Factor Nine News

The Coalition for Hemophilia B

Fall 2019

Topics in Hemophilia B

- The Beats Music Program
- Generation IX Project
- Men's Retreat
- Women's Retreat
- Treatment News
- Research Update
- The Coalition Goes *On The Road*
  - Columbus, OH
  - Knoxville, TN
  - Atlanta, GA
  - Seattle, WA
  - Baton Rouge, LA
  - Fort Smith, AR
  - Phoenix, AZ
  - Schaumburg, IL
- 2019 Survey Coming Now!
- BConnected
- William N. Drohan Scholarship

THE BEATS
PAGE 2

SPREADING HARMONY WITH THE NEW BEATS MUSIC PROGRAM

BY MICHAEL PERLMAN

A melodious tune and a repertoire of harmonies are sweeping through The Coalition for Hemophilia B, whose mission has become more widespread as it continues to reach a more diverse audience with the newly launched Beats Music Program. A music program for Coalition members 18 and over debuted at the Gaylord Opryland Resort in Nashville, "Music City, U.S.A." on July 31 to August 4, 2019, and it became a shining success! Based on the power to engage the public through music and establish strong bonds for the bleeding disorders community, an annual tradition is underway.

Offering a multitude of benefits, music becomes a cultural and therapeutic bandwagon for those who play instruments, sing and become an audience or a fellow musician. Playing music is responsible for achieving unity and minimizing loneliness. It becomes a score for leadership skills, stress relief, elevating one's mood, boosting social interactions and sparking friendships, initiating creative thought and independence, teamwork, and generosity of spirit. Music bears the capacity to release endorphins, hormones secreted within the nervous system and brain to minimize one's perception of pain to foster an improved state of well-being.

An estimated thirty to sixty percent of people with hemophilia face depression, based on scientific research. According to a recent survey of Coalition members, a majority has "gravitated towards music as an outlet of expression and exploration." The Beats Music program is designed to nurture and empower those who have shown an interest in using music as a source of comfort and a means of self-expression. It offers an opportunity to Coalition members to grasp knowledge and inspiration from professional musicians and one another. Most importantly, this new and innovative program enhances the quality of life for people with hemophilia. According to the Coalition, it "establishes a support network between participants and their mentors and by encouraging participants to express and share experiences with others, inside and outside of the hemophilia B community."

Participants shared a wide range of feedback. Some testimonials are “The Beats is a program they should have created years ago. There are so many talented people with hemophilia out there. Getting them in a room with instruments is something we as people with hemophilia have been wanting forever” and “Confidence building! The collaboration was awesome!” A Coalition member said, “Not only was the program beneficial to the spirit but being able to share fellowship with others, to come together around a common passion for music, was inspirational and uplifting!” Inspirational feedback is also evident through “Although I have been into music since I was a kid, this program helped reignite my love for making music. It was also great to play with other blood brothers!” Another member stated, “This experience was an all-around showstopper! This community pulled together and pulled off a memorable musical performance. The connections that were built, the knowledge we learned and coming out of our comfort zone to make it all happen was awesome!”

Three musicians with hemophilia who performed dedicated their time to share in-depth stories of their background, aspirations, and how they contributed to The Beats Music Program, which in turn has assisted them.

Meet Michael Mohon, who is about to turn 32. He was diagnosed at birth. Hemophilia runs in his family for at least a few generations. He was raised in Phoenix, Arizona and is the assistant manager of a bar and restaurant and has aspirations of running a restaurant of his own. He said, “At an early age, I learned that life is very valuable. The life I know and live may not be the same tomorrow; thus, making me a stronger individual by celebrating my life each day to its fullest. “Without my mother and the care she provided, I would not be the person I am today. I am also thankful for my H.T.C. for giving my mother and me the tools to be a successful person with hemophilia with minimal bleeds and joint issues.”

Also, meet Zach Marcella who was diagnosed at 9 months and is now 27. Born and raised in Paris, Tennessee, he now resides in Dickson. His professional pursuit is to


WELCOME












obtain BSN credentials to more adequately serve the organization he is employed with, as well as "to become a better servant within the hemophilia community as a whole," he explained.

Shelby Smoak is 47 and was diagnosed with severe hemophilia B at 18 months. Since hemophilia did not run in his family, it caught them by surprise after a significant bleed. Shelby calls the Virginia Shenandoah mountains home and is an advocate and educator for Biomatrix Specialty Pharmacy. He is also active in the Coalition's programs including the Annual Symposium.

peak. With music, amazing stories and the people around you, it was unexplainable. My first impression was to be a fly on the wall to absorb everything that I see. Soon after, I opened up and interacted, allowing myself to learn more of what I can do to become a balanced musician."

He feels the main pro was the ability to collaborate with amazing participants. "We brought an idea we had in our heads and turned it into a physical thing that one day will be heard by other people with hemophilia, and light that fire inside that I got on this trip. Our song received generous compliments and feedback has been more than uplifting and confidence-boosting."

"THE SUCCESS OF THE PROGRAM HAS OPENED DOORWAYS AS ATTENDEES ARE NOW PARTICIPATING IN OTHER EVENTS THEY NORMALLY WOULD NOT ATTEND."

Kim Phelan Vice President

Two years ago at the Coalition's Men's Retreat in Arizona, Michael felt privileged to join his first program. Fast-forwarding to the Beats Music Program, he shared, "The moment that stands out most was writing, performing, and recording a song in less than 72 hours.

The chemistry between Zach Marcella on vocals, Collin Johnson on guitar, Adam Smith as producer and engineer, and myself on guitar were magical. It was everything that I imagined Nashville to be!" Their song titled *Silver Linings* became an attraction. He explained, "During the writing process, we focused on the idea of things which pull us from the darkness and storms that having hemophilia can bring. There is always a silver lining."

Back in freshman year of high school, Michael picked up his first guitar. He reminisced, "Music was the only option I had to entertain myself while having a bleed since you can only beat a video game so many times. Once I began playing, it opened more doors for me, and a therapeutic release turned into a passion."

As for The Beats Music Program, he said, "I was able to connect with other people who have been in the same boat as me, and soon realized that the same boat was more like a cruise ship with endless growth and possibilities." He felt he was able to grasp more rewards than he could ever imagine from its inaugural season. "The setting helped bring my creativity level to a

"Schools are eliminating music programs countrywide, so this may be the only outlet that the hemophilia community has," said Michael. Looking forward, he visualizes "expanding the music family to a network of musicians" to create even more music. "I wish it would be at least ten days. If given more time to collaborate with other musicians, it would produce more ideas and more songs. If some guys and I can write and record a song in three days, then anything can happen."

Zach Marcella collaborated with Michael Mohon and Collin Johnson for the song *Silver Linings*, with a primary focus on writing lyrics and performing vocals. As a singer, he

also collaborated with other musicians. “I enjoyed the experience of performing covers, as well as throwing new ideas at a wall and seeing what would stick,” he said.

He discovered his passion for music at age 5. “I begged my mother and my church’s music director to let me sing in front of the congregation. I’ll never know what the catalyst was that sparked my desire to sing, but it’s been a wonderful love affair ever since.” He also recalled the period when his voice began changing. “Simultaneously, I was taking piano lessons, so my love of singing transitioned into playing the most intricate classical pieces that I could comprehend. After that point, I started getting curious about women, figured I would be a little bit more noticeable if I grabbed a microphone, and the rest is history.”

Zach felt his experience at the inaugural Beats Music Program was “entirely positive.” He explained, “I enjoyed my time at the Gaylord Opryland Resort, as well as gaining knowledge from my experience shared with participants in off-site activities. While it is not always the easiest to step outside of one’s comfort zone, it is important for growth. Pushing themselves to perform on stage for the first time, putting their heart and soul into an instrument, or simply participating in activities they previously would have excluded themselves from showed that the program is capable of providing effective personal growth.” He continued, “I often have moments where I doubt myself to be adequate to survive, but this program provided an inspirational boost to my morale along with more confidence and vigor in my daily life.”

He often reflects upon Bruce Lee’s famous quote about

the adaptive nature of water. “Music can also encompass that adaptive principle. It can provide accompaniment to every emotional state known to mankind. Music takes its own presence and purpose, even above the intentions of the original composer. I have obtained hope from songs about despair and happiness from songs about anger and vice-versa.”

Zach feels classical music is the best reflection, where there are no lyrics that may otherwise confuse the listener. “There’s only a single individual or orchestra offering their soul into an instrument and letting the sound that is emitted convey their emotions. One major advantage of the Beats Music Program is the ability to let individuals experience this emotional transference and the therapeutic nature of releasing emotional buildup through the instrument of their choice.”

For Zach, the connections he established are extremely positive stories. He explained, “It’s not just in meeting the members and leadership of the Coalition, but by bonding with other blood brothers and sisters that share similar emotional burdens. I now have a new song completely written and recorded by members of this community to remember the Beats Music Program.”

He is maintaining communication with Michael and Collin. “We would love to continue composing new ideas and music that resonates with members of this community and beyond, which is a direct result of this program.”

In it for the long-term, Zach envisions the program’s expansion. “I would love to see musicians playing the trumpet, trombone and saxophone in future programs, as well as a greater representation of Big Band and jazz.”

As someone with hemophilia, his life challenges have significantly impacted his belief system and philosophy. “My bleeding disorder has required me to find strength beyond what I thought was possible and what I am

capable of just to breathe. We each have our puzzle to assemble. We are born into this world with no instruction manual. For some, attempting to find a way to fit the whole box of pieces together can be so stressful, that the individual never truly begins to assemble the puzzle. Others are forced to assemble the puzzle from day one. Sometimes assembling that puzzle is the only way an individual can continue to survive."

