THE COALITION FOR HEMOPHILIA B

FACTOR NINE NEWS

FALL 2020

WOMEN BLEEDERS- NEW!

B INSPIRED- TEENS

2020 SYMPOSIUM

CHECK OUT PHOTOS FROM THE SYMPOSIUM AT HEMOB.ORG/PHOTO-MOSAIC

LATEST UPDATES ON TREATMENTS & THE INDUSTRY

GEN IX

30 YEAR ANNIVERSARY
THE COALITION FOR HEMOPHILIA B

FACTOR NINE NEWS

CONTENTS

- Virtual Symposium: A Great Success
- Learning to Advocate for Ourselves and Our Community: The Generation IX Project 2020 Virtual Advocacy Program
- The Weakest Position of Power You Can Have
- Treatment News
- Special Newlyweds!
- Hemophilia Update
- New Facebook Group!
- Save the Date!

WOMEN'S SECTION

- Women with Hemophilia
- Making Self-Advocacy Your Fight Song
- No Longer Alone

TEEN SECTION

- It's More Fun for Teens Who Make Treating Routine
- Reaching Maximum Potential Through Dedication and Discipline
- Gen IX Teen Mentorship: From Scratch

Mission

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

30 YEAR ANNIVERSARY
THE COALITION FOR HEMOPHILIA B

See your photo taken at our photo booth during 2020 Symposium turned into a beautiful B mosaic.

Go to hemob.org, click on mosaic and look for your photos!

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 Hemo 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.

EMPOWERED TOGETHER AMAZING

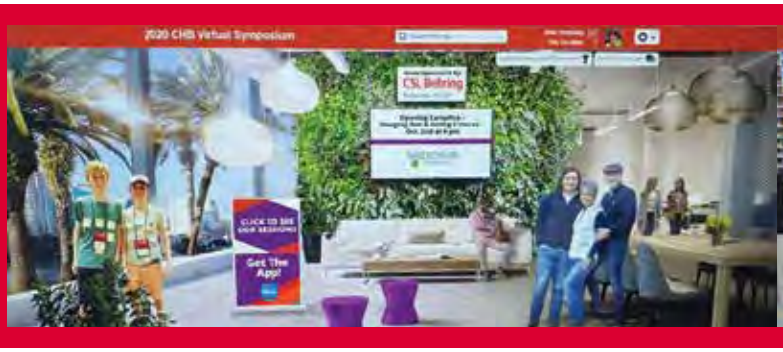
VIRTUAL SYMPOSIUM

A GREAT SUCCESS!

BY GLENN MONES

On the weekends of October 2-4 and 9-11, nearly 1,000 community members logged on with computers, tablets, and smartphones from living rooms and kitchen tables across the country (even some from ER and hospitals!) for the first-ever Coalition for Hemophilia B Virtual Symposium.

The event was created when our leadership determined the in-person Symposium originally planned for Orlando could not be held safely during the height of the COVID-19 pandemic. We understood we needed to put the health and well-being of the community first. However, we also recognized that there was a deep need for our members to be able to connect, learn, share strengths, experiences, and become empowered. We realized we not only had to offer a virtual experience but also that we needed to do everything within our power to replicate and even improve upon almost every aspect of our in-person events.
Our core team went “back to school“ learning methods to conduct an online

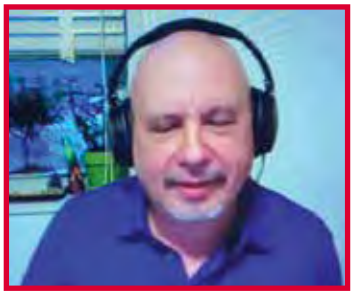


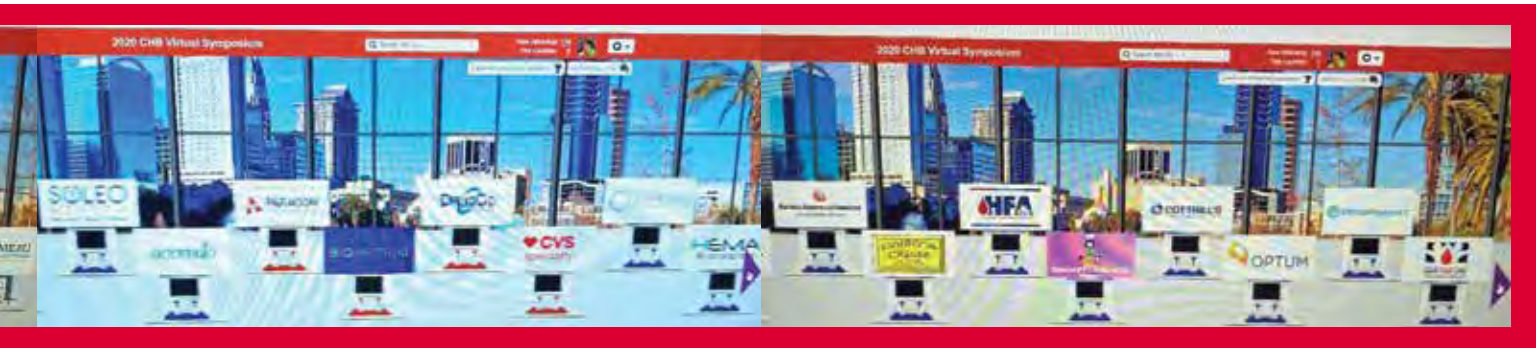
We received enough packages over the last few weeks from the pharma companies to fill 7 totally filled pallets. We would wrap them for storage and then put them on a rack to store until it was time to take out to our other warehouse where we would be packing them. Below shows the 50' line set up to pack boxes.

symposium with a wealth of best practices, the latest technology, and some of the finest service providers in the world. Everyone worked tirelessly for months to create a truly wonderful experience for everyone involved. One of our highest priorities was ensuring that participants would have plenty of opportunities to interact in a variety of group settings. That meant having sessions where people were not forced to rely only on chat windows to interact but could also see, hear, and talk to each other. Not the easiest challenge – but we made it work! Kim Phelan commented, "When I saw the community members seeing each other at the meet-and-greet and witnessing how overjoyed they were, I just started crying joyful tears."

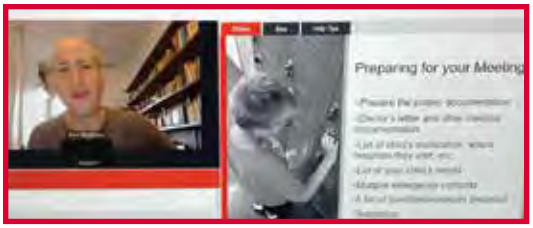
The program included a wide variety of educational sessions featuring presenters from around the country and topics that were requested by all of you, our community members. One of the most popular and engaging sessions was on *Emerging Therapies*. Our exclusive panel included medical and scientific experts from four companies with products in the pipeline. The session was skillfully hosted by our Coalition Board Chair, Dr. David Clark. Dr. Clark also gave his always popular *Hemophilia B Overview*, providing us all with a thorough overview of recent treatment advances and a preview of "what's coming next." Dr. Chris Walsh of the Mount Sinai Hemophilia Treatment Center in New York City also gave an excellent keynote presentation on the current and future state of treatment therapy.

A popular talk on the therapeutic use of cannabis to treat pain was offered by Detroit-based social worker Ellen Kachalsky, LMSW, ACSW. Her presentation explored the ways cannabis-based products have been used over time

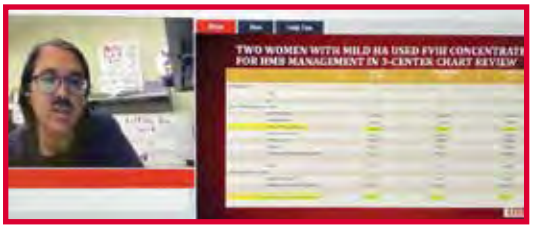





FDA APPROVED MEDICATIONS

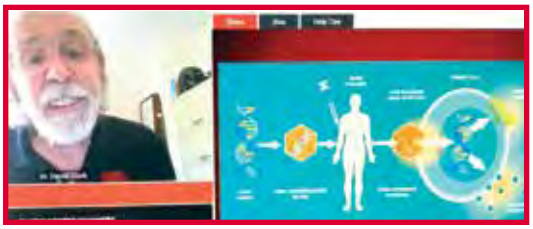

Preparing for your Meeting


SIG-E-CAPS


TWO WOMEN WITH MILD HA USED FVIII CONCENTRATE
FOR HMB MANAGEMENT IN 3-CENTER CHART REVIEW

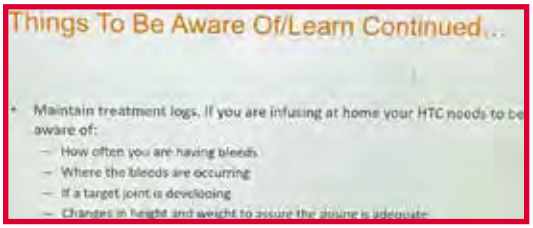

OCT 3
It's Your Story: Pen It! Prep It! Perform It!


Possible Accommodati




OCT 3
Emerging Therapies for Hemophilia B

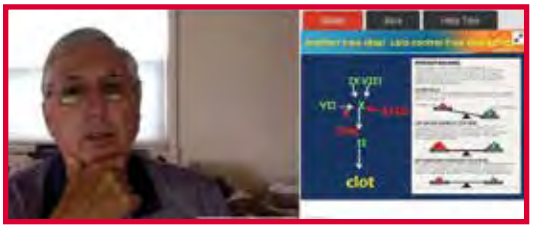

2020: A YEAR LIKE NO OTHER


Things To Be Aware Of/Learn Continued...
Maintain treatment logs. If you are infusing at home your HTC needs to be aware of:
How often you are having bleeds
Where the bleeds are occurring
If a target joint is developing

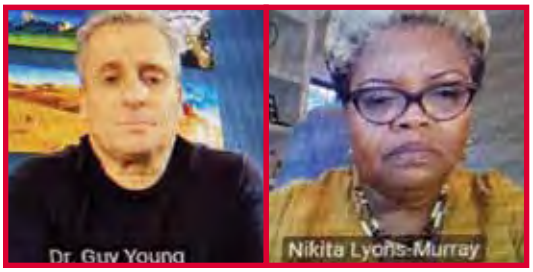

GENE THERAPY QUESTIONS


SUPPLEMENTAL SECURITY INCOME
SSI
SSI is a "means tested program" for persons with limited income which makes payments to disabled persons (including children) who have limited income and resources
MEDICAID IS THE SSI INSURANCE PROGRAM


5
By voting
Volunteer to help an election
Volunteer to help as a poll wo


clot

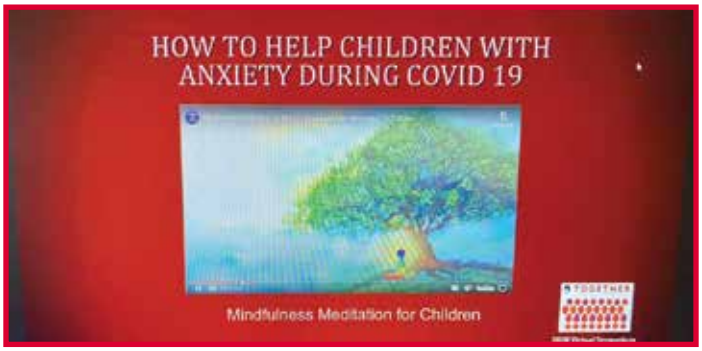

IS IT DEPRESSION OR THE BLUES: UNDERSTANDING THE DIFFERENCE

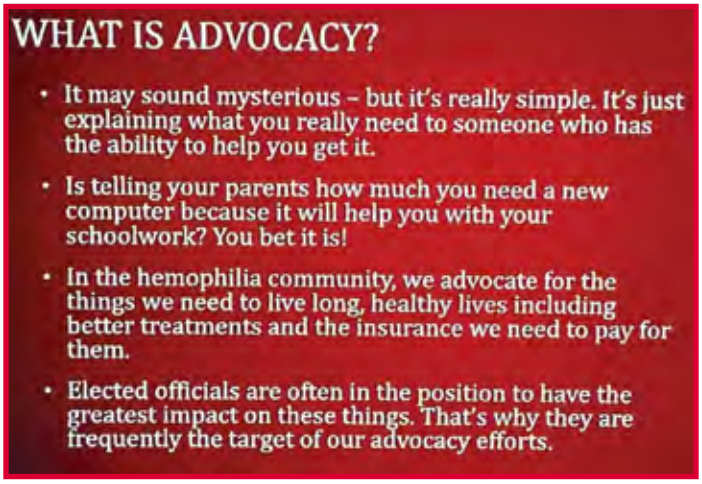

Dr. Guy Young
Nikita Lyons-Murray

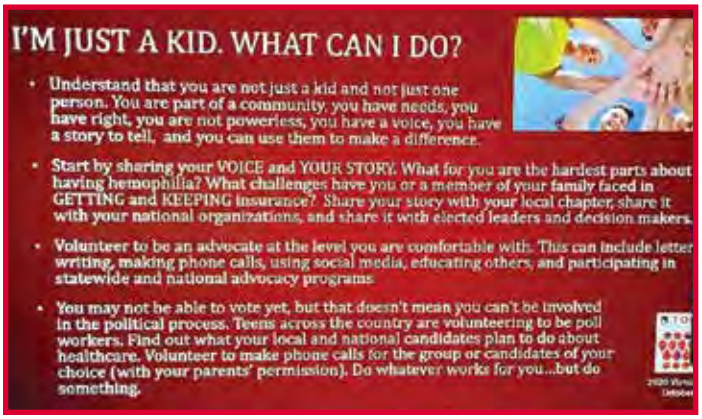
and its current legal status in different parts of the country.

