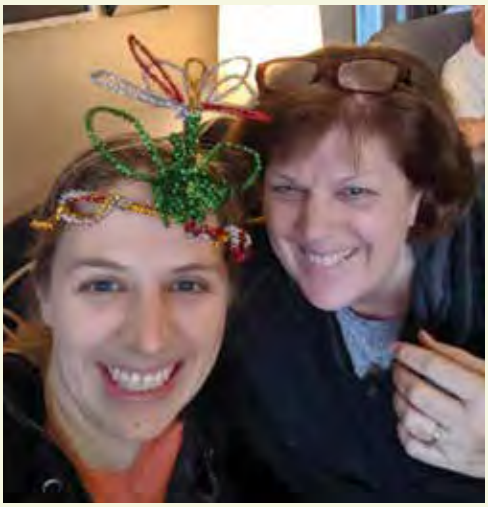
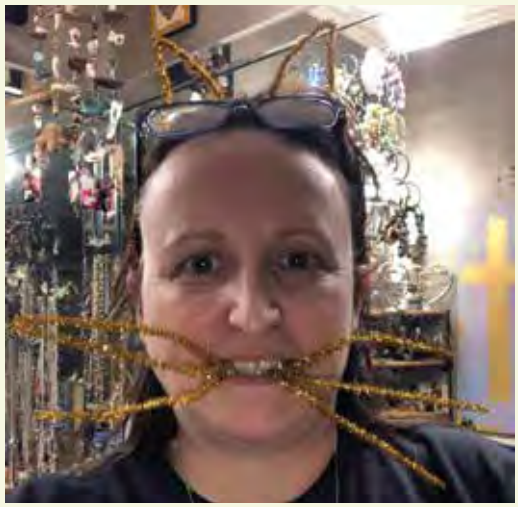
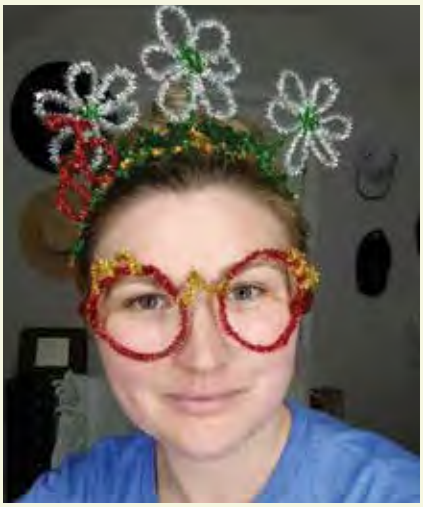


To view more photos, please visit us on Facebook! Click on *Photos* where you will find event albums.

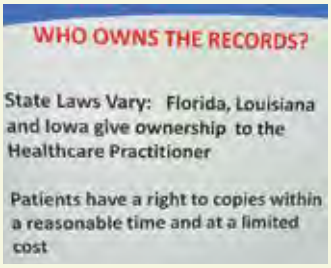
www.facebook.com/HemophiliaB/

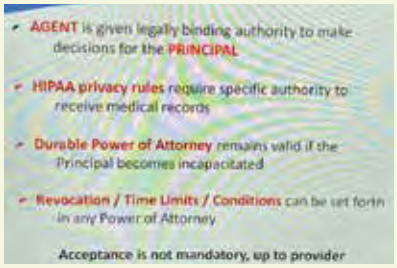


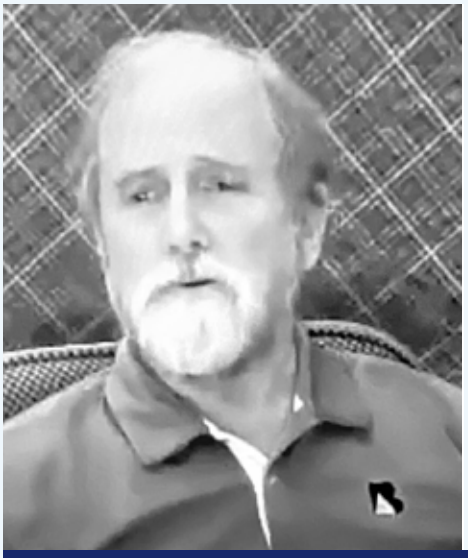


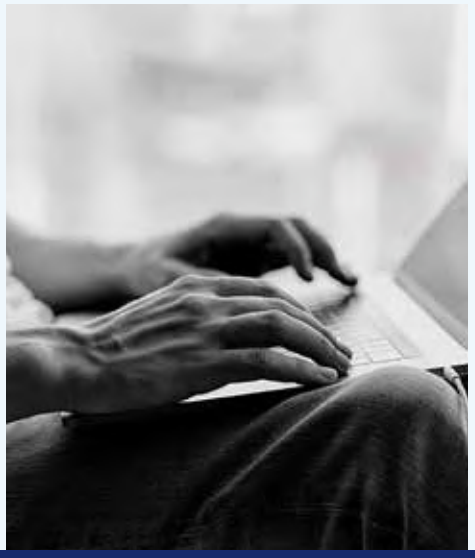














MEN'S RETREATS FALL & WINTER 2020

SPONSORED BY Pfizer

BY GLENN MONES

On the weekends of November 20-22 and December 11-13, 2020. Two groups of men from across the country gathered virtually for the 2020 The Coalition for Hemophilia B Men's Retreats. Participants included men with hemophilia B, fathers of children with hemophilia B, and spouses of those with hemophilia B.