Zach feels life is never to be taken for granted and it is essential to strive for individual solutions to problems. He explained, "We must dedicate ourselves beyond 110%. In becoming the best version of one's self, the individual will often find that the help along the way only improves. Maintaining healthy spiritual, physical, and emotional well-being is paramount to the ability to perform in the highest capacity."

Shelby Smoak began taking piano lessons in elementary school, but upon discovering the guitar in high school, "the love affair was cemented." At the Beats Music Program, he played solo originals which consisted of *Hey There Devil* and *Rusted and Raw*. The experience exceeded all of his expectations. He explained, "I came away with my first 'official' guitar instruction, especially after over 30 years of playing! I learned there is so much to learn and I also developed new friendships and a great appreciation of all the talent our community has to offer. Nashville provided the perfect inspiring musical backdrop!"

"The Beats Music Program can introduce someone to the therapeutic values of music, especially if somebody has consistently claimed they "have no musical talent,'" which Shelby claims that the latter statement is a myth. "Everyone can find something musical to do and will benefit from that experience." As for more seasoned musicians, he stated, "The program provides an unparalleled musical connection with other musician members in the community who struggle in ways that are unique to those with a bleeding disorder." Above all, the program will "offer everyone a more focused appreciation for music and how it can benefit you."

Among the many positive observations that Shelby had was a standout where a participant was very shy when it came to singing. "They thought they had very little to offer musically. They came to be involved and 'change things up,' as I heard them say. They became tired of some of the more typical conference-style programming. Being shy, he bucked much participation at first, but then through encouragement and support, he started to find his voice, and by the end was comfortable enough to sing before groups. It struck the chord that this isn't a program about talent necessarily, but about finding something like music that can break down those inhibitions and barriers that a bleeding disorder has erected."

Shelby participates in various music education programs. He feels that music's effect on the brain is unlike any other art form. "It is all brain. Some describe the effect as fireworks going off in your brain. It involves many parts of the brain regarding movement, memory, speech, and auditory functions among others. Music has been a support which calms me during pain and supports me during trauma. It is cathartic in all respects."

"Another Beats Music Program really needs to happen, and hopefully more people will learn about this opportunity and take advantage of it," he noted.

On another note is a Westfield NJ resident who is a professional singer, actress, author, and an acting and music coach named Renae Baker, who does not have hemophilia and has made a difference for the Beats Music Program and beyond. Along with Joe Turley accompanying on piano, she conducted the master class, *Expressing Yourself Through Song* and coordinated one-on-one singing sessions. She stepped right up to help create the show and became what she calls the "accidental MC for the show." She already plans to voluntarily coordinate and host next year's program.

As far as she can recall, she has "a love of tight harmony singing." She explained, "It inspired me to create a company of holiday carolers in 1997. This has given me a lot of experience bringing people together in song and how that creates peace between people. I gave a TEDx talk called *Can Caroling Lead to World Peace*? and my many years of caroling experience has also given me insights into Christmas spirit regarding what it symbolizes to people, what it can do for people, and how it hurts to be without it when we need it." This led her in the direction of writing a book, *Defeating Scrooge - How to Harness the Power of Christmas Carols to Revive Your Spirit Any Time of Year*, in addition to leading *Spirit Saver* workshops, which includes an online version.

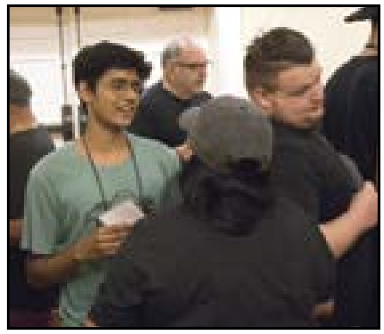
As an acting coach, Renae assists children and adults for auditions, landing them on Broadway, TV, and film. "For the Beats Music Program, I guided the singers to important performances by helping them make strong emotional choices and truly express themselves through song," she said. She has been singing since childhood and began playing piano at 9, which enhances her vocal coaching skills.

Among the performance numbers that Renae coached were *The Apple Tree Song*, *Don't Let the Old Man In*, *Great Balls of Fire*, *Photograph*, and *Hootchie Cootchie Woman*. She shared a significant advantage of The Beats Music Program. "Most participants who are not professionals need the healing, uplifting experience of making music, and are encouraged to come together to create, learn, and stretch themselves further in a safe environment, among the very special group that understands their experience."

Renae also explained music's immense therapeutic effect.



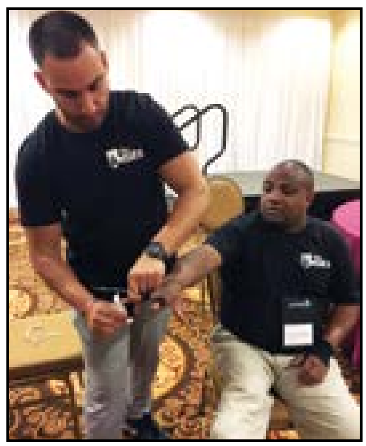
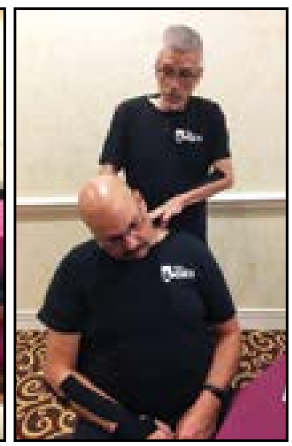














"Physically, music vibrates through you and raises your endorphin level, making you feel happier and in less pain. Singing makes you breathe more deeply, which feels good. How funny that we need the reminder to spend time breathing deeply, but most of us do and feel better almost instantly. Also, people need to relax neck and jaw muscles to sing and letting go of that tension not only feels good but can stave off diseases. Singing with the important emotional, dramatic choices and backing up the voice expands your sphere, taking you higher and connects your experience with others."

Renae felt the classes and workshops were informative, fun and offered a great means of connecting. She explained, "Having Joe as our accompanist was a blessing, since he has the rare ability to be so in tune with the singer that he can almost imperceptively vamp as they are searching for the words or having an emotional moment, and then pick up when they are ready. This contributed to the singer's feeling of safety while trying something new. It gave them room to explore acting choices, and they were shocked at how far we could take each performance in just a few minutes." As for working with singers one-on-one, Renae shared, "The singers were not expected to give professional performances, but to be made comfortable singing in front of others and to express themselves. They gave meaningful performances."

Renae learned what a day-to-day basis for someone with hemophilia can be like. "I learned these dear people have such pain, struggle, healthcare and insurance concerns in their lives that are understood by relatively few people. They thrive when they can be together with others who truly understand. I have never seen so many class clowns in one room before! They must laugh, make music and express themselves, or life will just be too painful." Each singer who Renae assisted stepped outside of their safety zone and embraced the challenges that were presented. "It turned out that a woman I worked with in the *Express Yourself Through Song* sessions lives only 15 minutes away from me in New Jersey.

continued on page 14...








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®, 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

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Coagulation Factor IX (Recombinant), GlycoPEGylated

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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 chorcid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

More detailed information is available upon request.

Available by prescription only.

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

Revised: 11/2017

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

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

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Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA

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...continued from page 11

I was able to sit with her in the hospital recently as her mother was dying. We sang the song she sang at The Beats Music Program but in a hospital hallway. I helped her with some of the aftermath and sang at her mother's funeral. I didn't know her last June, and now she is a dear friend who I was there for during a painful time."

Renae said, "I am grateful for the open spirits of the Coalition and that they share stories and experiences with people such as myself who had minimal knowledge. I am also grateful that they have each other on which to learn, to laugh, and make music and create."

Nashville, TN resident Tara Smith, an office manager at a pre-construction and investigative engineering firm is the wife of producer and songwriter Adam Smith. They became acquainted with the Coalition upon attending the 2018 Annual Symposium and meeting Coalition Vice President Kim Phelan. Calling her an inspiration that led to their collaboration, she said, "Kim is so gracious, her passion is very contagious, and she fiercely loves our community."

Tara co-wrote a song with her husband. She said, "I can see why people love to co-write and collaborate on music projects. It is very bonding and quite a great feeling to be a part of creating something." As a result of the program, Adam was able to build connections and collaborate with Michael, Colin, and Zach on a song titled *Silver Linings*, which they composed during the event. She explained, "They stayed an extra day after the event was over and came to our home to record with Adam. It is a beautiful song that focuses on the strength that comes from a community! It is a perfect reflection of our experience. I imagine there will be many more days spent in the studio together for these four!" She continued, "It was the coolest thing to see everyone share their thoughts on the song they were creating. Several people took turns recording instruments, and they were given the freedom to play whatever part they wanted. The creativity was magical, and the energy was positive and encouraging."