Several industry-sponsored sessions also covered important medical topics. These included a talk on bleed prevention by Tammuella Chrisentery-Singleton, MD of Tulane University, sponsored by CSL Behring. In another session, sponsored by Sanofi Genzyme, Dr. Guy Young, Director of the Hemostasis and Thrombosis Program at Children's Hospital Los Angeles, explained the importance of "looking beyond trough levels" when establishing a treatment regimen for hemophilia b.

The Symposium also featured sessions designed to empower community members with tools and skills to help them advocate for themselves and their families. Topics included using humor to navigate life's difficulties, dealing with depression and other mental health challenges, advocating for the community with our elected representatives, accessing insurance and government assistance programs, and many more. One session featured the film *Let's Talk*, an "immersive journey through the lives of five members of the U.S. bleeding disorders community, as they open their hearts and lives to show how we can gain strength through struggle, and that perhaps we aren't so different after all." The film was produced by Believe Limited, in partnership with Mental Health Matters Too.







Since our community does not live by education alone, there were also plenty of opportunities to just laugh and have some fun – connect, build birdhouses, children's storytelling, magic shows, etc. which we all needed this year!

Our *Final Night* was amazing ! We watched the *Bleeders Band* perform, played games, enjoyed a talent show and entertainment by comedian John Pizzi from "America's Got Talent."

The feedback we have received from many participants was overwhelmingly positive, enthusiastic, and gratifying. "I'm so grateful I could attend this event," said community


Rock
Patrick Torrey
Chris Villarreal


EMPOWERED TOGETHER AMAZING


THE COALITION FOR HEMOPHILIA


THE COALITION FOR HEMOPHILIA

member Lori. “I am loving every minute of it!” she added. Briana, another attendee, said, “This is the BEST, most real symposium yet. NAILED IT !” Matt a recent addition to our hemophilia B family said, “I’m brand new to the Coalition and I keep wondering how I’ve never heard of this before.” Matt added that he is “genuinely impressed by the leadership, the activities, the conferences, and how much good is being liberally offered.”

The Coalition for Hemophilia B is grateful to our corporate sponsors for having made this very special event possible. These include CSL Behring, Medexus, and Pfizer (Platinum Level), Novo Nordisk and Sanofi Genzyme (Gold Level), and Takeda, Tremeau, and UniQure (Silver Level). We also want to thank our many exhibitors, speakers, facilitators, and of course, all the families and individuals who participated!

In 2021, we plan to hold the Symposium in person while keeping some virtual components for those who can not physically attend. In the meantime, we have a range of other virtual programs planned including our very popular “Family Meetings on the Road.” Please check our website (www.hemob.org) and social media for the latest news and updates. We look forward to seeing you again soon!

The Coalition for Hemophilia B expresses deep gratitude to our sponsors for their funding and participation in our 2020 Virtual Symposium. This very special event would not have been possible without your generous support.

PLATINUM LEVEL

CSL Behring · MEDEXUS PHARMA · Pfizer

GOLD

novo nordisk® · SANOFI

SILVER

Takeda · TREMEAU PHARMACEUTICALS · uniQure

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®

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

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

rebinyn®
Coagulation Factor IX (Recombinant), GlycoPEGylated

rebinyn®

Coagulation Factor IX (Recombinant), GlycoPEGylated

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

Rx Only

This information is not comprehensive.

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

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

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

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

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

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

What is REBINYN®?

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

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

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

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

Who should not use REBINYN®?

You should not use REBINYN® if you

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

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

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

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

You should tell your healthcare provider if you

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

How should I use REBINYN®?

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

REBINYN® is given as an infusion into the vein.

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

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

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

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

Use in children

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

If you forget to use REBINYN®

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

If you stop using REBINYN®

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

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

What if I take too much REBINYN®?

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

What are the possible side effects of REBINYN®?

Common Side Effects Include:

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

Other Possible Side Effects:

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

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

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

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

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

More detailed information is available upon request.

Available by prescription only.

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

Revised: 11/2017

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

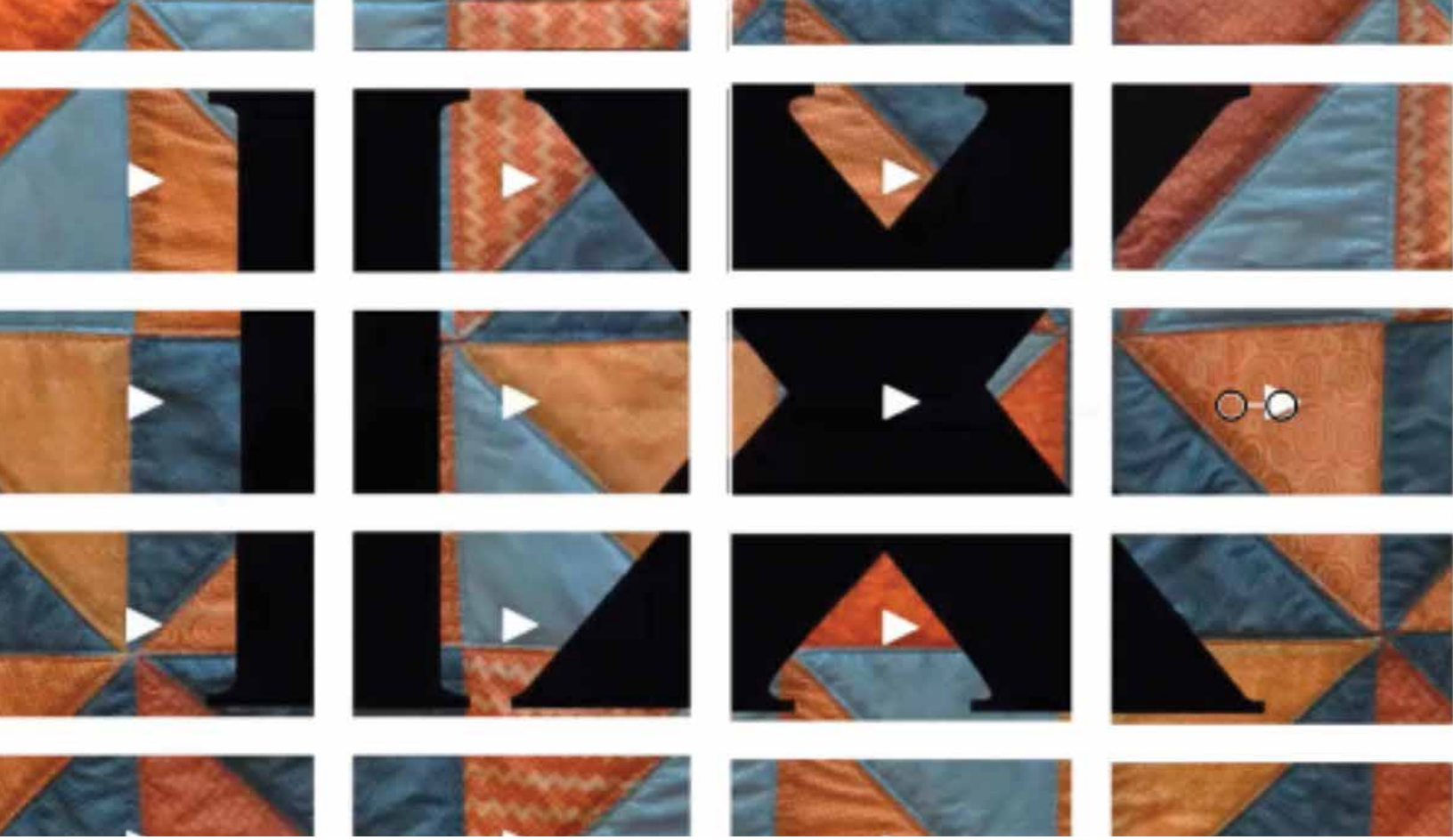
For Patent Information, refer to: http://novonordisk-us.com/patients/products/product-patents.html

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

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

USA17BIO03951 12/2017

novo nordisk®



Over three successive weeks in September, adult community members from across the country came together online each Tuesday and Thursday for the first-ever Generation IX Project Virtual Advocacy Program. Over the six evenings, participants learned and practiced skills and techniques through a variety of interactive experiences to become better advocates for their own needs and those of our broader community.

LEARNING TO ADVOCATE FOR OURSELVES AND OUR COMMUNITY:

THE GENERATION IX PROJECT 2020 VIRTUAL ADVOCACY PROGRAM

BY GLENN MONES

The event was presented in cooperation with GutMonkey, a company we have worked with for many years that specializes in experiential learning. The program was made possible through the generous support of Medexus Pharma. In previous years, this has been held as an in-person program in a variety of settings. This year, the program was transformed into an all-virtual program because of the limitations imposed on traveling to in-person events due to the COVID-19 pandemic. Re-creating the intimacy of previous programs presented significant challenges. However, through highly creative interactive sessions and the strategic use of smaller break-out groups, participants enjoyed a truly positive and unique experience.

Throughout the program, participants were given a variety of successively more complex tasks to complete as individuals and in groups designed to foster creativity, problem-solving, cooperation, and group cohesion. For example, during the first session, attendees broke up into teams with the surprising goal of making popcorn! Although the "finished product" provided a tasty treat enjoyed by all, the main idea was to figure out how to work together to produce the best outcome. During subsequent sessions, participants created individual graphic logos to express a personal advocacy goal or experience. These logos were then transformed into beautiful cross-stitch designs.

Finally, each attendee created a mini-film that explained the logo and the meaning behind it. In this way, everyone learned how to use multiple techniques and forms of media to express ideas and communicate them to others. These same tools can be applied to a wide variety of real situations, whether one is advocating with a school, an insurance company, or even a member of Congress.

During another session, everyone participated in a live "treasure hunt" using maps and clues. Members of the GutMonkey team served as "stand-ins" who received directions from the attendees viewing their progress in real-time.