As with many Coalition events this year, the program had to be held virtually because of travel restrictions and other safety requirements during the pandemic. Despite these circumstances, the men who participated found a warm, welcoming environment, a place to share feelings, to make new friends, to gain knowledge and empowerment and share life's challenges.

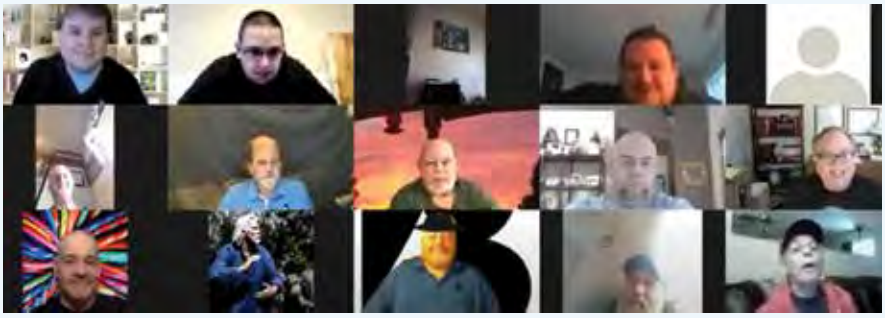
An important component of the retreats were the educational sessions, some of which were the same at both retreats and others that were different. This year's retreats brought a new emphasis on mental health and related psychosocial issues. The November session featured a session called *Mental Health and Covid*, led by community member Matt Barkdull. The session offered tools for dealing with the unique challenges these times present. Another session, called *Meditation and Depression*, was led by internationally known speaker and author Robert Friedman. The December program featured another session with Robert Friedman called *Mastering Self-Care During Covid-19*. The session talked about how to enhance emotional, mental and physical well-being during challenging times and turn those times into opportunities for growth.

Participants attended a variety of other sessions on subjects that included use of medical cannabis for pain, aging with hemophilia, getting and keeping a job under the ADA with Donnie Akers, Jr. Esq., and many others. As importantly, attendees had the chance to share their feelings and provide each other with support. Most participants have told us that those are their favorite parts of the program.

Both programs also included some fun sessions designed to let the participants just relax and be themselves. The one that perhaps stands out the most was the *Murder Mystery* held Saturday evening during the December event. A group of participant volunteers, aided by an outside facilitator, acted out a perplexing crime drama that the others had to solve. The players got into the spirit with costumes and makeup, and many were good sports about accepting roles of either gender!

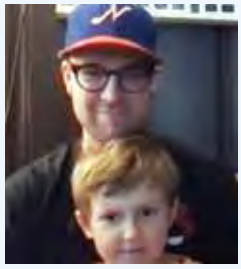
The Coalition for Hemophilia B is grateful to Pfizer for their exclusive sponsorship of both Men's Retreats. We look forward to holding these events in person again in 2021. Stay tuned for more details!

To view more photos, please visit us on Facebook! Click on *Photos* where you will find event albums.
www.facebook.com/HemophiliaB/