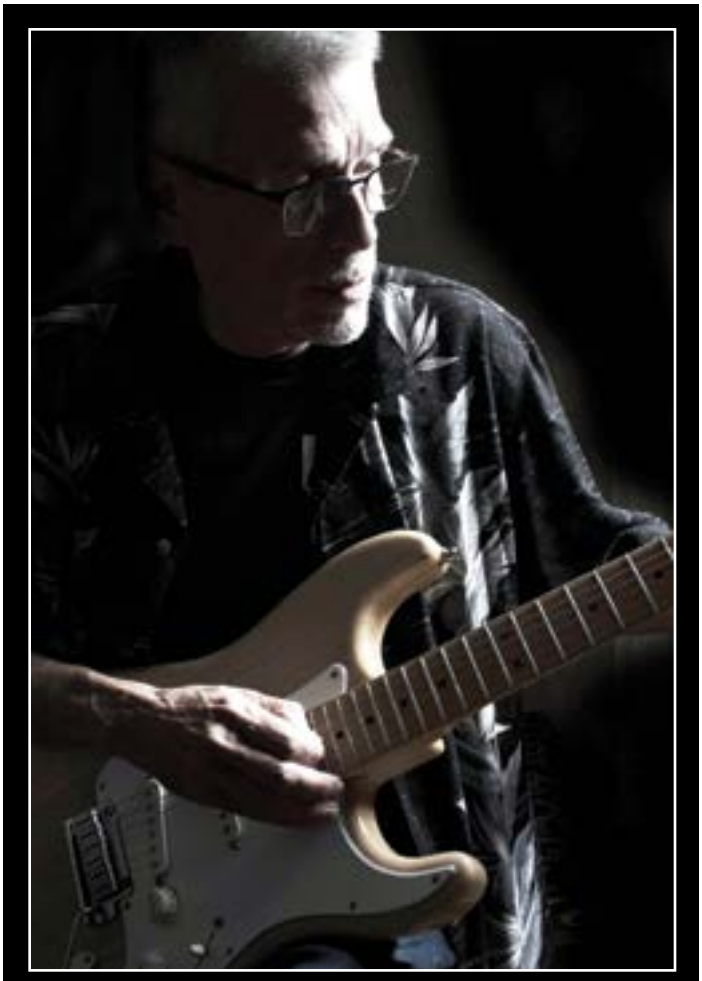
Tara and Adam's son, Arlo, was diagnosed with severe hemophilia B nearly two and a half years ago. She shared a story of coping.

"It was a total shock since there isn't a family history. It changes a person to see a baby being stuck with needles multiple times, trying to find a vein and doing this weekly. The challenges we have faced so far and the calm that we had to find made me realize I can handle a lot more than I thought. I can find peace in a tough moment, and it is easier for me to remember the larger picture and maintain perspective. Through events and the support and encouragement, I feel like I am becoming more confident in social situations and recognizing the value in connecting and the importance of pursuing community."

Tara takes pride in being a spectator. "Music is therapeutic since it shifts the focus off of yourself and forces your mind to go to a different place and escape for a while. Even though you might use your current struggle or past tough experiences to write a song, it is being used for a purpose, and you are choosing to embrace those struggles." She also feels that collaborating is therapeutic since the process of creating instantly fosters bonds. "It creates a level of vulnerability and openness that is so healing when shared. The Beats Music Program facilitated a safe and creative environment, which encouraged collaboration and sharing."

Tara is in it for the long run! "I would love to stay involved with this program and see it continue to inspire and connect people, as it can truly be a life-changing experience. With the talent and passion in the community, there are so many possibilities for songwriting and Artists & Repertoire. I am grateful for being supported and encouraged in our family's hemophilia journey, in a way that has empowered us to want to infuse hope into other people!"

"I am very proud of our organization and where we have come, and I plan to continue being the Coalition President for as long as I can," said Wayne Cook, who has held his position for over ten years, and is a lifetime mentor to the hemophilia B community. He resides in a small hamlet, south of Albany, NY, and retired from General Electric.



"I always try to stare my hemophilia right in the face and say 'Hey, you're not going to beat me," said Wayne who has faced many life challenges from multiple joint bleeds and joint replacements to the loss of jobs and insurances, and a long battle with hepatitis. Nevertheless, he explained, "I am still standing tall and showing the doctors that did my diagnosis, 'Hey, my life didn't end at 20 like you told my parents. I'm still here and am not going anyplace soon.' I still battle challenges not only with my hemophilia, but with my depression, but I try to make every day a good day and always try to reiterate why I'm here." What helps him by feeling blessed is his family. "Every day I am grateful to live to have a wife, children and grandchildren," he said.

Music is a platform that transcends him from the hustle and bustle of daily life. Wayne explained, "When I have days that my depression is acting up, I throw some music on, sit down, and start playing my drums to the music, or I work on my band's music. I feel The Beats Music Program will open doors for other individuals with hemophilia to express themselves when they are not into sports. It will be therapeutic for individuals dealing with depression and other chronic illnesses. It will be a way for individuals to get together and collaborate with music and have the camaraderie on a common ground, where no one passes judgment onto them."

Wayne found himself to be in awe not only of the program but the great speakers and fantastic teachers. "They worked hard to teach individuals to learn or hone their craft, and the attendees on how talented each individual is. They immediately bonded on what they all have in common. I relearned my love for music and playing with others who love it too."

Wayne witnessed growth through several stories. A friend of his who is a bass player had a desire to learn how to sing, perform solo, and not have a fear of failing, and he delivered a beautiful performance. Additionally, a young boy, who did not have a musical background, learned how to sing and perform on stage for the first time. As for a young man without musical experience who learned how to play drums, Wayne said, "He was so in tune with the lessons, that he was able to get up on stage with five other drummers and perform an awesome drum circle solo." Regarding a collaboration of three young musicians, he said, "In 24 hours, they wrote and performed a beautiful song consisting of a duet on guitar and another who sang. It was truly breathtaking!"

The Coalition for Hemophilia B has received unanimously positive feedback for implementing a program that reaches a more diverse audience in the hemophilia community. Kim Phelan Vice President of The Coalition for Hemophilia B explained, “We want to see this program reach everyone who wishes to express themselves through the love of the arts and to open it up to younger attendees. The success of the program has opened doorways as attendees are now participating in other events they normally would not attend. “We are very grateful to our sponsors Aptevo and CVS for their support of this program.”

Lindsay J. Goats, the event photographer and a Nashville resident, shared her perspective through the lens. “As a photographer, you hope one of your pictures will open someone’s eyes to another person’s pain. I see music being produced because of the Coalition, and I see self-confidence being born. It’s amazing! I hope that someone will see a portrait of mine and read about that person.”

Lindsay is very proud of how people with hemophilia live life to the fullest, even when they don’t feel well. She said, “It was great to realize the sense of community and support and the acceptance of those who are curious to learn about the conditions they have. They were very honest.” Among her favorite subjects were Shelby Smoak and Ramon Starks, Jr. “The stories of their lives within every inch of their face... it’s a photographers dream!”

Lindsay discovered a fascination with hands and loves snapping photos of them. She said, “There’s just as much of a story behind someone’s hands than the face. I had the privilege of reading Shelby Smoak’s book, *Bleeder*. He’s such an interesting person, so being able to match the face to the story was truly amazing.”

She recalls a story that Rick shared about his childhood after he and his brother were diagnosed with hemophilia. He had shared that at times their teachers acted like they were damaged goods and how it made them feel rejected.

After the four-day event, she felt she sometimes takes much for granted. She said, “These people push through bleeds, where every medical decision has to be weighed with extreme care. My faith in humanity was reborn while working this event,” said Lindsay. “There’s a lot of judgment in our world, but these folks who did not know me, welcomed me and shared some of their most intense emotions freely, and they didn’t care who heard it.”

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THE ONLY EXTENDED HALF-LIFE FACTOR IX THERAPY THAT DELIVERS

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Whether dosed every 7 or 14 days in clinical trials

UP TO **14** **DAY DOSING**† FDA APPROVED

Dosing schedules that fit your lifestyle

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

High and sustained steady-state FIX levels

*Zero median annualized spontaneous bleeding rate when dosed at 7 or 14 days in clinical trials.
†Once well controlled (1 month without spontaneous bleeding or requiring dose adjustments on a weekly dose of ≤40 IU/kg), people 12 years and older can be transitioned to 14-day dosing.
‡The average dose for people receiving prophylaxis every 7 days was 37 IU/kg and every 14 days was 73 IU/kg.
§Hemophilia FIX Market Assessment. Third-Party Market Research.

Is it time for a switch? Learn more at IDELVION.com

#1 FACTOR IX CHOICE WHEN CHANGING THERAPY§

Important Safety Information

IDELVION 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 number of bleeding episodes.

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

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

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

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

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Important Safety Information (cont'd)

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

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

In clinical trials for IDELVION, headache 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.

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 May 2018 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.

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

GENERATION IX PROJECT ADVOCACY PROGRAM IN PORTLAND, OREGON

From September 25–29, young leaders in the hemophilia B community gathered at Camp Collins in Portland, Oregon, for advocacy training. Camp Collins is a YMCA camp with a long history and heritage hosting youth recreational and education programs, and so it was a perfect locale for accommodating our innovative program.

Generation IX Project is a joint effort of The Coalition for Hemophilia B and GutMonkey, with sponsorship from Aptevo Therapeutics. GutMonkey, an experiential learning company, has worked closely with the hemophilia community for many years. Advocacy training was the latest program in the Generation IX Project, with other sessions concentrating on leadership and mentorship.

This year's advocacy training focused on the theme of *Repair*. Participants looked at the natural and inevitable occurrence of fractures in relationships and community, learning skills to mend these fractures through creative problem solving, innovative storytelling, highlighting our strengths, looking to others for support, and appreciating the transformative power of rest.

The group began each day with an invitation to learn how to make "fancy coffee" with a variety of tools and resources, led by Joe, the "in-house professional." This morning ritual gave everyone an opportunity to engage with a regular practice as a way to cultivate safety and self-care in our daily routines.