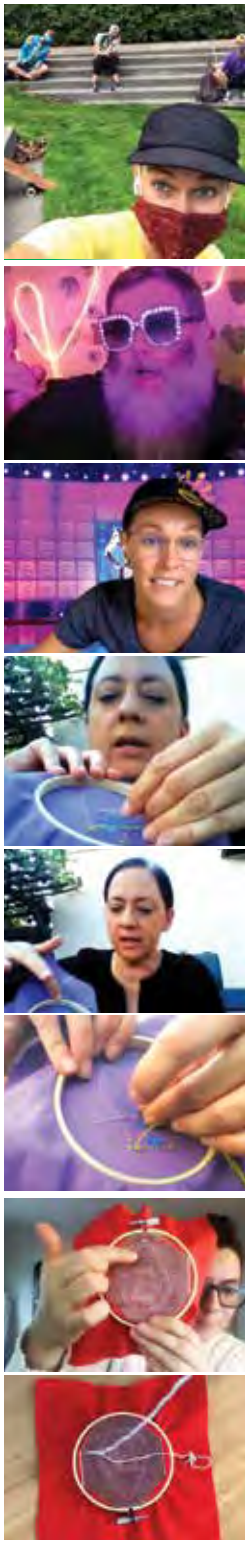
The reactions of participants have been very enthusiastic. One attendee Lori said "I absolutely loved the program. Even though we were virtual, I found it engaging. I loved the activities! Mostly, I loved spending time with my blood brothers and sisters, talking shop. It helped me think of new and creative ways to focus my advocacy efforts in our new normal." Jeron said, "I really like the way we all made new friends and memories that will stay with us forever. We came together as a family." Still another participant said "I have attended many different virtual meetings, trainings, sessions and this was a very engaging and exciting one. I appreciate all the thought, energy, care, and love that was put into organizing such an amazing virtual session. Though I am sure we all wish we were in person, this was a very welcomed break to the current COVID way of life."

The Coalition's Generation IX Project is the only national program for people with hemophilia B. In addition to Advocacy, the Project also features programs focusing on Leadership and Mentorship that take place throughout the year. We are grateful to GutMonkey, our program partner, and to Medexus Pharma, the sole sponsor of the Generation IX Project. Medexus' support allows us to offer these important programs at no cost to the participants. Spaces are limited so everyone interested in attending a program must apply in advance.

If you would like to learn more about Generation IX programs, please visit www.hemob.org/programs-1/generation-ix-project on our website. We would love to see you at a future event.

Thank you to our generous supporter of this invaluable program!

MEDEXUS PHARMA



THE WEAKEST POSITION OF POWER YOU CAN HAVE

AN INTERVIEW WITH TARA AND ADAM SMITH
BY RENAE BAKER

If you've participated in the Coalition's new BEATS music program, you have seen Adam Smith sharing his home recording studio in Nashville, TN. At the inaugural program in July of 2019, I met Adam, his wife Tara, and their two small children, Nova and Arlo. Arlo is now four years old and has severe hemophilia B.

I sat down with Adam and Tara over Zoom to learn more about their journey so far. Tara begins their story three or four months after Arlo's birth.

TARA We started noticing tiny, little bruises on Arlo in the places where we would pick him up.

There was no family history, so bleeding disorders were not on their radar. Around 5-months old, Arlo began having really big bruises that seemed inexplicable.

TARA Our pediatrician was dismissive of the situation. She even did labs and told us that Arlo didn't have hemophilia. So, we kept an eye on it for a while. When we went back for his 6-month appointment, he had even bigger bruises, and I told her this just didn't seem right to me, so she sent us to Vanderbilt for testing.

February 22, 2017 is ever-present on Tara's and Adam's minds. That's the date they received Arlo's diagnosis – severe hemophilia B. The diagnosis stunned them. They felt completely uninformed and frightened.

ADAM The doctor called us and said, 'This is what he has. See you in two weeks! It was completely unacceptable that we had to wait 2 weeks after Arlo's diagnosis.

TARA This was awful! They should've called us in the very next day!

Tara and Adam had to figure out how to tell their families. After spreading the joyous news about the birth of their big, healthy baby boy, they were hesitant to bring them down with this heavy news. During those two weeks of limbo with no doctor's advice to help them, Tara posted about Arlo's diagnosis in a Facebook mom group from the birth center where she'd delivered Arlo.

TARA It felt like a safe place to talk about it. I said that we had just found this out and that we didn't know much about it yet.'

ADAM Yeah, we were deep into googling everything. We really didn't have any idea. Is he going be able to walk? Is there anybody out there on the Internet who has this right now who can give us some perspective?

The barrage of information they haphazardly found, ranging from the history of the disorder, to outdated articles and suggested protective gear like helmets and knee pads, made for a harrowing two weeks. Happily, they got a bite on the mom's Facebook group by the end of the first week. A fellow member knew someone with an eight-year-old son with hemophilia. She put them in touch, and they had a long conversation during which this new friend explained all of the "normal activities" her son is able to do in spite of his bleeding disorder. This did a lot to quell Tara's and Adam's fears.

TARA It was totally everything to be able to talk to someone early on who can tell you it'll be okay!

This new friend turned out to be very connected to the bleeding disorder community in Tennessee and was instrumental in helping Tara, Adam and their children become so as well.

TARA We have been fortunate to get really involved in the community only three months into learning about hemophilia in our family.

Tara highly recommends that people proactively get involved. "Before you know about The Coalition for Hemophilia B, it can be hard." Tara found The Coalition For Hemophilia B on Facebook, made her way to the website, and signed up to get more information.

TARA They're really good! They're the best organization when it comes to staying in touch with people and supplying information.

Some of this information pointed Tara and Adam to the Coalition's Annual Symposium, which they feel grateful they were able to attend during the first year of Arlo's diagnosis.

TARA We've made some really good friends at these events!

ADAM You'll be at one of these events and all of a sudden, you have a conversation where you're like, 'Oh, you get it! You're the only person in the past year I've talked to who understands this one tiny piece of what I've been going through!' Don't underestimate those tiny moments of connection!

TARA Yes! Instant chemistry! There are people you meet where it's like 'Aah! I don't have to start from the beginning and explain what it is!' I mean, we had so many conversations with people the first few months after the diagnosis that went exactly the same way every time. Other people were always trying to grasp at straws to find the good thing about it. We'd explain it and they'd ask, 'So, there are different severity levels?' and you could really see that they were hoping...

ADAM …that we'd appease them.

TARA …and say, 'Oh, yes. He's not that bad.' But we'd say, 'Yes, he's the most severe –' and they'd ask if he would grow out of it one day. 'No, it's forever.' It's so exhausting. We felt like we processed it pretty quickly and just dove in and embraced it, but it was harder to get everyone else around us to be comfortable, and we felt responsibility for them to be okay too.

ADAM They feel bad for us. They want to figure out how to help us.

Tara and Adam seem in lock step with each other as they not only navigate their way through being a family identifying and thriving with a rare bleeding disorder, but

also with their processing the reactions of others outside the community with grace and understanding.

ADAM We live in a fishbowl. People watch you. They are watching to see how you handle it. Without knowing it, you could be inspiring people, and they take your lead. The weakest position of power you could have is vulnerability.

It seems downright poetic, but Adam and Tara are learning that there is power in their vulnerability - power to help others.

ADAM It's funny - we get to help people process it. We can actually help them along a lot. We'll see it click in their minds and people will be like, 'Hey! These crazy people can really be okay with these intense things!

Tara and Adam both muse over the vulnerability of other people who feel helpless in the face of their family's circumstances.

TARA We have to deal with it every day so we stay cheerful and say okay! What do we need to do today?

ADAM It's funny to see other people in such a vulnerable place see how doable it is.

We talked about how this applies to Covid-19 and the novel coronavirus.

ADAM For people in the bleeding community, it's just another thing. This whole community has always dealt with something over which we have no say.

TARA We've learned the hard way.

Adam and Tara are discovering that having a new challenge, like coronavirus, triggers old emotions. They thought they had mastered their emotions over this diagnosis, but they are still lying just under the surface.

ADAM We've lost faith in the safety of probability. There's no comfort there. I'll hear someone talk flippantly about health insurance and say, 'I never use it.' That triggers me. One day you don't need it and the next, your son's life depends on it. We used to have pride in that attitude, but it all changed in an instant.

TARA We've gained perspective. We're thankful for that. We're honored to be able to experience that and have the kind of community that understands.

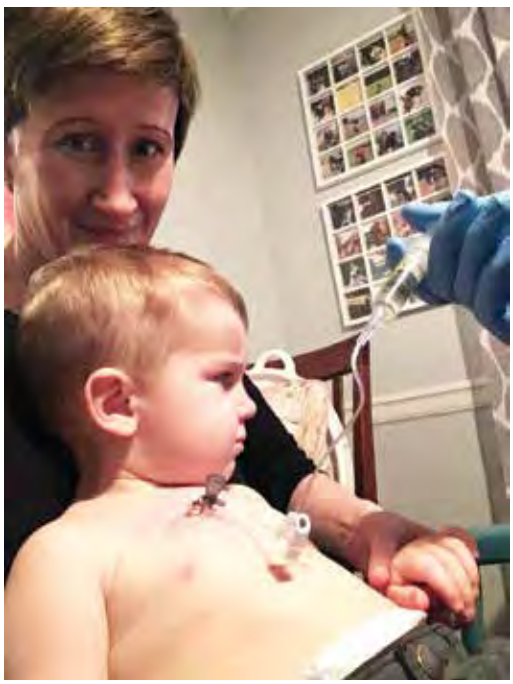
ADAM We have to remind ourselves that not everybody does, so we have to have the grace to not expect the response we wish they would have. We can't fault people with not knowing.

TARA It gives us opportunities to help people understand and be a good example. COVID has become an over-arching thing in people's lives. We understand this because of hemophilia B, so it's like a silver lining to be this help for others. I am touched and inspired by their attitude. I see them as ambassadors or angels, but they are very humble about the role they play in helping others navigate extreme circumstances. We don't want it to come across as know-it-alls or like it's our mission. It's more like a subtle infusion of newfound wisdom.

ADAM Just, we're here!

They sure are here! After they'd attended their first symposium, Tara saw that the Coalition was going to do the Beats music program. She reached out because they live in Nashville, aka Music City, USA, where the program was being held. Adam, it turns out, works in the music industry. He is a producer, songwriter, and multi-instrumentalist working out of his home studio, which is called The Robot Factory.

ADAM People come to me with ready-made songs or maybe they'll need help finishing their songs with recording, producing, and mixing. I get to do what I love!



For the first Beats program in 2019, Adam hosted Coalition members on a tour of his studio, which proved to be a very popular outing. In October of 2020, The Beats program was held virtually via Zoom due to the coronavirus. It was called The Mini Beats. Adam gave a talk about his recording studio and work. He participated in a panel discussion about the healing power of music and created music tracks for the vocalists and others for a final taping of the performances to be presented at a later date at the time of this writing. Adam feels that The Beats is a great way to interface. He believes one of the biggest needs in the community is to get together and lean on each other.

ADAM That's the real stuff! Arlo doesn't have an inhibitor, but I love to go to the inhibitor family camps and be a music guy.

Tara and Adam hold The Coalition For Hemophilia B in the highest regard and they are happy to contribute.

TARA The Coalition has been most supportive of all the organizations. They reach out if they know of a need even if we haven't reached out for help.

ADAM Gratitude doesn't even begin to cover it knowing people like that are even out there.

TARA Kim (Phelan) has such great compassion. It's like a family.

ADAM People who don't want anything back but for you to be happy. So much else in life is like a transaction. Not this.

TARA Maybe it's because it's so specific to hemophilia B, but the people involved are very special.

ADAM In many ways, we're in a vulnerable position, but with the Coalition, we don't feel like victims.

On the one-year anniversary of Arlo's diagnosis, Tara started an Etsy shop called Arlo and Marie Designs selling T-shirts, accessories and jewelry.

TARA I had been searching the internet for advocacy-related things that would raise awareness.

She did find some, but the messages seemed too "loud" for her style, so she created a product line with subtle-yet-supportive imagery, like a bar necklace with the roman numeral IX and a heart on it, and modern "BRAVE" gear, including a BRAVE teddy bear that Arlo always has next to him during infusions. To check out her site, go to www.etsy.com/shop/arloandmariedesigns or google "hemophilia BRAVE."

TARA It was mainly for my family, but then I started getting orders from others. I'd ask them how they found my shop and it became another avenue of outreach in the community!

Because people have found her shop and see the support and encouragement her products offer the bleeder community, Tara and Adam are now being asked to speak at conferences and tell their story.

ADAM It was like, 'Whoa! We just changed from being in the seats to presenting!

The best advice they ever received?