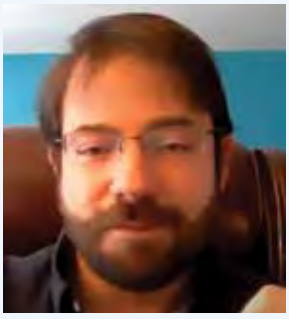


FALL RETREAT NOVEMBER 20-22, 2020

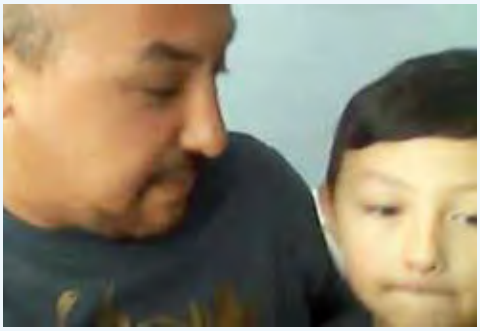


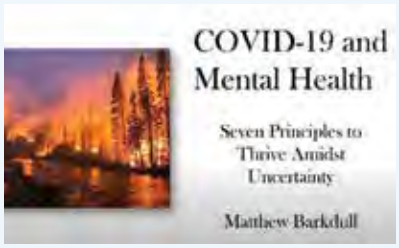



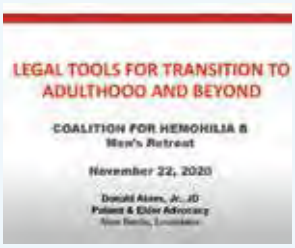


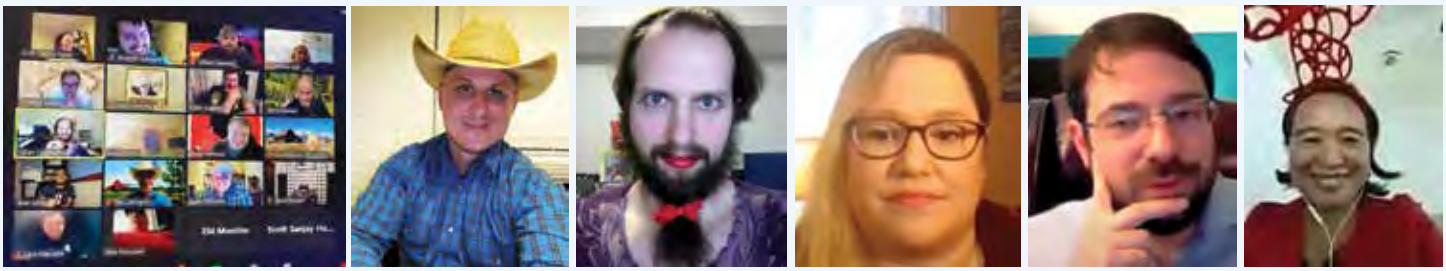




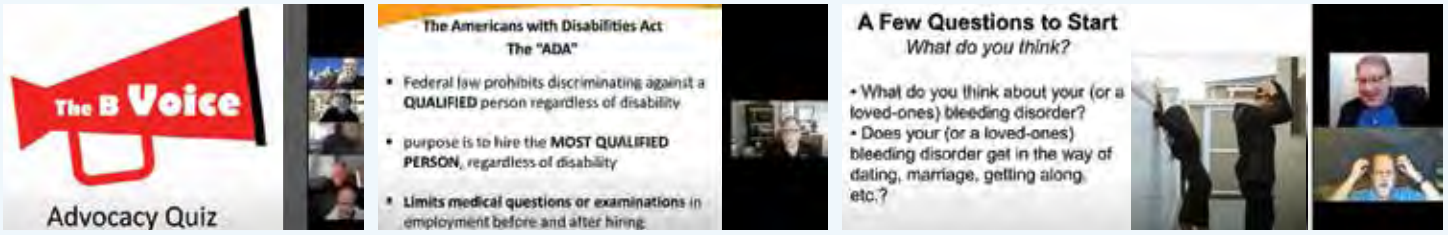



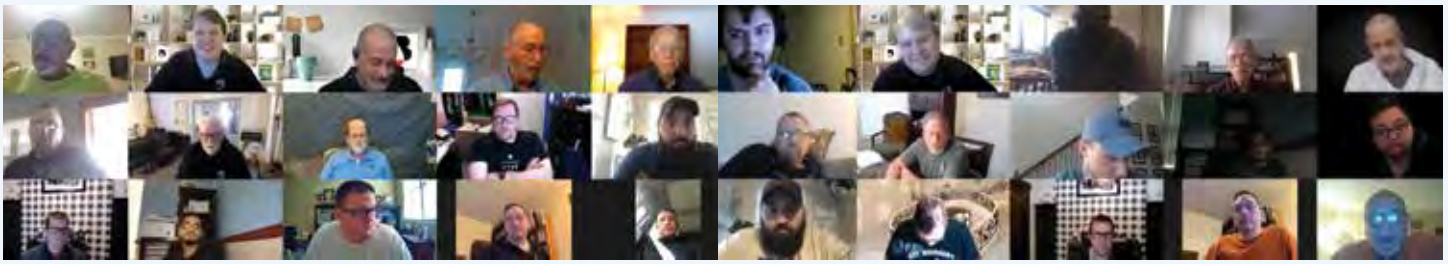



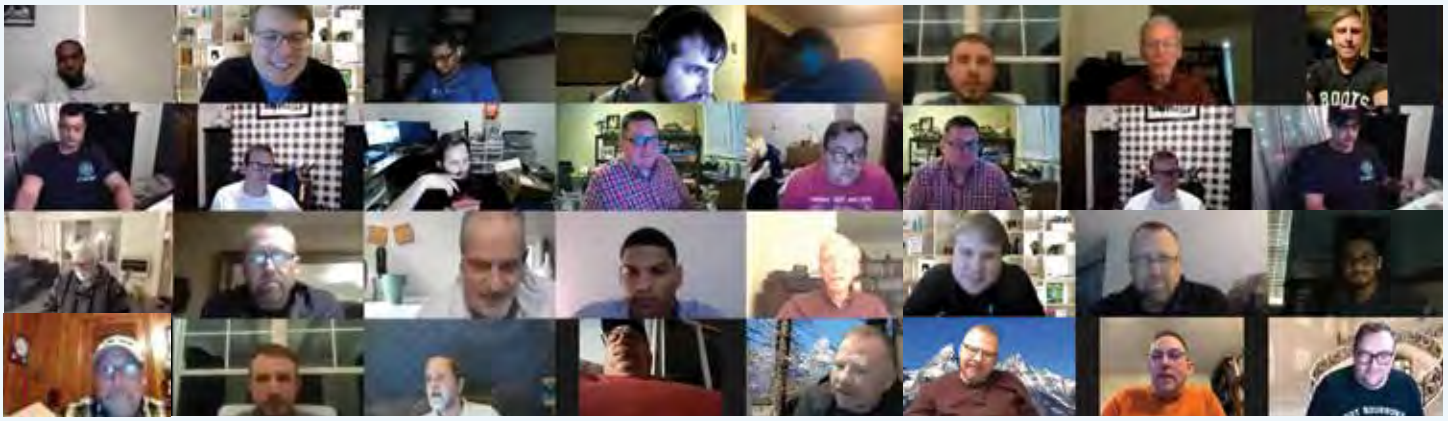


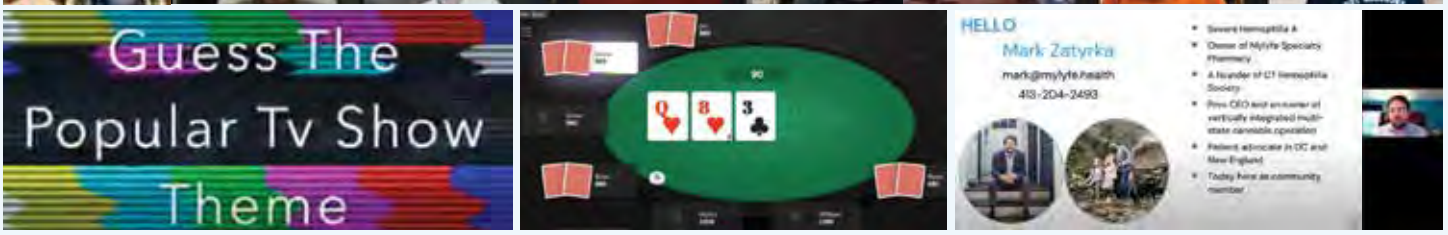


WINTER RETREAT DECEMBER 11-13, 2020









“THE BEATS” GO ON!

BY GLENN MONES

On October 23-24, 2020, with a song in our hearts, 25 people from around the country with a shared interest in music and hemophilia B came together on Zoom for our second annual “The Beats” music program. This groundbreaking program started in 2019 with an in-person inaugural event in Nashville, Tennessee, one of the true music capitals of the world.

It was created when several community members who are deeply involved in music, noted that some of the traditional programs did not speak to everyone. They knew there were others looking for ways to explore their creative side as a community and that music was a great way to make that happen.

They also understood the power of music to heal and nurture both the body and the mind was well-documented by science and medicine. Leaders of The Coalition for Hemophilia B had the vision to create a program that would make this dream a reality and The Beats was born! Our first event was wildly successful, and the participants and other community members couldn't wait for the next edition.

Though we could not meet in person this year, our 2020 program turned into a 2-day virtual "Mini Beats" event. The program featured a variety of opportunities for the participants to learn as a group and included individual lessons with renowned teachers in their chosen instrument, including guitar, drums, trombone, and vocals.

Subjects included music recording techniques, the healing power of music focusing on the benefits of rhythm-based wellness exercises to help attain psychological, emotional and physiological well-being, and writing music from the heart.