With warm, comforting beverages in hand, each day's agenda kicked off with a whole group circle discussion—a *Community Circle*. This was a time to reflect on individual and group experiences from the previous day, needs in the present moment, and hopes and desires for the day ahead.

Each day's program then continued with specific activities designed to reflect and expand on the theme of repair. On the first full day of the program, the group ventured into Portland to explore the Willamette River, which flows right through the center of the city. Partnering with a local dragon boat team, the Gen IX Advocacy cohort paddled up





and down the river, gaining incredible views of the city as well as many natural settings. The Wasabi Paddling Club, a group of adventurous women, some of whom are cancer survivors, served as incredible teachers and guides for this novel experience. Together, the paddlers explored the concepts of community, competition, and camaraderie.

Following the river adventure, the group visited the town's nonprofit Hollywood Theatre for a local film festival. Keeping with the repair theme, participants viewed various short films and learned different tools and techniques from these films on how to get messages across and tell stories in unique and compelling ways. This activity primed everyone for creating documentary storyboards focusing on bleeding disorders later in the program.

During another day's program, the group took the repair theme to another level through *Kintsugi*, the Japanese art of repairing broken pottery by mending the areas of breakage with lacquer dusted or mixed with powdered gold, silver, or platinum. This was facilitated by the incredible support of community member Kevin Finkle. Kintsugi treats breakage and repair as part of the history of an object, rather than something to disguise. The participants explored this metaphor as it related to our communities, relationships, bodies, and lives.

On the second to last night, the group was honored to be led in a restorative yoga practice by local therapist and teacher Megan Peterson. Using props such as blankets, bolsters, yoga blocks, and yoga straps, participants were guided into asanas (postures and poses) that allow the body to relax, rest, and repair.



Attendees also learned a lot from a variety of workshops led by members of the medical staff. Topics included the *Importance of Language, Infusion Skills, and Advocacy Scenarios with Practitioners*. All participants got to participate in each workshop with a small group of peers, with the medical staff bringing their expert knowledge and personal and professional skills.

The program also featured an excellent in-depth session on *Blood Product Safety and Insurance* led by Kim Isenberg, VP of Policy, Advocacy, and Government at the Hemophilia Federation of America.

Additional Gen IX programs will be coming in 2020, so please stay tuned and check our website, www.hemob.org, for more information. The Coalition for Hemophilia B is thankful to Aptevo for its generous sponsorship of this program.

Aptevo™ Therapeutics

ATTENDEE COMMENTS

(Submitted anonymously)

"I really enjoyed the experiential aspects and how they aligned to advocacy. The Dragon Boats were captained by Breast Cancer survivors — and there are no fiercer advocates than survivors... The film festival [taught us] various levels of advocacy from personal to governmental."

"I learned so much from discussion with others about what I can advocate for and how to do it."

"This program connects me to the larger community by forming strong interpersonal bonds with other people with hemophilia. That inspires a greater desire to speak out on my own and their behalf. I drew inspiration from the examples of advocacy and advocates featured throughout the week [and] was presented with new ways of thinking about problems and how to better work with various stakeholders to solve them.

FALL MEN'S RETREAT IN PHOENIX, ARIZONA

Men from around the country gathered September 19–22, 2019, for our much-awaited Coalition for Hemophilia B Fall Men's Retreat. The event was hosted at the scenic Arizona Grand Resort in Phoenix.

The Men's Retreats, which are held twice a year, are a time when men living with hemophilia B, as well as men who are caregivers, can come together in a safe space, learn, share experiences and feelings, and support each other in dealing with some of the challenges that life with this bleeding disorder presents.

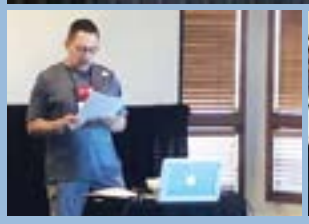
The weekend featured many powerful sessions and learning experiences. For example, several workshops addressed psychosocial issues that many men in the hemophilia B community deal with. One session, called *There Is No Health like Mental Health*, looked at the impact a bleeding disorder can have on mental health and offered techniques for dealing with these issues. The session was led by Debbie de la Riva, LPC. Debbie is a licensed professional counselor and a long-time leader in the bleeding disorders community.

In a session on stress management, Fernando Reyes, M. Ed. Psy, offered participants techniques for lowering stressful thoughts and nervousness. In addition, frequent Coalition presenter Robert Friedman led a session called *What's So Funny*, focusing on the physical, mental, and emotional benefits that humor brings and how anyone can achieve and incorporate these benefits in their daily life.

Participants were very engaged in a session called *It's Your Story: Pen It! Prep It! Perform It!* led by Natalie J. Sayer. The session offered dynamic techniques that community members can use to advocate for themselves and their families by telling their own powerful stories.

Several workshops gave attendees key information that can empower them in a variety of situations. There were workshops on talking to your children, led by hemophilia dad Dr. William Patsakos; a *Gene Therapy Update* led by Coalition for Hemophilia




Gene Therapy






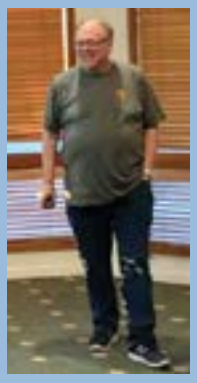












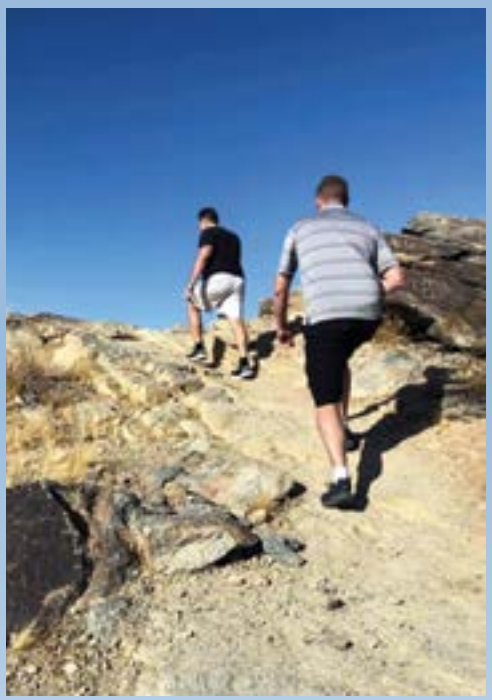



B Chair Dr. David Clark; and a session called *Getting and Keeping a Job with a Bleeding Disorder*, led by attorney and long-time Coalition adviser Donnie Akers.

In addition to programs, the men experienced powerful rap sessions, fun social gatherings, and other opportunities for the men in attendance to connect on a deep level, learning that they are not alone in dealing with the challenges of hemophilia and life. This was an opportunity to renew old friendships, make new ones, and return home with knowledge, strength and a sense of empowerment.

The Coalition for Hemophilia B would like to express our deep gratitude to Pfizer for sponsoring this important program. The benefits to the community members who attended are beyond calculation and none of it would have been possible without Pfizer's support. We would also like to thank the presenters, volunteers, and team members whose individual contributions helped make the event a great success. More programs are coming in 2020, so don't miss out. Check out website,hemob.org, for more information as it becomes available.





ATTENDEE COMMENTS

"As a dad to a child with hemophilia, I wasn't sure that I belonged at an event like this. With the friends I made and all the things I learned, I'm so thankful my wife encouraged me to attend. You all did an amazing job!"

"The men's retreat is an amazing program where we can have that time to forget our everyday problems and learn lots of good educational topics that prepare us to go back to our lives with a better attitude, feeling more empowered—and leave having met so many new friends."

"Great experience. Really enjoyed the time connecting with others and sharing stories/experiences. Thank you for putting this on and having me. I had a really good time."

"Outstanding event with pertinent learning and great connectivity."

"I didn't realize how much I would learn at this event!"

Aptevo BioTherapeutics LLC, Seattle, WA 98121

FALL WOMEN'S RETREAT IN ARIZONA

Hugs, laughs, tears of joy, and warm welcomes were in the air as women from around the country came together from September 25–29, 2019, for the Coalition for Hemophilia B's Fall Women's Retreat. The event was held at the Arizona Grand Resort in Phoenix.

By Chris Villarreal

The program was an opportunity for women in the community, including women living with a bleeding disorder and caregivers, to share a weekend of self-care, self-love, education, and advocacy that not only impacts the women themselves but also their families. Daily self-care began with a nature walk, aqua aerobics, or yoga.

The participants attended a variety of sessions that provided them with education and information they can use to deal with the challenges that can accompany hemophilia B. One session, led by attorney and long-time Coalition for Hemophilia B adviser Donnie Akers, focused on how to be the best patient advocate while navigating the emergency room, the doctor's office, and other medical settings. The discussion included the importance of Informed consent and a patient's rights in the doctor-patient relationship.

Several sessions looked at ways that women can become empowered, whether physically, mentally, or as advocates. Social worker Debbie de la Riva, LPC, led a session teaching the ladies about the importance of mental health and how to train the mind to work positively when dealing with tough mental challenges and situations. In another workshop, executive coach and leadership

speaker Natalie J. Sayer taught attendees how to relate their personal stories in ways that allow them to be powerful advocates for themselves and their families. The women in attendance also had the opportunity to learn from Nikita Lyons Murry, an Education Core Manager with Sanofi Genzyme. She spoke about the *Power of Empowerment*, helping the women to cultivate self-assurance, strength, confidence, and the ability to establish supportive partnerships with others.