ADAM Sometimes people are going to offer you help. Just take the help.

TARA It's there for you. Over the lifetime of having a child with something, you earn those things just by existing.

Arlo is doing great, his parents report. "He's learning little things he can do to get through tough moments. He just learned to whistle, so he does that before we poke his port. Nova is a great cheerleader for her little brother, sitting next to him when he gets his factor.

TARA The other day, Arlo had a bad pain in his knee and Nova said, 'I wish I had hemophilia, so I could be the one with the pain.'

ADAM Little kids get empathy really young."

SHOOTING FOR STRONG ON-DEMAND BLEED RESOLUTION, THIS NHL HEAD COACH CHOSE IDELVION

David Quinn has had an incredible and inspiring journey on his road to becoming head coach of the New York Rangers. David attributes finding his coaching passion to his hemophilia B diagnosis. **IDELVION provides David with strong bleed resolution when treating his hemophilia B on-demand.**

Learn more about effective on-demand bleed resolution at **IDELVION.com**

> ***"I chose IDELVION because I have confidence in the product and I have confidence in CSL Behring"***
>
> *—**David Quinn**, Head Coach of the New York Rangers*

Important Safety Information

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

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

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

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

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

In clinical trials for IDELVION, headache was the only side effect occurring in more than 1% of patients (1.8%), but is not the only side effect possible. Tell your healthcare provider about any side effect that bothers you or does not go away, or if bleeding is not controlled with IDELVION. **Please see brief summary of prescribing information on adjacent page and full prescribing information including patient product information at IDELVION.com.**

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

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

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

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

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.

women bleeders

A NEW FEATURE SECTION

ARTICLES TO SUPPORT WOMEN WITH HEMOPHILIA B

Women with Hemophilia

BY DR. DAVID CLARK

Many physicians would say that this title doesn't make any sense – women don't get hemophilia. Today we know that women can have hemophilia, and in fact, the number of women with hemophilia is significant. Unfortunately, this "news" hasn't reached many physicians, especially those who do not specialize in hemophilia treatment. This is in spite of the fact that the medical literature has contained reports of hemophilia carriers with factor VIII or IX deficiency and bleeding problems since the 1950s. Because the idea that women don't get hemophilia has been so ingrained, early researchers tried to imagine all kinds of other explanations for these reports. The genetics seemed so simple that there had to be an alternate reason. Now we know that the genetics are actually much more complex, and it is obvious that women can and do have hemophilia. The rest of this article will focus on hemophilia B, but the situation is very similar for hemophilia A.

The lack of recognition of an obvious condition can make it difficult for women with hemophilia to be taken seriously about their disease. This is not a small issue. It is estimated that there are up to five times as many carriers as there are men with hemophilia. Another estimate is that at least one-third of carriers have factor levels below the normal range. Although not all of them have bleeding problems, a significant number of them do. Today, more and more Hemophilia Treatment Centers (HTCs) are treating carriers. However, there is still a general lack of information on bleeding tendencies and treatment options for women.

Carriers have a defective factor IX gene on one of their X chromosomes, which they can pass on to their offspring. All of the daughters of a man with hemophilia are carriers, sometimes called obligate carriers. On average, half of the daughters of a female carrier will also be carriers and half of her sons will have hemophilia. The carrier daughters might or might not have hemophilia. It depends on other things, most of which are not yet understood.

Chromosomes are structures in the nucleus of a cell that hold the genes. Except for a few specialized types of cells, every cell in the human body has 46 chromosomes

which together hold a copy of all of the body's genes. This collection of all the body's genes is known as the genome. Two of the chromosomes are the X and Y chromosomes, which determine the sex of a person. The X chromosome is shaped like an X and the Y like a Y. Men have an X and a Y chromosome, and women have two X chromosomes. Men inherit their X chromosome from their mother and their Y chromosome from their father. Women inherit one X from each. The factor IX gene is located on the lower leg of the X that is missing on the Y chromosome. Therefore, men have only one factor IX gene, but women have two, one on each X chromosome.

Because women have two X chromosomes, carriers usually have a second X chromosome that contains a normal factor IX gene. It is only the rare carrier who has defective factor IX genes on both X chromosomes. She would be the child of a carrier mother and a father with hemophilia B; on average, half of their female offspring will have two defective factor IX genes. These women unquestionably have hemophilia, even by old-school standards. (This situation may not actually be as rare as the medical establishment has assumed. They look at the overall population and figure that the chances that a man with hemophilia will meet and marry a carrier is very small. This overlooks the fact that the hemophilia community has bonded together so that many hemophilia families know each other.) In the rest of this article, we'll focus on the more common situation of carriers with one normal and one defective factor IX gene.

The idea that women do not get hemophilia comes from the fact that most carriers still have one normal factor IX gene. Reasoning that males with one normal factor IX gene do not have hemophilia, it was thought that most carriers should also not have hemophilia. However, this reasoning is faulty because it doesn't take into account a phenomenon that has only more recently become appreciated. It turns out that even though every cell in a carrier's body contains a normal factor IX gene, those genes are not all active. Because having two copies of every gene on the X chromosome could cause problems, the body has a mechanism to inactivate one of the two X chromosomes in women. Therefore, a carrier's cells will only contain one active factor IX gene; the other one on the inactivated X chromosome will not produce any factor IX. This process is called "X chromosome inactivation" or "lyonization" after Dr. Mary Lyon, the researcher who discovered it.

Lyonization is normally a random process, so each cell has a 50-50 chance of having the active factor IX gene be the normal one. Therefore, in the liver, where factor IX is made, about half of a carrier's cells will make normal factor IX and half will make defective or no factor IX, depending on the gene mutation. Thus, a carrier will usually have approximately half of the amount of normal factor IX that a non-carrier woman has. Several studies have found that the factor IX level in hemophilia B carriers can range from less than 1% to as high as 150% of normal (<0.01 to 1.50 units/ml of plasma). That encompasses the whole range from severe hemophilia (<1%) through moderate (1 - 5%) and mild hemophilia (5 - 40%) and the complete normal range of 50 - 150%. Thus, just based on factor levels, carriers can have anything from severe hemophilia to no bleeding problems at all.

No one knows why one carrier might have a factor level of 25%, for instance, and another might have a level of 150%. For persons without hemophilia, the actual level appears to depend on parameters such as blood type and body mass index, but those parameters do not seem to affect levels for men or women with hemophilia. One thing that does appear to cause the extremely low factor levels seen in some carriers is a phenomenon called "skewed X chromosome inactivation" (sometimes called extreme lyonization). Again, for reasons that are not well understood, the lyonization process may preferentially inactivate one of the X chromosomes. If the X chromosome that is inactivated in more cells is the one with the normal factor IX gene, the carrier will primarily produce defective or no factor IX. She will thus have a much lower level of normal factor IX in her blood than would be the case if the inactivation were 50-50.

Carriers with low factor IX levels bleed the same way and have the same kinds of problems as males who have hemophilia and similar factor levels. They are susceptible to easy bruising, joint damage and bleeding problems after dental and surgical procedures among other things. They

can also develop inhibitors and target joints. In addition, as women some of them have excessive menstrual bleeding, a tendency toward miscarriages and excessive bleeding after giving birth. These are all issues that can be managed once it is accepted that they have a bleeding problem.

Bleeding in carriers can also be due to other bleeding disorders. Just because a woman is a hemophilia carrier does not keep her from having other bleeding problems. Some carriers, for instance, might have von Willebrand Disease (vWD), which is much more prevalent in the general population than hemophilia. Many of the symptoms are similar between some types of vWD and hemophilia, so proper testing is important to establish the actual cause.

The nomenclature is also an issue. In the past, carriers who exhibit bleeding problems have often been called "symptomatic" carriers, as though they have bleeding symptoms but are somehow different from males with actual hemophilia. It is becoming more obvious that these women are not different. They have hemophilia as shown not only by their bleeding symptoms, but also by their genetics and factor levels.

One recent study has shown that carriers tend to develop reduced range of motion (ROM) in their joints, just like males with hemophilia. This indicates that they have suffered joint damage, presumably from bleeding into the joints. The reduced ROM gets worse with age and in general is worse in proportion to the carrier's factor level - the lower her factor level, the greater the reduction in ROM.

According to the usual criterion, people with factor levels above 40% aren't considered to have hemophilia and don't suffer joint damage. However, a couple of the carrier studies have noted that women with factor levels in the 40 - 60% range sometimes do have bleeding problems and joint damage. The reason for this is also unknown. Note that a similar occurrence can happen in men with hemophilia – the factor IX level does not always predict the severity of bleeding symptoms. For example, some men classified as severe because their factor level is below 1% bleed more like moderates, and some with mild or moderate levels bleed more severely. As above, the reasons are unknown.

With the recognition that carriers can have significant bleeding problems and suffer joint damage, it becomes more important to identify them, assess their bleeding tendency and offer them treatment, if needed. Several studies have shown that women with bleeding problems are usually identified later in life than men. Men with hemophilia are often identified soon after birth, while many women with hemophilia are not identified until their teens or later, depending on the severity of their condition. Also, because of the incorrect perception that their bleeding is not as serious, only 27% of females with severe hemophilia B are on prophylaxis, according to the UDC database.

Many HTCs offer carrier testing, but often not until a woman has reached adolescence. Since it is now known that joint damage can start to develop undetected at an early age, it would be beneficial to identify affected women early enough to help prevent those issues. Other reasons that it is beneficial for carriers to know their status include decisions about participation in sports, taking certain medications like aspirin, getting tattoos or piercings and most of the other things that men with hemophilia need to consider. Iron deficiency anemia also appears to occur more frequently in carriers.

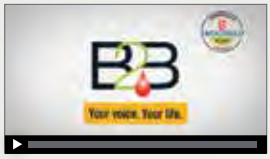
The situation for women with hemophilia is slowly getting better, but there is still a lot of misinformation in the medical community. In addition to the Coalition for Hemophilia B, the Hemophilia Federation of America and the National Hemophilia Foundation, as well as a number of concerned medical professionals, have all recognized that there are significant unmet needs among the women of the hemophilia community. They have also founded the Foundation for Women and Girls with Blood Disorders (FWGBD), which has a lot of useful information on its web site. Hopefully, with their continued advocacy and leadership women will be able to receive the care they need.

B2B: Your voice. Your life.

Interested in stories and personal experiences *from* the hemophilia B community *for* the hemophilia B community? Turn to B2B.

Download or view books
filled with patient stories that can inspire, empower, and educate

Listen to podcasts
that capture the voices of the hemophilia B community



View videos
that feature members of the community sharing their personal experiences

And there's much more, too!

More topics include . . .

- Educating your child's school about hemophilia B
- HEMO 101 for caregivers
- Managing relationships with hemophilia B
- Life for girls and women with hemophilia B

VISIT WWW.HEMOPHILIAVILLAGE.COM

to explore all B2B materials

PP-HEM-USA-1093-03

Printed in USA/September 2018

Making Self-Advocacy Your Fight Song

AN INTERVIEW WITH KIRSTIN DRYE
BY RENAE BAKER

If I could, I would go back in time and tell my younger self to fight to be allowed prophylaxis at a younger age" Kirstin says at the end of our Zoom interview.

Kirstin, who lives in Virginia, was six when her brother, Spencer, was born. "When he was a baby, there were some situations that led to his diagnosis. That's when hemophilia first entered my life. Prior to that, we weren't aware of hemophilia B."

There was no family history. As a young girl, Kirstin was told that this bleeding disorder was "all boy-related." There was a chance she could be a carrier, but at the time, the family was mainly concerned with acclimating her to what hemophilia meant in terms of her little brother.

When Kirstin was ten, there was a study being conducted about children and their family history where it was discovered that Kirstin's mother had a mutated hemophilia B gene. That's where it had originated in their family. Kirstin was tested. It was expected that her test would show that she was a carrier, but they discovered that her levels were low as well. She remembers going back and forth for several blood tests because the doctors just couldn't believe a female could have such low levels.