Most importantly, attendees had opportunities to connect musically with others to brainstorm concepts, share new ideas, hone skills, increase confidence, and build new friendships. Participants were encouraged to keep building on what they've learned and nurture the relationships they've created.

"Being able to express yourself through music has infinite benefits to each and every human," said Beats participant, Briana. Kathy, another participant, said, "I love the encouragement and support as a woman with hemophilia B." Brian said, "A big thank you for making this engaging for everyone, despite the challenges we're facing," Special thanks to our sponsors, Novo Nordisk and Medexus Pharma (Conductor Level), and CVS Specialty (Performer Level). We look forward to welcoming everyone to the third Beats program in Nashville in the summer of 2021.

This was truly a powerful and engaging experience for all participants. We encourage you to see the final night event, soon to be up on Facebook and be on the look out for applications for our 3rd event taking place in Nashville in July, 2021.

To view more photos, please visit us on Facebook! Click on *Photos* where you will find event albums.
www.facebook.com/HemophiliaB/

WOMEN'S RETREAT WINTER 2020

SPONSORED BY SANOFI GENZYME

While 2020 had its fair share of challenges, nothing was going to get in the way of our ability to have amazing Women's Retreats! Our event kicked-off with an art therapy *Paint and Sip.*" The participants gathered in two separate groups and professional artists led them through the process of painting a silhouette of a girl wandering the world with her red umbrella. Equipped with blank canvases, paints, and brushes, the ladies were encouraged to let their hair down and let their creative juices flow, as they each, with calm and simple strokes, let the colors take over to create a unique reflection of themselves. To wrap up the first day of the retreat, the participants went into a rap session *Chit, Chat, and Chocolate*, moderated by Dr. Mina Nguyen. This was a hot session where three women shared powerful stories, which opened the platform for all to share. This has always been one of our most intensely powerful programs.