During the Saturday sessions, the women tapped into their creative side with an *Art Therapy* workshop. During this workshop, they used different tools and supplies to decorate their own mandala to take home with them. Mandalas are intricate circular works of art with roots in Asian spiritual traditions. In this case, the goal was to create something that would remind each participant to follow their dreams, to catch a breath and never forget about their own self-care.

Keeping with the theme of sharing stories and feelings in ways that are deeply empowering, the attendees gathered for two special sessions. One, called *Seeing the Good in Others*, was led by Jenifer Fraker, and the other, called *Chit Chat and Chocolate*, was led by Deena Lipinski, Heidi Lynch, and Brittany Williams. In these sessions, the facilitators worked together to create a safe, nurturing space for the participants to share their stories and feelings. Part of one exercise involved the women using an app to send empowering words to another woman who was sharing her story. These words were later incorporated into personal framed picture souvenirs that





DREAM







EXIT



could be taken home as a reminder of what had been shared.

The retreat featured many opportunities for the women to gather in small groups, make new friends, and share strength and support. Everyone left feeling empowered, refreshed, and equipped with tools and connections of a lifetime.

If you have an interest in attending retreats in 2020, please check our website at hemob.org for more information and registration as it becomes available, or email farrahm@hemob.org

The Coalition for Hemophilia B would like to express our deep thanks to the event sponsor, Sanofi Genzyme. We would also like to thank the many presenters, volunteers, team members, and of course the participants for making this amazing program possible.

SANOFI GENZYME
Empowering Life

ATTENDEE COMMENTS

“I was so happy to be able to attend the retreat and finally meet other women within the Hemophilia B community. This was my first event with any Hemophilia organization, and I felt instantly connected. I can honestly say these women will be my friends for life. Every day, thinking back on their stories, I feel I can persevere through any obstacle that comes up in our life connected to Hemophilia.” — T. P.

“The retreat gave me a chance to be me: not wife, mom, or teacher—just me. It was an incredible experience. I came back refreshed and with two important things: knowledge to help me be a better wife, mom, and teacher, and new, amazing friendships. Thank you so much for supporting the women in our community.” — J. F.

“I learned so much! It was my first time attending and I was made to feel so welcome. Thank you to the Coalition team and all the wonderful women I met who openly shared from deep within their souls. I feel so grateful for this opportunity. This is such a powerful program.” — K. S.

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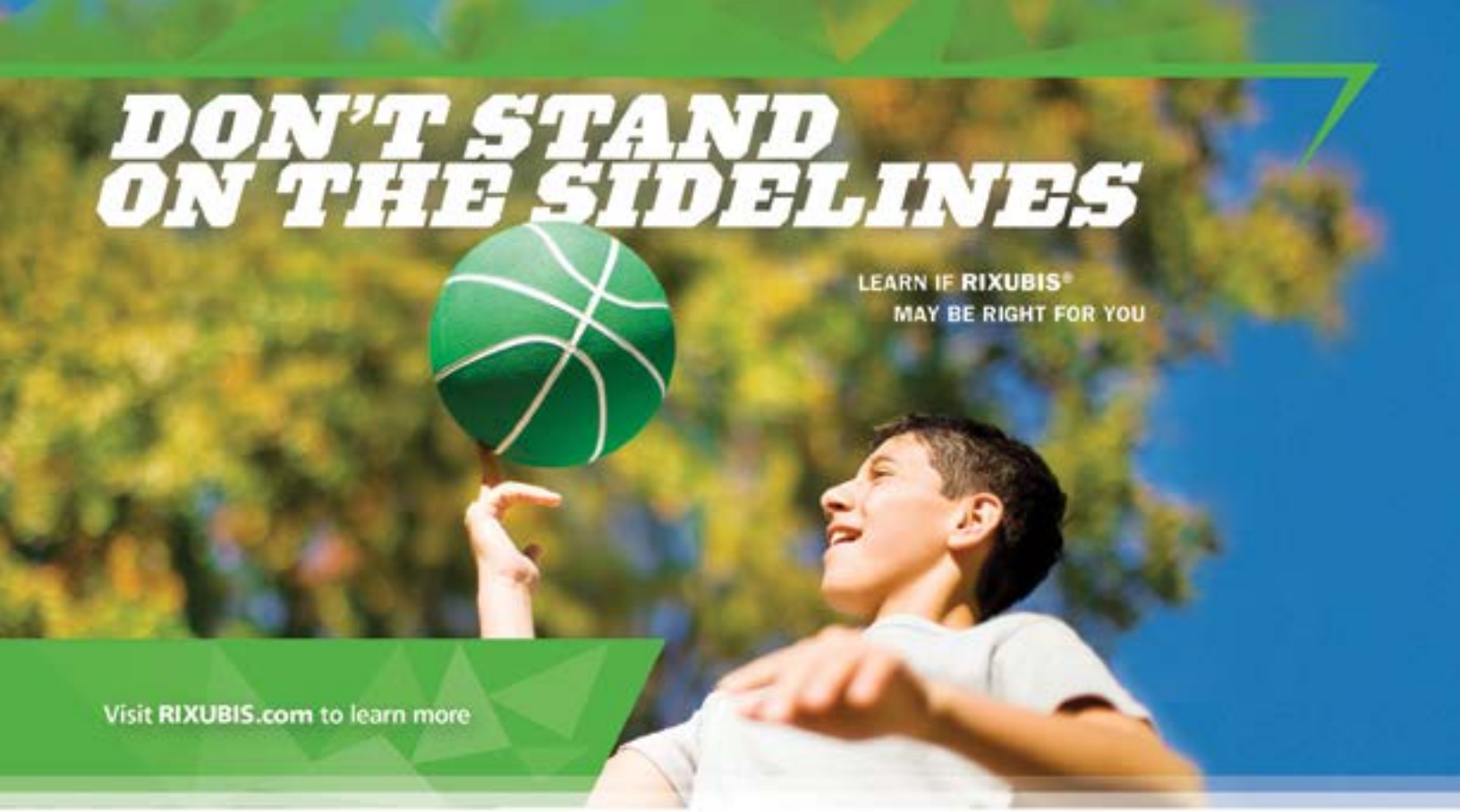

Visit **RIXUBIS.com** to learn more

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.

What else should I know about RIXUBIS?

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 talk to your healthcare provider.

Takeda

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

Important facts about RIXUBIS®:

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

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

What is RIXUBIS used for?

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

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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.

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Takeda

TREATMENT NEWS

By Dr. David Clark

Gene Therapy Firms Issue Ethics Guide

8/27/19 The Alliance for Regenerative Medicine (ARM), an industry group of gene therapy companies, has released a Statement of Principles setting forth a bioethics framework for gene editing. The statement includes an endorsement of gene editing in somatic cells (any cells of the body except reproductive cells) and a disapproval of editing in reproductive cells. They also support the use of standards and regulatory frameworks in the development of safe gene editing methods. According to ARM, "Gene editing technologies have not matured to the point where human trials of edited germline [reproductive] cells are appropriate. Many important safety, ethical, legal, and societal issues involved with this type of gene editing remain unresolved." The ARM statement was partly in response to the twin girls born in China who were the first babies modified by gene editing.
[Alliance for Regenerative Medicine press release 8/27/19]

Catalyst Reports on DalcA and MarzAA Progress

11/7/19 Catalyst Biosciences provided news on two of their products under development during their third-quarter corporate update. Dalcinonacog alfa (DalcA) is a variant factor IX with higher activity and half-life being developed as a subcutaneous treatment of hemophilia B. In their Phase IIb study, the two patients treated so far exceeded the efficacy endpoint (a goal for the study) of greater than 12% factor IX activity, with no evidence of inhibitor development. They expect to report the final study results in the first half of 2020.

Marzeptacog alfa (activated) (MarzAA) is a subcutaneous, activated factor VII variant for the treatment of hemophilia A and B patients with inhibitors. The final results of their Phase II study showed reductions in the average annual bleeding rate (ABR) from 19.8 to 1.6 while meeting all of the other secondary endpoints for safety, tolerability and lack of inhibitor formation. Seven of the nine subjects had no bleeds at their final dose level. They plan to start a Phase III study in 2020. Catalyst is also performing a Phase I study to further refine the pharmacokinetics and dose selection.
[Catalyst press release 11/7/19]

Catalyst Gene Therapy

8/1/19 Catalyst Biosciences has licensed AAV technology from Stanford University for gene therapy and is currently optimizing the AAV vector.
[Catalyst press release 8/1/19]

FDA Announces New Rare Disease Initiatives

10/22/19 At the National Organization for Rare Diseases (NORD) conference, Dr. Norman Sharpless, Acting FDA Commissioner, described FDA's new Rare Disease Cures Accelerator (RDCA) initiative, which has the goal to expedite the development of drugs to treat some of the 7000 rare diseases (including hemophilia) affecting 25,000 - 30,000 people in the U.S. They plan to establish more cooperative and collaborative approaches for research and development of treatments for rare diseases. Along with this, FDA plans to offer grant money to researchers studying rare diseases. They also hope to bring more patient voices and data into the field.
[FDA.gov; "Dr. Shapless's remarks to the 2019 NORD Conference on Rare Diseases"]

Freeline Announces Updated Data for Gene Therapy Patients

9/9/19 Freeline Therapeutics, a British gene therapy company, announced updated results for their hemophilia B gene therapy treatment, FLT180a. The first two patients, who were treated with a low dose of the product in the Phase I/II study showed steady state factor IX levels of 40.0 ± 5.5% over 66 and 74 weeks post-treatment. There were no infusion-related reactions and no evidence of liver inflammation.
[Freeline press release 9/9/19]

Factor X Variants Show Efficacy in Hemophilic Animals

Factor VIII (FVIII) and factor IX (FIX) work together in the clotting cascade to activate factor X. That is the reason that a deficiency of either FVIII or FIX results in similar symptoms in hemophilia A or B, respectively. If either FVIII or FIX is missing, the clotting process stops at the VIII/IX step and can't go further. Several groups are working on factor X products as potential treatments for hemophilia A and B. Activated factor X (FXa) would not need factors VIII or IX to produce clotting. However, FXa can be toxic because it is relatively uncontrolled in the clotting process and can produce serious thrombosis (too much clotting). FXa also has a very short half-life of only about 50 min.