"That was a difficult thing for me as a ten-year-old because I didn't want to be different and I didn't want something only boys have. This made it hard to navigate my preteen years." Kirstin felt angry. Not only was there a stigma around blood disorders back then, but she was keenly aware of the judgements between children that are common during the preteen years. "I had some friends who strayed away, and friendships shifted because they were scared or didn't want to engage more in it. Whether it stems from the kids or is the influence of their parents, unfortunately, that's something that happens with diagnoses like this. It leaves you feeling very alone."

Kirstin didn't know another female with hemophilia, and back in 1993, there were very few females who had been diagnosed with it. When she was about thirteen, her mother saw an article in a national newsletter about a young woman who had been diagnosed with hemophilia A. She wrote to the editors and asked if they would give

her the contact information of this woman. She thought, "If I can connect my daughter with this woman, it would be a very big deal for her to have another affected female to connect with!" Though Kirstin was ten years younger than her new friend, Danielle, they became pen pals and stayed in touch. Kirstin found great comfort in having a connection with another female bleeder.

As puberty hit, Kirstin had to deal with the challenge particular to female bleeders. Her doctor started her out on a low dose birth control to help control the bleeding, but only doubling the highest daily dose helped, and she continued that regime for 10 years.

During high school, Kirstin loved to swim. She especially liked the butterfly stroke, which in return would cause frequent shoulder bleeds. She remembers her mother was always at the edge of the pool with an ice pack to apply right away. They'd go to the hematologist only to be frustrated time and time again. Although she had been diagnosed at an early age, it was still a challenge to get acceptance of her bleeds from medical providers and to get access to the treatment she needed. "Even within private practices, doctors didn't always agree," Kirstin laments. "One doctor would say, "This is just a sprain." Another would walk in and announce, "This is definitely a bleed and she needs to be treated for it."

Kirstin's family started to find local mentor families. "One of those families was on the board of a national organization

and they told us, “You guys have to come to this meeting! You’re going to love it!” It was an HFA meeting. As Kirstin recalls, “It was great! There weren’t girls who were necessarily diagnosed there, but at least there were girls my age participating in the sessions because they had someone in their family with it, so it gave me others to bond with in the community.”

It would still be many years before Kirstin would witness the significant and increasing camaraderie among female bleeders. Kirstin exclaims. “It’s really cool to see the support between females grow throughout the bleeding community now!” Kirstin heaps praise on The Coalition For Hemophilia B. “I am so very thankful to find the hemophilia B community because that’s what pulled me out of a dark hole at such a delicate age. I jumped right into it, trying to get involved with advocacy.”

Kirstin was finding that learning how to advocate for herself was of paramount importance. She documented everything and pleaded her case to her hematologists to get them to accept that she was having bleeds and needed prophylaxis. She found herself saying to her hematologist, “Hey - I might be mild but I bleed more severely, and I need to be on prophy!” Still, Kirstin wouldn’t be prescribed a preventative course of factor until she was thirty-two years old. She shakes her head, “Now, it’s all about dealing with the damage to my body and joints that occurred during those years without proper treatment.”

As a young adult, Kirstin became deeply involved with bleeding disorder communities. “I was twenty-two years old, trying to figure things out, and I thought, ‘Hey! Let’s get on a board of directors!” she says with a laugh that reveals the work and time that entails. She has served on the board of her local chapter, The Hemophilia Association of the Capitol Area, as Vice President and President.

Kirstin continues, “The Coalition’s support for females is huge! I recall there was some chatter among the other bleeding communities; some tip-toeing around the issue of women bleeders, but it was really The Coalition For Hemophilia B who pulled the trigger to get women with hemophilia B together, and now we see the other organizations also supporting women with bleeding disorders.”

There are a few people to whom Kirstin finds herself constantly grateful. Her mother, who not only advocated for Kirstin when she was child, but who also set a strong example of the kind of leadership that gave Kirstin the wherewithal to step up to leadership positions within the community.

Another is Carl Weixler, a fellow Coalition member who has had a huge impact on her. “He is responsible for



pushing me to infuse myself the first time. I was about 27, we were at a meeting, and he strongly encouraged me to try. She explained, “I have little bitty rolling veins. No one likes my veins. Doesn’t matter what nurse or doctor it is.” Her record was being stuck 14 times before gaining access to a good vein. To make matters worse, she has a fear of needles, and would lose her color and hyperventilate. She warned Carl and did grow pale, but with his reinforcement, she did it!

And then there is Brian. Brian and Kirstin have been best friends since high school. “He’s the best in the whole world!” she gushes. They didn’t date in high school, but she noticed that he cared about her family, whether he was hanging out with her brother, Spencer, to play video games when he had a bleed and had to stay home, or helping move her into her college dorm after her father had a heart attack.

They continue to be best friends, only now they are also married. When Kirstin was first prescribed her regular factor, she still had to deal with her fear of needles. “I told myself, ‘I have to talk myself out of this.’ I had advocated to get the prophy for myself, now I had to get over the needle thing! For the first two years, my husband had to be next to me every time I treated myself. He had to make sure the dogs and our cat were far away. I’d be treating myself and start turning pale, and Brian would cheer me on saying, ‘Kirstin, come on! Come on! Come on! Focus!’”

It took a lot, but Kirstin eventually overcame her fears enough to be able to self-infuse herself even when she was alone. She looks back at the times when she wasn’t able to walk for twenty-four hours before a doctor would finally approve her use of factor. Now she treats every four to five days. “Learning to advocate to get prophylaxis for myself was my biggest challenge but it was absolutely life-changing!”

As grateful as Kirstin is to these supportive individuals, she can’t help but bring the conversation back around to The Coalition for Hemophilia B. “It’s all about the community. The people are accepting and loving, and they understand all the crazy weirdness and the insanity that we deal with. Even when it’s an experience we can’t relate to, we’re all just there for each other. We go through cycles with more difficult health challenges. I went through a period where, mobility-wise, I wasn’t getting around very well and that’s hard when you’re in your early thirties and your friends just don’t understand that you can’t go on a two-hour walk with them. You try to explain it to them, but it’s hard to understand if you don’t live it. I remember being at a Coalition meeting and needing to use a scooter. My community friends could read me. They knew something was wrong, and they went out of their way to check on me and make sure I was okay. There is a special connection with The Coalition for Hemophilia B and community members. It’s strong.

Kirstin considers the growing bonds within the community which stem from helping each other through their very difficult events. “It’s a beautiful thing to see the torch get passed to new factor IX families. That’s what keeps us there. It’s the most important aspect.” Kirstin echoes a sentiment heard within the Coalition over and over, “I wouldn’t change having hemophilia because of the people and what it’s brought me through the years.”

IXINITY®
coagulation factor IX
(recombinant)

Discover more about IXINITY®

Visit IXINITY.com

MEDEXUS PHARMA

sign the 9 is now online!

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

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

Scan me or visit signthe9.com today!

MEDEXUS PHARMA

IXINITY®
coagulation factor IX
(recombinant)

TREATMENT NEWS

BY DR. DAVID CLARK

Aptevo/Medexus Receive Prophylaxis Indication for Ixinity

9/25/20 FDA announced that they have approved a routine prophylaxis indication for Ixinity. Ixinity is produced by Aptevo BioTherapeutics, which was acquired by Medexus Pharmaceuticals in February. Aptevo is also currently conducting a Phase IV study of Ixinity to obtain approval for use in children under 12. [FDA letter to Aptevo 9/25/20]

CSL Behring Discontinuing Mononine

9/21/20 CSL Behring has announced that they will discontinue Mononine, their plasma-derived factor IX concentrate for hemophilia B. Their decision was based on the declining number of patients currently using the product. CSL estimates that current supplies of Mononine will last until mid-2021, giving patients time to find a replacement for their treatment.

While most hemophilia B patients have switched to recombinant products, some patients actually do better on plasma-derived products. The reasons are unknown. At this point, AlphaNine from Grifols remains the only plasma-derived factor IX product available in the U.S. [Hemophilia News Today article 9/21/20]

GC Pharma and Atomwise Look for Oral Hemophilia Treatments

9/8/20 GC Pharma and Atomwise are partnering to identify and develop oral small-molecule drugs for hemophilia treatment. Atomwise is an artificial intelligence (AI) company whose AtomNet system can screen 16 billion compounds for therapeutic activity in less than two days, using computers. The partners hope to identify a compound that can be taken orally to inhibit anticoagulants and restore the balance in the clotting system. Inhibiting anticoagulants is a popular route for a number of companies developing hemophilia treatments.

GC Pharma, based in South Korea, is the former Green Cross Corporation that owned Alpha Therapeutics. Alpha was the developer of AlphaNine, a plasma-derived factor IX product. AlphaNine and the former Alpha plant in Los Angeles are now owned by Grifols. GC Pharma is the largest plasma product manufacturer in Asia. [Atomwise press release 9/8/20]

Cost to Develop a New Drug

3/3/20 The cost of developing a new drug has been estimated to be between $314 million and $2.8 billion. The costs are difficult to determine because most large, established pharmaceutical companies don't release that information. A new study has tried to pin down the numbers more precisely for drugs approved by FDA between 2009 and 2018. The authors found that in the blood therapeutics area the average expenditure was $793 million.

The costs include expenditures on clinical trials that failed. Data on clinical trial success rates from three previous studies shows that only about 11% of products that start Phase I end up being approved. For products entering Phase II, only about 22% are eventually approved. For products entering Phase III, about 53% gain approval. Products that complete all three phases and are submitted to FDA for approval have a success rate of about 84%. [Wouters OJ et al., JAMA, 323(9) 2020]

Fitusiran Clinical Study Placed on Hold Due to Thrombosis

10/30/20 Sanofi has placed a clinical hold on their Phase III studies of Fitusiran due to cases of thrombosis. Fitusiran is an RNA-interference drug that reduces the body's ability to produce antithrombin, an anticoagulant. The clotting system is normally in balance between clotting factors that promote clotting and anticoagulants that inhibit clotting to help control the system. The decrease in clotting factor activity caused by hemophilia pushes the balance over to the anti-clotting side, making clotting difficult. A number of products under development aim to inhibit anticoagulant activity as a means to restoring the balance so the blood clots as needed without requiring factor infusions. This has to be done carefully, though, because by reducing anticoagulant activity you are also reducing control of the system. Thrombosis is unwanted clotting that can be very harmful, even fatal.

Fitusiran is a monthly subcutaneous drug that is currently in Phase III clinical studies. It was originally developed by Alnylam but was taken over by their partner Sanofi about three years ago. It has been in Phase III studies (the last stage before applying for licensure) and has been showing good results. It appears to be effective for people with hemophilia A and B, with or without inhibitors.

It would greatly decrease the treatment burden for inhibitor patients. However, the study has been paused because of non-fatal thrombosis in more than one patient. This follows an incident of fatal thrombosis in an earlier study phase. Sanofi is working with FDA to determine the next course of action. We have no additional details.

There have been similar issues of thrombosis with some of the other anticoagulant inhibitor products being developed. It may not be the case here, but one problem has been how to treat a patient on one of these drugs if they have a bleed. If you give them factor or inhibitor bypassing agents, you can easily push the balance in the clotting system back toward too much clotting. It's a tricky situation that all of the developers of anticoagulant inhibitors will need to address. [Endpoints News article 11/6/20]

GENE THERAPY NEWS

CSL/uniQure Gene Therapy Update

10/27/20 Dosing of patients in CSL/uniQure's Phase III study of their etranacogene dezaparvovec (AMT-061) has been completed. They plan to report 26-week data on the 54 patients by the end of 2020. [uniQure press release 10/27/20]

Freeline Gene Therapy Update

10/29/20 Freeline Therapeutics has completed dosing in their Phase I/II study. Ten patients have been treated across four dose levels and all have completed six-month follow-up. None of the patients have had bleeds that required factor infusions. They have started enrollment for the lead-in portion of their Phase IIb/III study and expect to start treatment in mid-2021. [Freeline financial report 10/29/20]

Intellia and Regeneron Present Animal Data for Hemophilia Gene Therapy

9/29/20 Intellia Therapeutics and Regeneron Pharmaceuticals have partnered to develop gene therapy treatments for both hemophilia A and B based on the CRISPR gene editing method. The AAV vector methods being used for all of the current gene therapy clinical studies insert the new factor gene as a separate piece of DNA in the nucleus of liver cells. Because an AAV-delivered gene does not integrate onto the chromosomes, the new gene can be lost when liver cells divide. That is a problem for gene therapy in children because their rapidly growing livers would quickly lose their copies of the new gene.