Saturday kicked off with a gentle guided meditation through movement with Claire Louise Clifton, a qualified holistic trainer. The ladies learned to tune into themselves through slow meditative movements which allowed them to feel a sense of calm and centeredness to help them flow in their day. Afterward, we had a session on *Cognitive Conversations*, a Sanofi Genzyme speaker program "which explained how different parts of our brain work and why we make some of the choices we do." It explored insights from the field of behavioral science and how they can be applied to conversations related to managing hemophilia.

After a short break, the ladies joined together on camera and watched an inspiring short documentary, *Wearable Tracy*, featuring Lee Kim, as she explores and discovers a special way to connect with strangers through her pipe cleaner headwear. Using the drawings of her young, artistic daughter, Lee would create headpieces simply using pipe cleaners and wear them daily to connect with strangers wherever she went. Lee shared how powerful this simple gesture is and how it has turned frowns upside down, inspired conversations with others that mostly likely would never have happened, and brought joy to people. Lee worked directly with the ladies, pairing them off and having them create their own headwear inspired by each other's stories. Lee found an amazing way to remind ourselves that interpersonal connections are so important, especially now.

One of our first-time attendees from Illinois had this to say about her experience - “I can’t remember when I’ve had so much fun. I must admit that I have always wanted to attend a Women’s retreat, but due to family and work, I have never had the opportunity. Thanks to the recent events and generous sponsors, hosting a virtual meeting made it possible for me to join in and connect with my community.”

Afterward, the ladies went into a dinner break and upon return, Dr. Amber Federizo brought an empowering presentation on sexual health where the ladies quickly learned that they were not alone. To help get the “taboo conversation” out in the open, Dr. Amber brought the discussion to levels of bleeds and the different tools that women can use to cope with those bleeds. “Dr. Amber Federizo really opened my eyes to things I thought I was dealing with alone. Her insight and candor while discussing these sensitive topics really made it easy for us to open up and share our similar experiences.” - H.L. Memphis, TN.

Next, integrative nutritionist Catherine Canadeo led a talk about tapping into the intention to heal from within. Providing contemporary and traditional tips and tricks to add to their daily routine, Catherine shared her wealth of knowledge with the ladies. Saturday concluded with a fun pajama party! The ladies got to wear their favorite pajamas, crowns, and cozy socks and through laughter, the women remembered that self-care is more than internal work. It truly consists of fostering relationships and connections.

On Sunday afternoon, the ladies were joined by Catherine Canadeo for a self-care Sunday vibrational healing and the power of storytelling. Afterward, Jillian Richardson, a certified coach who works with people one-on-one and in groups to help them establish deeper connections and relationships in their lives joined us. Through her *Better Boundaries* workshop, she shared skills to establish healthy boundaries and understand the importance of self-care. We provided notebooks and comfortable space for the ladies to journal on the different ways they can apply Jillian’s self-care tools in their lives. Learning the importance of boundary setting helped empower the women to make room for their needs so they can be the best version of themselves and better help their family. Coupled with a great presentation from Donnie Akers on legal tools to help navigate health insurance, the women closed the weekend with a final look at all they learned.

Although our retreats looked different in 2020, the gatherings were filled with the same Connections, Education and Support! A very special thank you goes to our Sponsor, Sanofi Genzyme, for making this possible.

To view more photos, please visit us on Facebook! Click on *Photos* where you will find event albums. www.facebook.com/HemophiliaB/

THE COALITION GETS COOKING!

On Thursday, December 17th, members of the Coalition for Hemophilia B were treated to an evening of cooking, humor and inspiration with Cordon Bleu trained, Chef Mike Hargett.

The event was presented by BIOMATRIX and sponsored by Medexus Pharma. In addition to his accomplishments in the kitchen, Mike is known as the only double transplant recipient living with hemophilia. He has made significant changes to his lifestyle to maximize his personal health and quality of life. He also shares the details of his journey and everything he's learned to serve as an inspiration to others.

Chef Mike gave live demonstrations of how to prepare three easy and delicious dishes that anyone can make. They included pan seared chicken thighs with caramelized apple and onion sauce, garlic and herb smashed potatoes with sour cream and onion sauce, and sparkling holiday cranberry limeade. Mike carefully explained how to cook each component of the recipes and used a lot of humor to keep participants entertained and engaged. Some attendees even cooked along, and others took notes and plan to try the dishes in the future. The recipes are available from the Coalition.

Participants also heard a presentation called "Putting on the Mask First" given by Terry Rice, Director of Advocacy and Education at BIOMATRIX. The talk focused on the needs of caregivers for persons with a chronic illness. These virtual programs are a great change of pace during the pandemic, and to keep connections strong. Stay tuned for more events in the near future.