The various groups working on FX are using two different approaches. One is to make a modified FXa molecule that has a lower activity and is thus more easily controlled. The other is to make a FX that can be activated by thrombin, a protein generated by several steps in the clotting process. That way, the FX can circulate in a safe, inactivated fashion until it is needed. Note that these products

should work for both hemophilia A and B and for patients with inhibitors. Two groups from France have recently published results for their FX products under development:

10/22/19 A group from French research institutes and the Nantes HTC reported on studies in a hemophilia A mouse model (mice deficient in factor VIII) with a thrombin-activatable FX. They found enhanced clotting compared to the control mice who received a placebo. They also measured clotting in the lab using plasma from hemophilia A and B patients, including an A with inhibitors. All of the samples showed improved clotting after being spiked with the modified FX.
[Muczynski et al., Thrombosis and Haemostasis, Epub ahead of print 10/22/19]

11/7/19 A group from LFB Biotechnologies, a French biotech company, is working on a thrombin-activatable factor X called Actiten. They have previously tried it in laboratory clotting assays with factor VIII (hemophilia A) deficient plasmas, some from inhibitor patients, and in factor X deficient plasmas. It was able to correct the hemostatic defect (the lack of clotting ability) in the plasmas. In the current report, they have shown that it normalized the clotting time in hemophilia A rabbits, that it was well-controlled by the anti-coagulant system, and that it produced no evidence of thrombogenicity (too much clotting).
[Abache et al., Haematologica, Epub ahead of print 11/7/19]

LogicBio Shows Durability of FIX Expression in Mice

10/23/19 LogicBio Therapeutics is developing modified AAV virus vectors for gene therapy treatment of pediatric patients with hemophilia B. Genes delivered to liver cells by their proprietary GeneRide™ vectors will integrate into the cells' genomes (the complete copy of all the body's genes that is in every cell), unlike most of the current methods that intentionally do not want their genes to integrate into the genome. Treating pediatric patients is an important goal because kids start to develop joint degradation at an early age.

When liver cells divide, each new cell has a complete copy of all of the genes that are in the genome on the chromosomes (structures in the nucleus of a cell that hold all of the genes) of the dividing cell. However, with most of the current gene therapy methods, the new gene remains as a stand-alone molecule in the cell nucleus, not on the chromosomes. Non-integrating genes are lost when a cell divides. That's not a big problem for adults whose livers are done growing, but in a pediatric patient, the new therapeutic gene would quickly be lost as their livers are growing rapidly by cell division.

The current use of non-integrating genes for gene therapy is a matter of caution and safety. Most of the early gene therapy studies used integrating genes, but the genes just inserted themselves randomly in the genome. They made no attempt to control where the new gene inserted itself, and in two patients (in non-hemophilia studies) the new genes inserted themselves in locations that inadvertently turned on cancer genes, giving the patients leukemia. After that, there was a moratorium on clinical (in-human) gene therapy studies until the process could be understood and controlled better.

After much research, studies gradually began again. Most of the new treatments use non-integrating vectors. That is a simpler answer but eliminates the possibility of treating younger patients. However, several organizations, including LogicBio, have decided to attack the problem more directly by using gene editing methods to integrate their new factor IX gene into the genome in a location where it won't cause trouble. So far, all of these companies are inserting their new factor IX gene into the albumin gene. Albumin is the most prevalent protein in the plasma. By putting the new gene under the control of the albumin gene promoter (the section of DNA that controls how much protein is made), it can produce a lot of factor IX, without the danger of triggering cancer or some other disorder.

LogicBio has shown that their method works in young mice. They have shown durable (lasting) expression of factor IX at levels of 50% of normal or greater with no serious side effects. These are promising results that could lead to future treatment of pediatric patients.
[LogicBio press release 10/16/19 and posters presented at the European Society of Gene and Cell Therapy Annual Congress, 10/23/19]

Mitsubishi Tanabe Enters Hemophilia B Gene Therapy Race

8/6/19 Mitsubishi Tanabe Pharma, a Japanese pharmaceutical company, is joining with Jichi Medical University to develop a gene therapy treatment for hemophilia B. They will use an adeno-associated virus (AAV) vector, but little else has been announced. The project is being funded by a grant from the Japan Agency for Medical Research and Development.
[Mitsubishi Tanabe Pharma press release 8/6/19]

NovoSeven Safety Study

9/18/19 A recent study shows that the rate of thrombotic events (unwanted clotting) for NovoSeven in the treatment of hemophilia A and B patients with inhibitors remains low, as was originally observed in clinical studies from 1999 to 2009. Only 21 thrombotic events have been observed in 18 patients treated for 12,288 bleeding and surgical episodes. Risk factors for thrombosis include simultaneous treatment with FEIBA and patients with a history of cardiac (heart) and cardiovascular (blood vessel) diseases.
[Rajpurkar et al., J. Blood Med., 10:335-340, 2019]

At Pfizer Hemophilia, we have always been deeply committed to you and to listening to what you have to say. Over the years, what you've shared with us has proven invaluable. The events we sponsor, the technology we develop, and the educational materials we create are all designed in response to the requests, needs, and desires of the hemophilia community.

We are grateful for having the chance to partner with you.

—Your Pfizer Hemophilia Team

TREATMENT NEWS

Novo's Concizumab Safe and Effective in Phase II Studies

8/23/19 Novo Nordisk is developing concizumab as an inhibitor of the anti-coagulant Tissue Factor Pathway Inhibitor (TFPI). This is one of several drugs being developed to restore clotting by tweaking the coagulation system. Anti-coagulants are the "brakes" on the clotting process that keep it under control so that the blood only clots when necessary. By reducing TFPI activity (inhibiting the inhibitor), concizumab "lets up on the brakes" a little to allow clotting, even in the absence of factors VIII and IX. This should work for both hemophilia A and B patients, with or without inhibitors (toward factor VIII or IX).

One Phase II study was in hemophilia A patients without inhibitors and one was in hemophilia A or B patients with inhibitors. Concizumab, given by daily subcutaneous injection, produced no severe adverse events or thromboembolic outcomes (too much clotting). Significant reductions in annualized bleeding rates (ABRs) were seen. Novo is continuing on to Phase III studies.

According to Dr. Amy Shapiro, one of the study leaders, "The most critical population for which exist unmet needs are the factor IX-deficient patients with inhibitors." [Shapiro et al., Blood, epub ahead of print 8/23/19]

Pfizer Reports on TFPI Inhibitor Marstacimab

7/23/19 Pfizer is developing marstacimab (formerly PF-06741086) as another inhibitor of TFPI - see above. Marstacimab is currently in clinical studies for the treatment of both hemophilia A and B. Pfizer published the results of laboratory studies looking at how marstacimab works in clotting assays (tests) in both normal and hemophilic blood and plasma (blood or plasma from a patient with hemophilia). They found that it worked as well as recombinant factor VIII or IX in the assays. Pfizer is currently recruiting for a long-term Phase II clinical study of the treatment.
[Patel-Hett et al., Haemophilia, Epub ahead of print 7/23/19]