Intellia/Regeneron are using the CRISPR method to integrate the new gene onto the chromosomes under the control of the existing albumin gene. This is a safe spot where the new factor gene won't disrupt the functioning of other genes on the chromosome. The control elements of the albumin gene should also promote the production of large amounts of factor from the new gene. The partners have shown in mice and in non-human primates (NHPs) that their treatment works.

In the mouse study, they showed that production of factor IX persisted for up to one year after treatment. They also showed that when they removed part of a treated mouse's liver, the natural regeneration of the liver restored the pre-removal levels of factor IX. This was in contrast to mice treated by standard AAV gene therapy who lost 85% of their previous factor IX production after part of the liver was removed. In NHPs they were able to produce expression of human factor IX from normal levels (50 – 100%) up to several thousand percent. [Forget AL, presentation at 16th Annual Meeting of the Oligonucleotide Therapeutics Society 9/29/20. Copy available on Intellia web site.]

Pfizer Gene Therapy Update

9/15/20 Pfizer reported that the subjects in their Phase I/II clinical study have demonstrated sustained expression of factor IX in the 20% range for four years. The mean annualized bleed rate (ABR) and the mean infusion rate remain significantly reduced in those patients.

Their Phase III study is now fully enrolled with 40 patients in the lead-in study to establish their baseline bleeding characteristics. Treatment with their fidanocogene elaparvovec gene therapy treatment has begun. Pfizer plans to report interim data with 20 patients at 12 months in 2021.

Pfizer is also looking at increasing their gene therapy dose above the current 5 x 1011 vg/kg level in order to increase the resulting factor IX levels. The competing uniQure/CSL gene therapy is being given at 2 x 1013 vg/kg and producing factor IX levels in the 50% range. However, note the article on the possible risks of high AAV vector doses in the Summer 2020 issue. [StreetInsider.com report 9/15/20]

Be Bio Developing Hemophilia B Gene Therapy Using B Cells

10/22/20 Be Biopharma has launched to develop new gene therapy treatments using engineered B cells. B cells are a type of white blood cell, part of the immune system. Scientists at Seattle Children's Research Institute (SCRI) have developed methods for genetically engineering B cells to produce therapeutics proteins, like factor IX. B cells have the properties that they last for decades in the bloodstream and can produce large quantities of proteins. The large quantities of proteins are normally antibodies,

but the SCRI scientists have shown that B cells can also be re-programmed to produce substances like factor IX instead. The original work at SCRI was done on factor IX gene therapy but Be Bio has not announced their intended target. [Fierce Biotech article 10/22/20]

Durability in Hemophilia A and B Gene Therapies

One of the big questions in hemophilia gene therapy is how long factor production will last. That is, how long will the body continue to produce "good" factor VIII (hemophilia A) or factor IX (hemophilia B) from the newly inserted gene. This is being called the treatment's durability. Ideally, the effect would last for the patient's lifetime. However, until we have data from patients over decades, we won't really know a treatment's durability.

So far, the factor IX gene therapy treatments have shown good durability with subjects from the early University College London/St. Jude study showing no decrease after more than 10 years. However, for hemophilia A, the factor VIII gene therapy studies from Biomarin and Pfizer/Sangamo have shown some decrease in factor production. Biomarin estimates that their factor VIII gene therapy might only last 8–12 years. All of the A and B treatments use similar strategies and AAV vectors, so why is there a difference? Will we eventually see decreases with factor IX as well? Right now, this is a mystery, one of many in gene therapy. [David Clark – news analysis. See also Pierce GF et al., Gene therapy to treat haemophilia: Is robust scientific inquiry the missing factor, Haemophilia, DOI:10.1111/hae.14131, 2020.]

Special Newlyweds!

With a few close friends and family in attendance, Kimberly Phelan and William Gati were married on August 28th at the Montauk Community Church in Montauk, New York. The radiant bride was cheerfully escorted down the aisle by Jojo, their sweet little yorkie, while on the flute, the handsome groom played "Simple Gifts" as he awaited his bride at the altar.

For a brief moment, the groom abandoned his bride at the altar, but it was only to let other well-wishers into the Zoom room! Following a heartfelt exchange of vows, a joyful celebration followed with a delicious wedding cake and bubbly champagne for all to enjoy. The small group then made their way to the beach and continued the intimate celebration on the delightful, warm and happy day!

Congratulations to Kim and Bill! We love you!

HEMOPHILIA UPDATE

BY DR. DAVID CLARK

Pregnancy-Associated Bleeding in Carriers and Women with Hemophilia

Two recent studies looked at bleeding complications during and after pregnancy in women with bleeding disorders and hemophilia carriers.

8/21/20 A study from the UK looked at postpartum bleeding in carriers and women with bleeding disorders between 2008 and 2017: 16 A carriers, 8 B carriers, 8 factor XI-deficient patients and 14 with von Willebrand disease. None of the A or B carriers received factor around the time of delivery. They found that women with bleeding disorders had a significantly higher risk of bleeding during the primary postpartum period (within 24 hours after delivery) despite being managed according to current guidelines. [Wolf S et al., Blood Coagulation and Fibrinolysis, online ahead of print 8/21/20]

8/25/20 A study from seven French hemophilia treatment centers between 2014 and 2019 looked at 104 carriers (A or B) and 119 neonates representing 124 pregnancies and 117 deliveries. They observed 35 bleeding episodes, 83% of which occurred during the postpartum period. 37% of the postpartum bleeds occurred during the secondary postpartum period, which is from 24 hours to 12 weeks after delivery. Three of the neonates suffered cerebral hemorrhages. Caesarean delivery was associated with more postpartum bleeding, and women with factor levels less than 40% were at higher risk of bleeding during the secondary postpartum period. [Nau A et al., Haemophilia, online ahead of print 8/25/20]

Both groups stress that their results warrant further investigation to help improve the experiences of women with bleeding disorders giving birth.

Abnormal Bone Growth in Hemophilia Patients

8/11/20 In rare cases people with hemophilia can grow bone in their muscles and soft tissue. This is called heterotopic ossification (HO). HO is probably caused by bleeding into the muscle or tissue. It is recognized as a possible complication of trauma, surgery or other injuries, but infrequently also affects hemophilia patients. It is usually considered a problem of abnormal healing. Interestingly, in HO, bone grows at three times the normal rate resulting in jagged, painful joints.

In hemophilia, HO occurs most often in the lower extremities, but a recent report describes surgery on a severe hemophilia A patient whose elbow had effectively fused due to HO. He had had pain and limited range of motion already for about 15 years before the joint became essentially immobile. He had no history of trauma to the elbow that could explain the problem. X-rays showed a bridge of bony material connecting his humerus (the long bone in the upper arm) and ulna (one of the two bones in the forearm). The article describes the surgery and subsequent physical therapy performed on the patient. Six months after surgery the patient had good elbow flexibility and the abnormal bone growth had not recurred.

The authors point out that, though rare, HO should be considered as a possibility in hemophilia patients, especially when a decreased range of motion occurs in the absence of joint bleeding or traumatic events. [Pasta G et al., JSES International, online ahead of print 8/11/20]

Good Long-Term Outcomes after Total Knee Replacement

10/11/20 Many hemophilia patients have had, or will end up having, total knee replacements (also known as total knee arthroplasty or TKA). Most results of TKA are favorable as described in a number of articles reporting on short- or mid-term results (as well as a number of informal reports from individual patients). However, a recent study from South Korea is one of the few to report longer-term results. The researchers looked at 78 TKAs in 56 hemophilia patients (70 A knees and 8 B knees) followed for an average of 10.2 years. They found that the average range of motion (ROM) increased from 64.2^{o} to 84.2^{o}. The average American Knee Society (AKS) scores for “knee” and “function” increased from 32.1 to 85.7 and from 41.5 to 83.3, respectively. A score below 59 is considered poor; from 70 to 84 is considered good; and a score above 85 is considered excellent. Having an inhibitor did not affect the results. Patients also showed significant increases in Quality of Life scores.

There were complications. Five knees required hospital readmissions for reasons such as stiffness or joint

bleeding. Three knees required replacement due to infection or joint loosening. Overall, at 10 years of follow-up, 76 (97.4%) replacements were still in place. After 13 years all but one (96.2%) remained functional. The main finding is that mid- to long-term results with TKA are satisfactory in terms of pain relief, functional improvement and Quality of Life. [Bae J-K et al., Journal of Clinical Medicine, DOI: 10.3390/jcm9103247]

Primary Prophylaxis is Associated with Lower Joint Degradation

9/6/20 Hemophilic joint damage, also called hemophilic arthropathy (HA) is caused by bleeding into the joints. A number of studies have shown that primary prophylaxis (regular infusions with factor IX starting after the first joint bleed or before the age of three) can significantly decrease HA. A new study from Colombia looked at 362 men with hemophilia B over the period 2015 – 2019 and found similar results. They found that those on primary prophylaxis had significantly decreased HA compared to those on secondary or tertiary prophylaxis. Secondary prophylaxis is started after two or more joint bleeds or after the age of three. It may be continuous or intermittent (e.g., only on days of sports participation). Tertiary prophylaxis is started after some joint damage has already occurred with the aim of reducing further damage. The bottom line is start early and infuse regularly. [Vargas JAH et al., Haemophilia, online ahead of print 9/6/20]

Low Bone Mass and Low Vitamin D Levels are Common in Hemophilia

8/25/20 Interestingly, a recent study shows that patients with moderate and severe hemophilia A or B, except moderate Bs, tend to have low bone mass and impaired bone strength, regardless of age. The study looked at the association of low bone mass with vitamin D levels in 78 A or B patients under 50 and in 33 patients older than 50. They did not find a correlation because all of the patients had low vitamin D levels. The average vitamin D levels in the subjects were about 20 ng/ml, while the normal range is 30 – 100 ng/ml. They did find that hemophilia A patients under 50 had significantly more occurrences of low bone density in the femoral neck (the top of the thigh bone that usually fractures when someone breaks their hip). Above age 50 there was no significant difference between patients with hemophilia A or B.

Vitamin D levels did not vary significantly with hemophilia type (A or B), severity (moderate or severe) or age. These findings highlight the need for most hemophilia patients to supplement their diet with additional vitamin D. [Linari S et al., Haemophilia, online ahead of print 8/25/20]

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.

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

Takeda

Important facts about RIXUBIS®:

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

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

What is RIXUBIS used for?

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

Who should not use RIXUBIS?

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

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

What should I tell my healthcare provider before using RIXUBIS?

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

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

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

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

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

What are the possible side effects of RIXUBIS?

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

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.