MEDEXUS PHARMA

[BIOMATRIX]

ENDING THE YEAR WITH LAUGHTER, FUN AND...GINGERBREAD!

1ST PLACE: AAMINA

On December 19, 2020, The Coalition for Hemophilia B brought a beloved holiday tradition to Zoom with our first Gingerbread House Competition!

Participants received kits in advance with all of necessary construction materials. Everyone had two hours to let their imaginations run wild as they created their edible masterpieces. More than a few young builders snacked on the decorations along the way. The construction process took place live so everyone could keep eyes on the competition while enjoying all of the creativity and cheering each other on! When the beautiful creations were done, we had to decide whether to eat them or move into them!

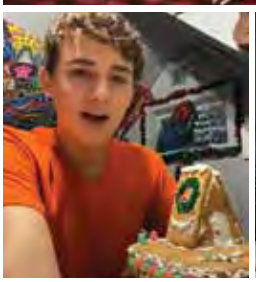
Congratulations to all of the winners including Aamina who's gingerbread house won the day. Lori's gingerbread house received the honor for second place, while Jordan and Melissa tied for third.

The evening ended with some fun *Kahoots!* trivia games featuring holiday traditions around the world and challenge questions to test your hemophilia knowledge. Congratulations as well to our trivia winners!

A special thank you to our program sponsors, Medexus Pharma and Paragon Hemophilia. Thanks to everyone who joined us for this fun end-of-year gathering. See you in 2021!

2ND PLACE: LORI

TIED FOR THIRD PLACE: JORDAN AND MELISSA



VIRTUAL MEETINGS ON THE ROAD

ALL MEETINGS ARE FROM 2:00- 6:00PM ON SATURDAYS

JANUARY 30 ILLINOIS, MISSOURI, MINNESOTA, IOWA, & NEBRASKA	**FEBRUARY 6** ARIZONA, UTAH, NEVADA, COLORADO, IDAHO, MONTANA, SOUTH DAKOTA, NORTH DAKOTA, WYOMING, & NEW MEXICO
FEBRUARY 20 MICHIGAN, WISCONSIN, AND KENTUCKY	**FEBRUARY 27** MARYLAND, DELAWARE, DISTRICT OF COLUMBIA, NORTH CAROLINA, VIRGINIA, TENNESSEE, & SOUTH CAROLINA
MARCH 13 CALIFORNIA, OREGON, WASHINGTON, HAWAII, & ALASKA	**MARCH 20** OHIO, INDIANA, OKLAHOMA, ARKANSAS, & KANSAS

*All are welcome to attend multiple meetings, but will only receive a box and dinner voucher once

30 YEAR ANNIVERSARY THE COALITION FOR HEMOPHILIA B

REGISTER NOW:
hemob.org/new-events/2020/10/24/virtual-meetings-on-the-road

GENERATION IX PROJECT

THE *VIRTUAL* LEADERSHIP EXPERIENCE

Young adults gather virtually for leadership training designed specifically for the Hemophilia B Community. Discover and develop new parts of yourself as a community leader that you can apply to personal, educational, professional and volunteer opportunities. Learn about leadership theory through experiential challenges with a team of motivated collaborators. Take part in virtual "hands-on" training components that make learning stick. Register early to receive a custom box of program materials to enhance your experience. This is *not* a typical webinar!

Ages: 18 to 35

Program Dates: Virtual Meetings on Tuesdays and Thursdays, March 1st to March 18th 2021

THE COALITION FOR HEMOPHILIA B PRESENTS

ARE YOU SMARTER THAN GLENN?

KAHOOT GAMES

RAFFLE PRIZES

CALLING ALL TEENS FOR A FUN NIGHT OF TRIVIA!

FRIDAY, JANUARY 29

7PM EST- 9PM EST

CHECK YOUR EMAIL FOR AN INVITE!

30 YEAR ANNIVERSARY
THE COALITION FOR HEMOPHILIA B

REGISTER FOR BOX DEADLINE BY FEB. 5, 2021

AGES 18-35 REGISTER AT HEMOB.COM

Brought to you by

30 YEAR ANNIVERSARY THE COALITION FOR HEMOPHILIA B

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MEDEXUS PHARMA

NEW FACEBOOK GROUP!

Join Our NEW 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.

We are so grateful for our community! Your sense of giving is awe-inspiring and your impact is significant. From the Holiday Fund, which was able to support 82 families with food baskets, winter coats, boots, and holiday gifts to Giving Tuesday, which raised over $4500 for our BCares assistance program helping many throughout the year, our Hemo B family is making a difference. We appreciate your generosity and we hope you have the most joyful and healthy new year. We can't wait to see you all and give double hugs.

Here's to an incredible 2021!

The Coalition for Hemophilia B

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

SCAN ME

VISIT OUR SOCIAL MEDIA SITES:

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

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

Living Life With a Capital "L" Charlie's Story

BY RENAE BAKER

Charlie is a very self-assured and easy-going teen who has put hemophilia squarely in the #2 position of his life. In the #1 position is Life with a capital "L," full of pursuing his passions and having fun."