Pfizer Announces Gene Therapy Manufacturing Expansion in North Carolina

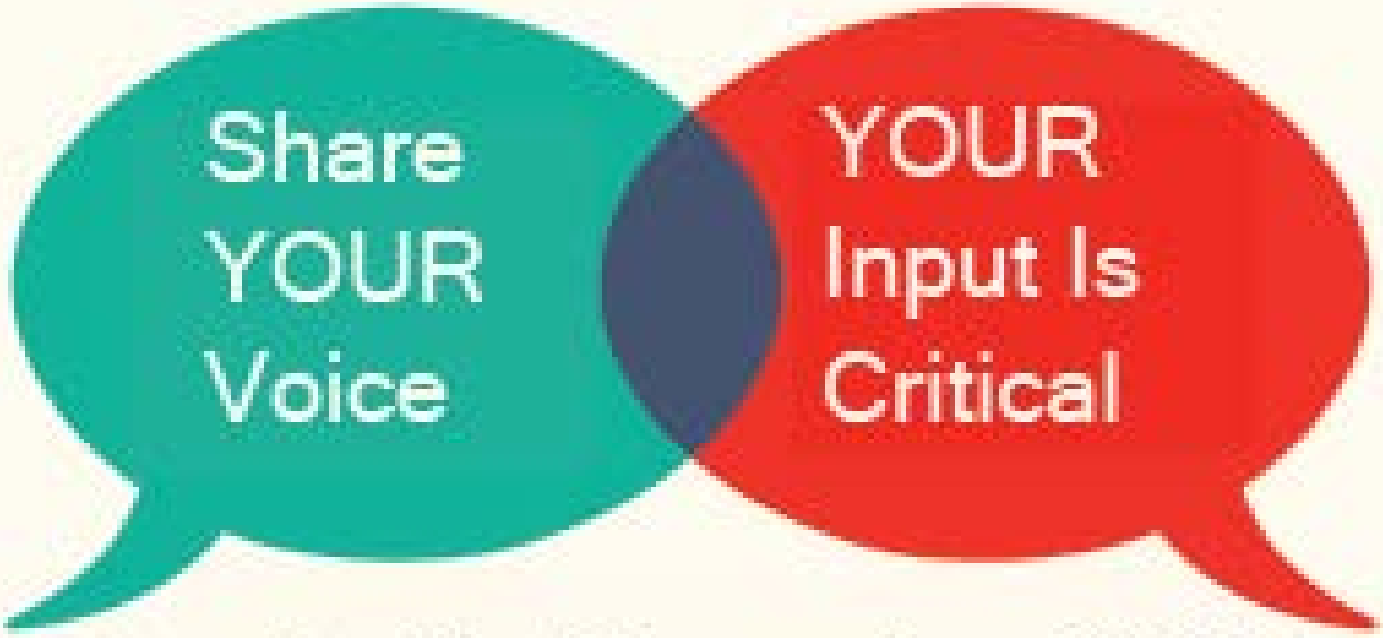
8/22/19 Pfizer acquired their Sanford, NC manufacturing facility in 2009 as part of their purchase of Wyeth Pharmaceuticals and BeneFIX. They poured $100 million into the facility in 2007 and have now announced that they will spend an additional $500 million on the facility to support gene therapy manufacturing. Pfizer currently has three clinical-stage gene therapy treatments under development plus ten preclinical projects. One of the clinical-stage programs is their gene therapy for hemophilia B, acquired from Spark Therapeutics.

Pfizer is currently concentrating most of its gene therapy operations in North Carolina. They have a small-scale development facility in Kite Creek, a medium-scale process development and optimization facility in Chapel Hill, as well as the large-scale manufacturing facility being built now in Sanford. Pfizer is investing heavily in gene therapy. In addition to the purchase of Spark's hemophilia B treatment, they bought Bamboo Therapeutics in 2016 and in March 2019 agreed to buy several gene therapies under development by Vivet Therapeutics. They are also licensing technology from Asklepios BioPharmaceutical (AskBio) for the Sanford plant. AskBio's Jude Samulski was one of the first developers of AAV gene therapy, dating back to 1978.
[Pfizer and AskBio press releases plus news articles from Reuters, Bloomberg and BioSpace]

Painkiller Being Resurrected for Hemophilic Arthropathy

10/9/19 Tremeau Pharmaceuticals is re-developing rofecoxib as a treatment for pain in hemophilic arthropathy (joint damage). Under the trade name Vioxx, rofecoxib was one of several COX-2 inhibitors that were blockbuster pain drugs until several were taken off the market in 2004. The discontinued drugs were found to roughly double the risk of heart attack and stroke in some patients. Vioxx's advantage was that it did not cause gastrointestinal bleeding. This was a blow to the hemophilia community because Vioxx was excellent at reducing joint pain.

Now that the mechanism by which rofecoxib caused the cardiovascular issues is well understood, Tremeau believes that it could be brought back. Its use would be limited to low-risk patients who would be closely monitored for side effects. Tremeau has announced that they are in active discussions with FDA and the hemophilia community and plan to start a Phase III clinical study of rofecoxib in 2020. [STAT News article, 10/9/19]



Coming in December 2019
Coalition Membership Survey

Why a Survey?

- Our last comprehensive survey was more than 5 years ago.
- New developments demand that we take stock of the experiences, needs, and concerns of the members we serve.
- Your responses provide useful information to support our efforts to improve or sustain the programs we offer you and assist with future program planning.

Bonus!

Patients completing the survey can ask to be entered into a separate drawing to attend our 2020 Symposium in March in Orlando and receive 4 tickets to Universal or a theme park of your choice. For those who complete the survey but are unable to travel, we'll have a second lotto drawing you can opt into and we'll gift you with something of similar value. After completing the survey, please email Farrahm@hemob.org with your name, phone, and email preference to be entered (your survey responses are anonymous and are not in any way tied to the bonus drawings).

All members will receive a survey in the mail.
Watch your mailboxes and inboxes! Survey is anonymous.

PLEASE TAKE THE SURVEY!

CHB December 2019 Survey

TREATMENT NEWS

uniQure Completes Enrollment in Gene Therapy Study

9/3/19 uniQure is developing a gene therapy treatment for hemophilia B called AMT-061, generic name etranacogene dezaparvovec. They are currently in Phase III clinical studies, the final phase before license application. They announced that they have now enrolled all 56 patients planned for the study, and because of high interest in the treatment, they will probably over-enroll with an additional six patients. All study participants are screened for pre-existing antibodies to the AAV5 vector, but no patients are being excluded because of pre-existing antibodies. Previous studies by uniQure suggest that the AAV5 vector may be viable for 97% of all severe and moderately severe patients. Subjects will be followed for five years after treatment. uniQure expects to provide preliminary results from the Phase III study by the end of 2020 and submit a BLA (Biologics License Application) to FDA in 2021. [uniQure press releases 9/3/19 and 10/28/19]

10/30/19 uniQure announced further interim results for the three patients treated in their Phase IIb study of AMT-061. At 26 weeks after treatment, the patients had factor IX levels of 33 - 57% of normal. The patients experienced no bleeds and none of the patients required infusions of factor products.
[Von Drygalski et al., Blood Advances, Epub ahead of print 10/30/19]

uniQure Announces Public Stock Offering

9/4/19 uniQure, a company developing a gene therapy for hemophilia B, announced that they have commenced a public offering of shares in their stock. They hope to raise approximately $225 million to support their ongoing product development activities.
[uniQure press release 9/4/19]

uniQure Announces Third Patent for Factor IX Padua Variant

11/5/19 uniQure announced the issuance of their third U.S. patent covering use of the Padua variant of factor IX. The Padua variant is a higher-activity mutation of factor IX that was discovered in a family in Padua, Italy. It and other variants with increased activity have shown promise in both factor replacement and gene therapy studies in progress. [uniQure press release 10/5/19]

RESEARCH UPDATE

By Dr. David Clark

Swedish Findings on Hypertension and Cardiovascular Disease

7/17/19 Because of their national healthcare system, all Swedish hemophilia patients (A's and B's) have been on prophylaxis for decades. Registries of their data provide a huge resource of information for studying the health effects of hemophilia and its treatment. A recently published report found that Swedish hemophilia patients have an increased incidence of hypertension (high blood pressure: HBP) compared to matched controls (people with similar gender and age but without hemophilia). The researchers found that 19.7% of hemophilia patients had been diagnosed with HBP, compared with 11.2% for the controls. Patients with hepatitis, but not HIV, were at a 3.3 times higher risk of HBP than the controls. This difference applied to both severe and mild or moderate patients. The study found similar rates of heart disease in patients and controls, but a lower percentage of deaths among the hemophilia patients. HBP is an important risk factor for stroke, but the authors did not look at that.
[Lövdahl et al., Thromb Res, 181, 106-111, 2019]

Coagulation Factors are Effective against Drug-Resistant Bacteria

8/9/19 Most things in the body, whether they are molecules or organs, have multiple functions. In addition to clotting, factor IX, for instance, appears to play a separate role in wound healing. Now a group of Chinese researchers have discovered that factors VII, IX and X also have antibacterial effects. These three factors are part of a larger group of clotting factors and anti-coagulants that have similar chemical structures. They are called vitamin K-dependent (VKD) proteins because they depend on vitamin K for their production in the body. When the three clotting factors are activated, they split into two sections, a heavy chain and a light chain (LC). The researchers found that the LCs have activity against Gram-negative bacteria, including several drug-resistant forms. The LCs cause the breakdown of the bacterial cell wall, destroying the bacteria. This previously unknown function is apparently part of the body's complex process to protect and heal itself after injury.
[Chen et al., Cell Res, Epub ahead of print 8/9/19]

Treatment for Patients with Allergies to Factor

8/22/19 Joe MacDonald and his wife Cazandra are the parents of two sons with hemophilia A. They are active in the hemophilia community and both write regularly for *Hemophilia News Today*. Their youngest son developed an allergy to factor VIII (FVIII), which meant that it couldn't be used to treat him. His is only the third reported instance of a FVIII allergy, although hemophilia B inhibitor patients often develop allergies to factor IX. Bypassing agents, which are used to treat inhibitors helped somewhat, but as Joe writes: "My family lived in fear, wondering if the hospital would always be our second home."

One day Joe and Cazandra thought: "Why can't we treat this as a peanut allergy?" Working with their hematologists and an allergist from National Jewish Health Hospital in Denver, they decided to try the drug Xolair, which is used to treat allergic asthma patients. After finally convincing their insurance company to approve their claims, their son was treated with both FVIII and Xolair for two years. This regimen eventually eliminated their son's allergy, and now five years after his first Xolair treatment he is no longer wheelchair-bound, plays basketball and rides his bike.

An approach like this could possibly help hemophilia B patients with allergies to factor IX. It is highly experimental and has only been tried this one time, but if you are in a similar position, it might be worth talking to your hematologist. A link to Joe's article is below.
[https://hemophilianewstoday.com/2019/08/22/allergy-factor-viii-xolair-omalizumab-leap-faith/]

FDA Safety Alert on Hepatitis C Treatments

8/28/19 FDA released a Safety Announcement on the use of Mavyret, Zepatier and Vosevi for treatment of patients with hepatitis C. In rare cases, treatment of patients with moderate to severe liver impairment has resulted in worsening liver function or liver failure. In most patients, the symptoms of worsening or failure have improved after stopping treatment with the drugs. All of the drugs have been widely used, safely and effectively, in patients with mild or no liver impairment.