Issued 05/2018
S46187 02/19

Takeda

BOX & DINNER REGISTRATION DEADLINE DECEMBER 28

FREE B COMMUNITY EVENT

SATURDAY, JANUARY 9, 2021

ACTIVITIES & PRESENTATIONS 1:00- 4:00PM CST

DINNER: 5:00- 6:00PM CST

FOR FAMILIES IN: TEXAS, ALABAMA, MISSISSIPPI, AND LOUISIANA

We bring patient education to you
because we know hemophilia affects the whole family

MEETING WILL INCLUDE **KAHOOT TRIVIA, RAFFLES, AND A DINNER PRESENTATION, WITH FOOD VOUCHERS** PROVIDED COURTESY OF THE COALITION

The meeting is **FREE**, but you **must register BY JANUARY 6** to receive the Zoom link and the agenda

REGISTER NOW: www.hemob.org/new-events

AT EVERY VIRTUAL MEETING, RECEIVE EDUCATION, ADVOCACY RESOURCES, AND SUPPORT

THE COALITION FOR HEMOPHILIA

Contact: Farrah Muratovic
farrahm@hemob.org
Phone: 212-520-8272

SPONSORED BY
CSL Behring
Biotherapies for Life™

BOX & DINNER REGISTRATION DEADLINE JANUARY 5

FREE B COMMUNITY EVENT

SATURDAY, JANUARY 16, 2021

ACTIVITIES & PRESENTATIONS 1:00- 4:00PM EST

DINNER: 5:00- 6:00PM EST

FOR FAMILIES IN: NEW YORK, NEW JERSEY, MASSACHUSETTS, CONNECTICUT, NEW HAMPSHIRE, MAINE, RHODE ISLAND, AND VERMONT

We bring patient education to you
because we know hemophilia affects the whole family

MEETING WILL INCLUDE **KAHOOT TRIVIA, RAFFLES, AND A DINNER PRESENTATION, WITH FOOD VOUCHERS** PROVIDED COURTESY OF THE COALITION

The meeting is **FREE**, but you **must register BY JANUARY 13** to receive the Zoom link and the agenda

REGISTER NOW: www.hemob.org/new-events

AT EVERY VIRTUAL MEETING, RECEIVE EDUCATION, ADVOCACY RESOURCES, AND SUPPORT

THE COALITION FOR HEMOPHILIA

Contact: Farrah Muratovic
farrahm@hemob.org
Phone: 212-520-8272

SPONSORED BY
CSL Behring
Biotherapies for Life™

BOX & DINNER REGISTRATION DEADLINE JANUARY 12

FREE B COMMUNITY EVENT

SATURDAY, JANUARY 23, 2021

ACTIVITIES & PRESENTATIONS 1:00- 4:00PM EST

DINNER: 5:00- 6:00PM EST

FOR FAMILIES IN: FLORIDA, GEORGIA, PENNSYLVANIA & WEST VIRGINIA

We bring patient education to you
because we know hemophilia affects the whole family.

MEETING WILL INCLUDE **KAHOOT TRIVIA, RAFFLES, AND A DINNER PRESENTATION, WITH FOOD VOUCHERS** PROVIDED COURTESY OF THE COALITION.

The meeting is **FREE**, but you **must register BY JANUARY 20** to receive the Zoom link and the agenda

REGISTER NOW: www.hemob.org/new-events

AT EVERY VIRTUAL MEETING, RECEIVE EDUCATION, ADVOCACY RESOURCES, AND SUPPORT.

THE COALITION FOR HEMOPHILIA

Contact: Farrah Muratovic
farrahm@hemob.org
Phone: 212-520-8272

SPONSORED BY
CSL Behring
Biotherapies for Life™

BOX & DINNER REGISTRATION DEADLINE JANUARY 19

FREE B COMMUNITY EVENT

SATURDAY, JANUARY 30, 2021

ACTIVITIES & PRESENTATIONS 1:00- 4:00PM CST

DINNER: 5:00- 6:00PM CST

FOR FAMILIES IN: ILLINOIS, MISSOURI, MINNESOTA, IOWA, AND NEBRASKA

We bring patient education to you
because we know hemophilia affects the whole family

MEETING WILL INCLUDE **KAHOOT TRIVIA, RAFFLES, AND A DINNER PRESENTATION, WITH FOOD VOUCHERS** PROVIDED COURTESY OF THE COALITION

The meeting is **FREE**, but you **must register BY JANUARY 27** to receive the Zoom link and the agenda

REGISTER NOW: www.hemob.org/new-events

AT EVERY VIRTUAL MEETING, RECEIVE EDUCATION, ADVOCACY RESOURCES, AND SUPPORT

THE COALITION FOR HEMOPHILIA

Contact: Farrah Muratovic
farrahm@hemob.org
Phone: 212-520-8272

SPONSORED BY
CSL Behring
Biotherapies for Life™

"ON THE ROAD" VIRTUAL MEETINGS

Each Virtual on the Road Meeting will have designated states.

January 9, 2020:
TX, AL, MS, LA

January 16, 2021:
NY, NJ, MA, CT, NH, ME, RI, VT

January 23, 2021:
FL, GA, PA, WV

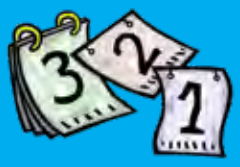
January 30, 2021:
IL, MO, MN, IO, NE

February 6, 2021
February 20, 2021

February 27, 2021
March 13, 2001

March 20, 2001

More information to follow.



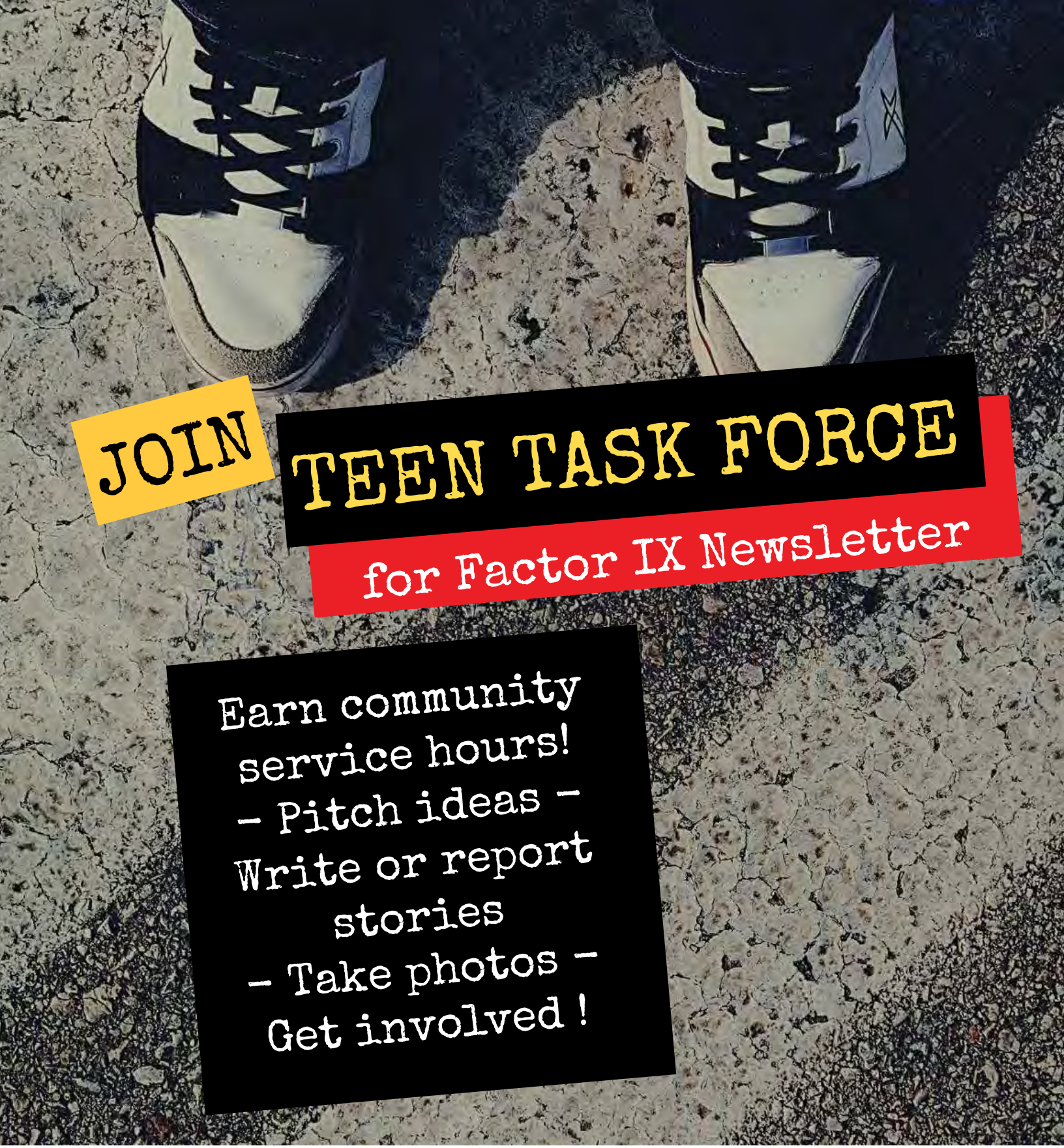
The Coalition for Hemophilia B

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

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



JOIN TEEN TASK FORCE

for Factor IX Newsletter

Earn community service hours!
- Pitch ideas -
Write or report stories
- Take photos -
Get involved !

Join the Coalition for Hemophilia B Teen Task Force! Email Rocky Williams for more info: Rockyw@hemob.org

FROM SCRATCH

BY ROCKY WILLIAMS

Over the course of three weeks in November, the Coalition for Hemophilia B hosted the first virtual Generation IX Teen Mentorship program, and it was a smashing success! From scavenger hunts to baking to leatherworking to TikTok, the event was full of surprises and learning experiences organized around the theme "from scratch." The Teen Mentorship program, which started in 2014 as the first-program of the Generation IX Project, was sponsored by Medexus Pharma and co-presented by our program partner GutMonkey who specialize in experiential learning.

"Gen IX Mentorship is a unique program that brings together volunteer adult mentors and teen participants from all over the country to laugh, grow, and make lasting connections," said Jacob, one of the mentors. "I found the training provided by GutMonkey and CHB rewarding as I learned how to reach and engage with individuals with different personalities and encourage them to shine. Through tackling creative challenges and open discourse about life with hemophilia B, I felt uplifted witnessing firsthand the hearts and minds of the next generation that will become leaders in our community."

Many others expressed similar views. "I love this program because it helps you to make deeper connections with people who experience the same issues that you do," said Ron, another mentor. "They are like an extended family that you see three times a year." According to Andrew, a mentee, "the Gen IX Teen Mentorship Program was such a fun and refreshing experience. I felt that this program is perfect to get rid of worries from the outside world!"

During the three weeks of the program, mentors met on Mondays, and mentors and mentees met on Thursdays. At the first combined meeting, participants established a set of agreements which included things like "show up and stay, make space and take space, choose your challenge, and engage tension, don't indulge drama." Mentors and mentees then moved on to fast-paced icebreakers called Gen IX Frenzies and a scavenger hunt for items around the house, such as "grab your favorite snack that you have in the kitchen right now, go find something you haven't used in the past month," and "find something that represents your theory on the purpose of life." The activity was a fantastic way to get people sharing fun things about themselves while learning about others."

The "from scratch" theme was in full swing as attendees dived into boxes they received with items for baking and leatherworking. The mentors worked as a team to create a graham cracker pizza dough that they later used to create s'mores dessert pizzas. It involved a lot of firsts as few had made dough before, much less with yeast and graham crackers as main ingredients. Mentors and mentees learned leatherworking together by making a passport or notebook holder from scratch. The process involved using a lot of tools that were new to many, including metal rulers and rotary cutters to measure and cut the leather, and an awl to poke holes in the leather. They also learned how to dye leather and practiced stitching.

Games brought the attendees together in both competition and collaboration. The B-Anomia game involved thinking of items that fit a category and a letter, like "donut flavor + S," and participants came up with an item that fits the

description and are awarded points for quick and correct answers. Although some might say there are no wrong answers, salsa as a donut flavor got a “thumbs down.” There was a lot of shared laughter throughout the game!

Collaboration is important too, so the attendees played a Mentorship Road Rules Challenge. They divided into teams who were each in charge of virtually navigating a GutMonkey staff member around the St. John’s neighborhood in Portland to various checkpoints. Mentors were not allowed to talk and had to find alternative ways to communicate with mentees.

“This program, for me, was an opportunity to hang out with other people who understand bleeding disorders,” said Bryen, a mentee. “Having hemophilia isn’t an issue with this group because everyone has ‘been there, done that, got the t-shirt.’ I liked the co-op game part of it. I don’t get to do a lot of games with friends, so it’s nice to be able to have the opportunity.”

The comedic highlight of the program was learning to create TikTok videos about misconceptions on hemophilia. Mentors and mentees worked together to create the videos. One group showed someone with hemophilia B supposedly needing to always be wrapped up and protected. They also illustrated the wrong belief that a small cut could cause someone to bleed to death.

“I absolutely loved this program,” said Kyra, a mentor. “GutMonkey never fails with putting on an amazing event. There was so much valuable information to be absorbed that I could use in my job as a program manager.” According to Ron, “this experience taught me some life skills that I can apply to my own life through situations that I have to encounter in the future. Great and excellent program with amazing people who lead it with precision. From the bottom of my heart, I want to say thank you and I am truly grateful for this program.”