The list of Charlie's passions is topped with water sports. Charlie is a very active participant in surf lifesaving. "Surf lifesaving," Charlie explains, "is lifeguards competing against each other, swimming, running, and paddling." The California Surf Lifesaving Association states its mission is to promote Beach Safety awareness and Professional Lifeguard standards through public education, training programs, exchange programs, junior lifeguard programs, competition, and other means. The ultimate goal is to prevent and reduce aquatic injuries, accidents, and death at open-water beaches in the United States and throughout the world.

If you're like me, you probably never heard of this before. "Not a lot of people know about it. At the competitions, barely anyone is there," Charlie says. At 15, Charlie is too young to actually be a lifeguard, but his 16th birthday is coming up soon. Until then, he can compete and continue learning lifeguard skills. He began his training when he was 10 years old. When asked if he feels ready to be hired as a professional lifeguard, he gives a nonchalant, "Yeah," with a smile that is at once confident and sheepish.

Charlie was diagnosed with severe hemophilia B at 10-months old. I asked him how remote high schooling has been through the pandemic "We've had online school since last March. I just can't wait to go back." He misses seeing his friends. He finds interacting with the teachers a lot more difficult online than in-person. He sympathizes that they are not "all the best at this online stuff." Asked what he does like about remote learning, he says, "I have more time to be out and do stuff; hence my breaking my little finger."

Last summer, Charlie took his bike out and was doing some jumps. One didn't land right, and he injured the little finger on his right hand. He infused but quickly realized it was more than a bleed. After months in a brace for the fracture, he realized it wasn't healing when he had another bleed, so he had to have surgery to fix the injury and prevent more bleeds.

The surgery, which was a week before our interview, went well, and he was looking forward to the dressing being removed just a couple of weeks later. "I can't wait to get

this off," he says, holding up his arm and the bulky bandages which span from his fingers to near his elbow, "It's annoying." Charlie feels he manages his hemophilia well. "Every time I have any injury, I get it under control pretty quick. By day two, it's usually back to normal again." Asked for his recipe for success, he credits infusing immediately, when he has an injury, and then checking back the next day to see if another infusion seems like a good idea.

When asked about his life with a bleeding disorder, Charlie says, "To be honest with you, every day, when I'm doing something, I'm not thinking about having hemophilia. I'll be out doing stuff, and hemophilia is the last thing on my mind. The only time I think about it is when I get injured and when I have to do my infusions," he laughs, with a glimmer of understanding that his carefree attitude is likely not the most prudent. "In my normal life, it doesn't affect me much, and I like that."

Charlie has made a conscious decision to not let his hemophilia limit him. "If you let it limit you, that's not a good mindset to be in." Charlie embraces the control he is able to exercise over his bleeds. He is disciplined about his weekly prophy. "I don't want to have any long-term issues, so in my daily life, I'm not doing anything crazy, like jumping off of cliffs and stuff. But I don't let hemophilia dictate my decisions about whether I go on a bike ride or go swimming with my friends either."

Charlie and his family have attended The Coalition for Hemophilia B events and he especially likes hearing the speakers. In addition to school and his passion for surf lifesaving, Charlie is an accomplished water polo player who plays on the high school club and national water polo teams. He is serious enough about water polo to know that he wants to play on a college team. For Charlie, that means looking at California colleges, as there aren't many other states that have water polo teams.

Charlie's life is not all water sports all the time. "I'm in the Entrepreneurship Academy at my school." Charlie is energized about this program. "It's a different kind of class. It's more interactive than your usual math and science classes. I can see how I can apply this to real-world stuff."

Charlie gets a lot out of the guest speakers the class presents, and he enjoys learning about marketing and dreaming up what his own company might look like one day." His guesses it will have something to do with water sports, but Charlie is inspired by someone who speaks to his love of water polo, entrepreneurship, and his hemophilia. Miles Cole is a person with hemophilia, who plays Division I water polo for Princeton University and has just been awarded the 2020 Tiger Entrepreneur Award for starting a tech company, winning the funds, and developing a prototype to automate the IV injection process. Miles Cole sounds like a great role model!

Highly motivated to win a camp award, Charlie has been self-infusing from the age of 10. He did win the award and still treasures the trophy. Charlie encourages teens who aren't yet self-infusing to start that process. "It's worth taking lessons and learning how to do it because you feel




more freedom in what you can do. Infusing can be done on your own schedule. You won't have to wait for someone to do it for you. Especially if you've got events on your schedule, it's a lot easier to just do it for yourself." Charlie admits that it took him a while to get used to, and it hurt, "but then," he says, "I was just like, 'It's fine. I have to do it,' and after a couple of months I got used to it and thought, 'Oh, it's not that bad!'"

The best advice Charlie ever received was, "Don't let what other people think of you affect you. Do your own thing." The advice he'd like to give other teens with hemophilia: "Don't let hemophilia limit what you're doing."

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!

Harnessing the Power of Words: A Sibling's View

BY RENAE BAKER

Born in Lahore, Pakistan, Muhammad's family moved to Illinois when he was one. When his youngest brother was born with hemophilia B, his mother, Aamina, went back to school, received her master's degree and has been working in the healthcare field ever since. Muhammad, has two younger brothers, Ishmael and Ahmad. When Muhammad's youngest brother, tested positive for mild hemophilia B, Muhammad and Ishmael were also tested, but there has been no indication that they have the bleeding disorder.

Muhammad remembers his baby brother wearing a helmet, and having to be extra careful around him. It pulled on his big brother heartstrings when Muhammad and Ishmael couldn't include Ahmad in their wrestling and soccer play. They proved to be good big brothers though, as Ahmed has had very few accidents. Muhammad remembers when he was little, Ahmad tripped and scraped his hand on brick wall and had to be rushed to the hospital.

Muhammad reports that, although Ahmad realizes that there are some things his hemophilia prevents him from doing, he is also enjoying some special friends that it brings to him, especially through The Coalition for Hemophilia B. At the Symposium, Ahmad got to meet a great role model, Chris Bombardier, and now frequently says "CB is my friend!" Ahmad is thrilled that "CB" calls him his "Blood Brother."

Muhammad is happy that the whole family has The Coalition for Hemophilia B in their lives. "At Symposium," he says, "there were a lot of people who had (hemophilia B.) There were breakout sections with other kids. At first," he confesses, "I felt shy and confused, but then we did the ice breakers and I had fun!" Muhammad feels connected through the Coalition and is happy that he and his brothers have friends through the Coalition. "These friends are relatable, because they understand, and they're nicer."

When asked how he's dealing with the COVID-19 restrictions, Muhammad says, "Because of COVID, I have lots of free time I normally wouldn't, so I'm reading a lot of books. I just finished The Hunger Games." He has also been doing a lot of writing.

As Aamina tells it, Muhammad didn't really like writing until a dramatic event took place when he was in the third grade. His teacher, whom Muhammad calls "the best teacher," Miss LeRoy, was Jewish. There was an attack on the local synagogue. It touched him, and so he wrote a note of support to her. He was shy, so it took him five days to give it to her. The moment he gave it to her, she cried and hugged him and then took the letter to the synagogue and they invited Muhammad and his family to the synagogue's Hanukkah celebration. That letter is now framed in Ms. LeRoy's home. He had a similar experience when he wrote a letter to veteran. Through these experiences, Muhammad learned the power of words.

In the fifth grade, he found himself wanting to make a good impression, so he applied himself, and learned that he

had talent. “Then I liked writing!” he admits, smiling. Muhammad explains his writing style like this: “I try to do things from different perspectives and points of view every time. I do online research. For the story I wrote (that takes place in Venice), the hero’s dad likes plants and biology, so I researched Italian plants.”

Muhammad’s writing talent was brought to the attention of the Coalition when he won the recent Symposium talent competition by reading an original story he’d written. Muhammad has not only felt encouraged by The Coalition for Hemophilia B by this recognition, but they “magically” helped facilitate an exciting project to which he is currently devoting much time and focus. When COVID restrictions first took place, last spring, the Coalition hosted Friday night Kahoots game nights. Muhammad threw himself into them, winning an Amazon gift card, which he spent entirely on audio technical equipment, including a microphone and a subscription to a podcast-editing program, which he is now using to host his own podcast, “Politics Schmolitics.”

“I always liked politics and talking to my teachers about it, so this is a podcast about politics for kids. At the time of our interview, Muhammad was working on his fifth episode. Recent topics covered are his thoughts on the last election. “Politics have been more entertaining than usual,” he says. “It took so long to count ballots. The waiting was stressful.” Muhammad gets his news from many sources and tries not to be biased. “I listen to a lot of radio. My favorites are NPR, All Things Considered, different local music stations, and Spotify, depending on my mood.” Other news sources are, The New York Times, CNN, Politico, CBSN, and FOX. Muhammad has received positive feedback from his first four installments. An added perk of creating and hosting this podcast is that he finds himself becoming less shy and more “open” when he talks.

“The best part is that he learned the editing and everything by himself,” Aamina chimes in proudly.

When asked about the best advice he ever received, Muhammad quotes Jay Sheddy, the former monk-turned-motivational-speaker: “Don’t be afraid of new beginnings, new people, new energy, new surroundings, or new challenges. Embrace new chances at happiness.”

His advice to other kids: “Always try to find out one special thing inside you and don’t lose it, because that is what will differentiate you from others throughout your life.” Muhammad is glad to be a part of The Coalition for Hemophilia B family as a bleeding disorder affects the whole family and he said “It’s nice that siblings and my entire family get to be a part of it!”

To check out Muhammad’s podcast, go to: https://politicsschmolitics.buzzsprout.com/

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Winter 2020

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