For more information about the Generation IX Project, please visit our website at www.hemob.org.

Thank you to Medexus for generously supporting this program!

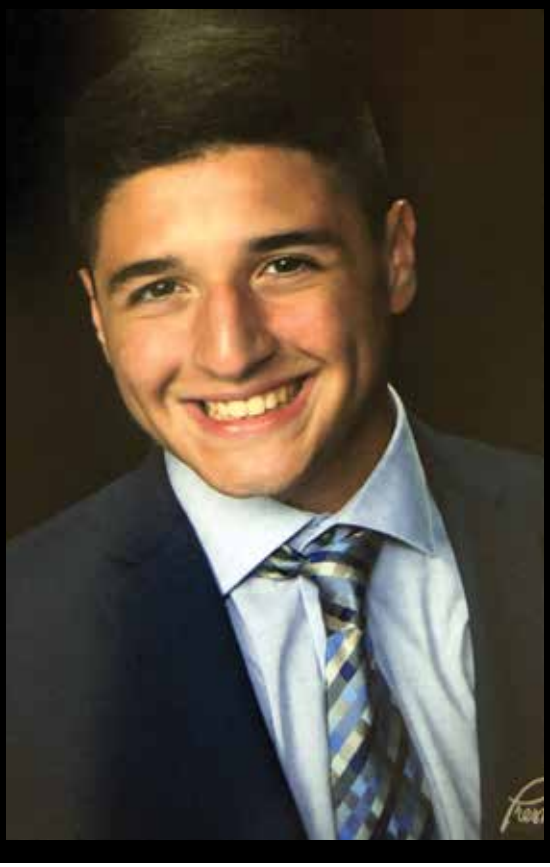
MEDEXUS PHARMA

Reaching Maximum Potential Through Dedication and Discipline

BY STEVEN

Hi, my name is Steven. I am eighteen years old. I am currently a freshman at Utica College. I am enrolled in the Advanced Doctor of Physical Therapy Program, which means I will graduate in six years rather than seven, and I am a middle infielder on the Utica College baseball team.

Oh, and I'm have hemophilia.



Before I get into my rant about how much I love sports, let me give you some background information about my hemophilia. I have severe hemophilia B, and I have been self-infusing since I was ten years old. Okay, now back to sports. I have been playing sports since I was four years old, specifically baseball.

Baseball has always been the love of my life. Although I used to also play flag-football and organized basketball, both of which I enjoyed immensely, nothing has ever compared to my love for baseball. Now I know what you are all thinking, "Is this kid crazy? He's going to get a joint bleed." And my response to that is, "Yes. Yes, I am crazy. I am crazy dedicated to not falling in love with mediocrity." This means that I want to achieve all that I desire.

From a young age, I have desired to play baseball as long as God would let me. So far, I have a fourteen-year career under my belt. And to be fair, I can say I have a lot of things: I have a lot of championships, and I have a lot of personal awards and recognitions, but one thing that I do not have is an injury problem. I work hard to strengthen my muscles to protect my joints, I'm aware of my body's limits and I NEVER miss my prophylaxis infusions!

If a person with hemophilia uses their medication properly and is aware of their physical limitations, I recommend that they give sports a try after speaking to their parents and HTC/doctor. However, I AM NOT influencing anyone with hemophilia to participate in extreme contact sports. The only reason that I have maintained the success that I have with my sports career is because I know my limitations and have worked hard to understand how my body reacts to the physical activities I participate in. Everybody, hemophiliac or not, needs to know their limitations in this world.

For instance, transitioning from high school to college is already hard enough. Add to that new routine, the new routine of a pandemic and still remembering to properly infuse. Now you might find yourself overwhelmed. COVID-19 has already taken so much from me. It has already stripped me of my senior year experience.

I lost things that include my high school varsity baseball season, my senior banquet, and a true graduation. Even in college, I am losing a full experience because there are so many limitations. I have to wear a mask at all times. The only exceptions to having to wear a mask are if I am in my room with only my roommate or eating in the cafeteria. Also, students are only allowed to have four people in a room at all times and aren't allowed to enter other dorming halls.

I have lost my fall college baseball season. This is where the coaches evaluate incoming players. We are allowed to practice, as long as we are twelve feet away from each other and are wearing masks at all times. There are also coaching restrictions. Things like practice duration and scheduling are totally different from regular years, courtesy of the NCAA. The NCAA is making it extremely difficult for graduating high school seniors to get recruited because there are even strict rules on talent scouting now. So, my heart goes out to those who are struggling to find their college now.

I was blessed to have committed to play baseball at Utica College on October 26, 2019, (i.e. pre-coronavirus.) However, I can't complain. I am here, I am healthy, and I am enjoying myself. This is more than some people can say about their lives during this hectic year. On top of this, I have straight A's. The workload is much different than high school, but it has its pros and cons. The biggest pro and con has to be time management. If you have solid time management skills and strategies, then you're in good shape.

College comes with freedom. If you choose to sleep instead of doing your work, you will risk falling behind. Luckily, I can say that my time management is not a problem for me, so the freedom is definitely a pro in my book. Overall, college is a great experience and I love it here at Utica College.

I would like to encourage everyone with this message: there is hope. The medical field is continuously advancing their treatments for hemophilia. I believe eventually there will be a vaccine for COVID-19 and we won't have to be restricted to seeing half of a person's face. I also believe the world can only go up from here. So, put on a mask, get outside, and push yourself to get better at whatever it is that you are passionate about.

Whether you're going to play sports or become the next President of the United States, don't let anything stand in your way. And most importantly, don't be defined by anything or anyone. Be the author of your own story!

It's More Fun for Teens Who Make Treating Routine

AN INTERVIEW WITH JERMAINE
BY RENAE BAKER

Jermaine is approaching 19 years of age but says he's "just a kid at heart." He is one of the 2020 high school grads with whom future generations will surely be fascinated. They will likely ask, "What was it like to study hard for four years and then not have a normal graduation ceremony or celebration?"

As he relates it to me, via Zoom, nearly five months post-graduation, "This year has been awful!" Jermaine had been doing better in school before March when the threat of Covid-19 shuttered his school, and all of his classes went online. He has ADHD and being with his friends had been a motivating force for him when it came to understanding and catching up with homework. By himself, in front of a screen, he found himself easily distracted. He did earn his high school diploma, but graduation had been "weird;" a lineup of cars, the participating graduates getting out of their car at the front of the line for a minute to receive their diploma, and then slipping - just as quickly - back into the car. Jermaine didn't see the pomp and circumstance in the plan and found it too depressing to participate.

He thinks back to what he misses the most from the "pre-covid-19" days - hanging out with friends, engaging in "probably too many rough sports" and just having fun. Back then, Jermaine admits, sheepishly, he wasn't careful enough about his hemophilia B. "I'm social. At school, I acted like a normal person. I told people I had to be careful, but I wasn't. I did things that I shouldn't have been doing." He would just tell himself "Bleeds happen." He was so used to having them, that they didn't alarm him.

Jermaine started self-infusing when he started high school and "Mom said I was too big to hold down anymore," he says, then quickly adds, "I started learning to self-infuse when I was eleven, but I always had someone else do it, unless I was basically bribed with money!" Jermaine lets out a quiet laugh at the memory of his younger, more mercenary self.

Jermaine admits that, once in charge of his own infusions, he didn't treat regularly. "I just didn't want to."

"This is not uncommon in teens," says Jodi Aman, licensed clinical social worker and author of *Anxiety... I'm So Done with You: A Teen's Guide to Ditching Toxic Stress and Hardwiring Your Brain for Happiness*, July 7, 2020. "When the consequences are not immediately tangible, it creates a choice to delay or procrastinate action for one's highest good. Teens are more susceptible to this because their prefrontal cortex is not fully developed, so it's more difficult to override the urge to resist doing something hard even though it may be the best thing for you."

Aman explains, "Your brain has two purposes. One is to survive and thrive, and the other is to preserve calories. When people are faced with doing something they don't want to do, there is a biological resistance that comes up. If your brain doesn't perceive it as something you need to do to survive and thrive right now, it's not worth using the calories up. When we get older, it's easier for us to override this because we realize this is something we do need to survive and thrive even if the results are not immediate. Teens, on the other hand, are hard-wired to resist. They may seem unmotivated or lazy, but they are actually highly motivated to resist. It's a biological response."

Jermaine is from a family of bleeders. He, his mom, uncle and cousins are well-connected to The Coalition For Hemophilia B. Jermaine tells me that his hemophilia B

journey has been “good and bad.” He credits much of the good to the coalition because, “I get to do a lot of fun stuff and go places. “Like I said, I’m a social person. What I like best about coalition events are the people I get to meet. It’s good to meet people my age who understand.”

Presently and quite suddenly, Jermaine understands more of what some of those people have been through and wants to share a story about a recent experience to enlighten those who haven’t yet experienced this. “I hadn’t been treating with prophy regularly. In fact, in the entirety of all last month, I only treated once. Then I had a bleed.” I asked him what happened. “I just woke up!” he said with a fresh, new, sober understanding of hemophilia B. A week into this bleed at the time of our interview, Jermaine explains that it is a bleed deep in the muscle in the groin area. “I went the entire day thinking it was a normal bleed, but it was really bad.” So, the next day, he and his mom went to the ER and had three ultrasounds, x-rays, blood and urine tests. He had to rely on crutches to get around. “I couldn’t sleep. There was pain the whole time. I actually cried. I had never taken pain meds before but asked for some for this!”

The next morning, his mother was at work when Jermaine awoke. He knew something was very wrong. “I crutched my way over to Grandma’s and called mom. ‘My thigh is numb!’” Jermaine’s mom called the HTC and was advised to go back to the ER. “It was a bad bleed deep into the groin muscle.” He was admitted to the hospital and placed on complete bed rest, which he assures other teenagers is “NOT FUN! I had never been on bed rest before, and my skin was crawling!” It would be hard to overstate the pain, irritation, boredom and lack of fun this misadventure has caused Jermaine, but he values the new lens through which he now views managing his treatment. “Now I’m looking at regular treating as something I want to do.”

Jermaine, freshly released from the hospital, reports that he has high blood pressure, so he will be checking in with his regular physician. Then the plan is to infuse factor every forty-eight hours and check back with the HTC in three days for more details. “I’m stubborn,” Jermaine acknowledges with self-effacing honesty, “but this bleed was the straw that broke the camel’s back.”

When I asked him what advice he has for other teens with hemophilia B, Jermaine says, very frankly, “Well, I don’t want to be a hypocrite. I understand they don’t want to (infuse factor regularly,) but you won’t be hurt as much or have as many bleeds if you do. I used to have the same mentality as you man, but when this happened to me, it all changed. I know it’s a nuisance, but it doesn’t take long. If it hurts, use numbing cream. You gotta make sure you take your factor man. Just take your factor.” These days, Jermaine is convinced that those few routine, disciplined, (if unpleasant,) moments of treating, will help ensure more fun in his daily routine.

Jermaine draws particular strength from one of his coalition experiences during which he flew out to Portland, Oregon for a Gen IX event. Pat “Big Dog” Torrey, “A great guy; super-funny,” as Jermaine describes him, provides the excellent leadership for this program. “The cabins were in the treetops, and we did these rope courses and a swing with giant pulleys. I thought - you know - anything could happen! But I liked it ‘cuz it was fun, and it really built trust with the great, new friends who came in from everywhere!”

It may very well be this kind of strength that helps Jermaine look toward the future. He has his sights set on continuing his education to go into the field of phlebotomy. “That’s basically poking people with needles. I feel like I’d be good at that. I have some years of experience!”

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Fall 2020

IN THIS ISSUE:

- **It's More Fun for Teens Who Make Treating Routine**
- **Join the Teen Task Force!**
- **Reaching Maximum Potential Through Dedication and Discipline**
- **Gen IX Virtual Mentorship Program From Scratch**

JERMAINE

It's More Fun for Teens Who Make Treating Routine

STEVEN

Reaching Maximum Potential Through Dedication and Discipline