Patients on these drugs should contact their doctor if they develop fatigue, weakness, loss of appetite, nausea and vomiting, yellow eyes or skin or light-colored stools. Do not stop taking the drugs without talking to your doctor, because that could allow your hepatitis C to come roaring back, if not further treated.

[https://www.fda.gov/drugs/drug-safety-and-availability/fda-warns-about-rare-occurrence-serious-liver-injury-use-hepatitis-c-medicines-mavyret-zepatier-and]

MASAC Recommends Liver Biopsies in Hemophilia Gene Therapy Studies

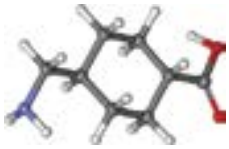
8/14/19 MASAC, the Medical and Scientific Advisory Committee of the National Hemophilia Foundation, issued a recommendation on the use of liver biopsies in clinical studies of gene therapy: MASAC Document #256. Because of the many unknowns in AAV targeting of the liver, MASAC recommends that developers collect serial liver biopsy samples from at least some of the patients undergoing treatment. "Serial" biopsies means taking periodic samples over the course of treatment to see what is really happening over time in the liver. Liver biopsy is an invasive procedure that has a potential for bleeding, but MASAC Document #233, which was issued in 2013, provides procedures and guidelines for safe liver biopsies in hemophilia patients.
[NHF web site: MASAC Document #256]

Factor Replacement Trials Must Include Women

9/3/19 Shellye Horowitz is a woman with hemophilia A who writes an excellent column for *Hemophilia News Today*. In a recent article, she addresses the pharmaceutical industry about the lack of clinical studies on women with hemophilia. She quotes a pharma rep as telling her, "We cannot easily conduct research on women with hemophilia as they do not bleed like men." To me (Dave), that's exactly why we need studies on women. If they bleed differently, we need to study more about that and learn the best ways to treat them. I've never understood the hesitation of companies to study women. You would think that they would be eager to look at a potentially huge expansion of their market.

I'm impressed and encouraged by Shellye and all the other carriers and women with hemophilia. They have been ignored for far too long, and I'm glad they are speaking out. We all need to support our blood sisters, many of whom are also our caretakers and family members.
[hemophilianewstoday.com, 9/3/19]

Benefit of Tranexamic Acid in Joint Replacement Surgery



9/4/19 As soon as a blood clot is formed, a set of proteins in the blood called the fibrinolytic system (fibrin is the protein that forms a clot and lysis means to break something down) starts breaking it down. This is part of the healing process to eventually eliminate the clot as new tissue forms to heal the injury. In a person without a bleeding disorder, the clotting system can easily keep up with the initial fibrinolytic activity, so a good solid clot is formed. However, in a person with hemophilia, the clotting process is weaker and can't keep up with fibrinolysis, so weak loose clots tend to be formed. Tranexamic acid (TXA) inhibits the fibrinolytic system to slow or stop breakdown of clots. TXA products on the U.S. market include Pfizer's Cyclokapron and Ferring's Lysteda. Akorn's Amicar is a similar anti-fibrinolytic drug that works the same way. These products can be used for both hemophilia A and B, as well as von Willebrand Disease (vWD). They are often used for dental procedures and can be used to treat bleeds in people with mild and moderate hemophilia.

A group of researchers from Sichuan University in China studied the use of TXA in joint replacement surgery in hemophilia A patients. They found that the use of TXA (in addition to the factor VIII normally used) in the perioperative setting ("peri" means around the time of: i.e., shortly before, during and after surgery) had a number of benefits. The study subjects had decreased blood loss, lower transfusion rates, lower factor VIII consumption, reduced swelling and joint pain, less inflammation and better joint function after surgery. Although the study was done on hemophilia A patients undergoing hip and knee replacements, it is likely that the results would also apply to hemophilia B and vWD patients and other joint types. More research is needed, but this is a very promising outcome.
[Huang et al, BMC Musculoskeletal Disorders, Epub ahead of print 9/4/19]

Number of People with Hemophilia Much Higher than Previous Estimates

9/10/19 There has always been a large variation in the estimated number of hemophilia patients in the world. A group of researchers in collaboration with the World Federation of Hemophilia (WFH) has published a new study of the prevalence of hemophilia. They looked at registry data from Australia, Canada, France, Italy, New Zealand and the United Kingdom. Since these countries all have government healthcare, the registries contain information on almost all of the patients in those countries. Also, patients in those countries are more likely to be identified and treated.

They found that the prevalence at birth for hemophilia B is 5.0 cases per 100,000 male births for all severities and 1.5 per 100,000 for severe hemophilia B. The prevalence in the general population is 3.8 per 100,000 for all severities and 1.1 per 100,000. (The respective numbers for hemophilia A are 24.6, 9.5, 17.1 and 6.0.) Note that they only looked at males. The difference between the prevalence at birth and prevalence in the general population represents mortality (deaths) in the population from all causes, not just hemophilia. From this they calculate a life-expectancy disadvantage, which is a measure of the reduction in life-expectancy compared to people without hemophilia. For hemophilia B, the disadvantage (in high-income countries like those studied) is 24% for all severities and 27% for severes. (30% and 37% for A) This suggests that even in developed countries, hemophilia care could be improved.

The authors estimate that there are actually about 1,125,000 people with hemophilia (A or B) in the world, 418,000 who are severe. This compares with 196,706

hemophilia patients who have previously been identified. It is thought that the large number of uncounted patients are mostly in under-developed countries, which demonstrates the huge need for identification and treatment worldwide.

The U.S. population is 329,446,037 as of September 11, 2019, according to the latest United Nations estimates. Based on that number there would be approximately 12,500 hemophilia B patients in the U.S., 3600 who are severe. That's about three times the 4000-patient estimate that we've been using previously. Again, this represents a large number of potentially untreated patients.
[Iorio et al., Annals of Internal Medicine, Epub ahead of print 9/10/19]

Interrelationship between Depression, Anxiety, Pain and Adherence in Hemophilia

9/20/19 In a survey of 200 hemophilia A and B patients, most reported symptoms of depression and/or anxiety, more than half of which had not been previously diagnosed. Depression and anxiety were linked, and patients with moderate-to-severe anxiety/depression were less likely to adhere to their prescribed clotting factor regimen. These patients also tended to have more uncontrolled pain and poorer social support (family/friends). The authors suggest that diagnosis and treatment of depression/anxiety may improve adherence and improve the level of pain control in patients with hemophilia.
[Witkop et al., Patient Preference and Adherance, 13:1577-1587, 2019]

Get More Sleep!

10/2/19 A recent study from Penn State indicates that patients with cardiac risk factors, including hypertension (high blood pressure) are more than twice as likely to die of heart disease or stroke if they routinely get less than six hours of sleep a night. That is important for hemophilia patients since they tend to have higher blood pressure than non-hemophilia patients. High blood pressure is a silent killer, and many hemophilia patients don't realize that they might be affected. So, get your blood pressure checked and get to bed!

Some people think that hemophilia protects them from heart attacks. While little is known about the risks, recent research suggests that hemophilia patients have just as many heart attacks as the general population, they just don't die from them as often. However, the damage caused by a non-fatal heart attack can still be substantial and can result in a significantly lower quality of life.
[Fernandez-Mendoza et al., Journal of the American Heart Association, Epub ahead of print 10/2/19]

How to Find Female-Sensitive Hemophilia Treatment

Duke University School of Medicine

10/8/19 Shellye Horowitz writes a column for *Hemophilia News Today* about her life as a woman with hemophilia. This week, she describes moving away from her HTC and the frustrations of trying to find care in a rural area. She gives a number of helpful tips based on her experiences.
[hemophilianewstoday.com, 10/18/19]

Humans May Be Able to Re-Grow Cartilage in Damaged Joints

10/9/19 It has generally been believed that once a joint is damaged, it can't repair itself. However, that assumption is now coming into question. First, there have been a few anecdotal accounts (personal stories, not formal scientific research) of hemophilia A patient's joints improving while on Hemlibra. Now, a study from Duke University School of Medicine shows that humans may have a hidden capacity to re-grow cartilage and tendons in their joints.

Animals like salamanders and zebrafish can actually re-grow whole limbs. Recent research has shown that these animals have a native regenerative ability that depends on microRNA (miRNA; short pieces of RNA genetic material that control genes). Using discarded human cartilage from joint-replacement surgery, the Duke researchers were able to determine that humans also have some capacity for cartilage re-growth that is controlled by miRNA. While the human capacity is limited, further research may find ways to enhance that capacity to heal joints, and even establish a method for re-growing whole limbs.
[Hsueh et al., Science Advances, 5(10):eaax3203, 2019]

Bone Defects Detected at Birth in Hemophilia Mouse Models

10/8/19 Joint damage is one of the primary issues in hemophilia, but less recognized problems include osteopenia (bone loss) and osteoporosis (low bone density). Using mice with hemophilia A or B or with von Willebrand Disease (vWD), an international team of researchers looked at the effect of the missing factors on bone density and integrity. They found that complete absence of factor VIII or IX resulted in congenital (present from birth) osteoporosis. The complete lack of von Willebrand Factor (vWF) conversely had no effect. Joint damage caused further bone damage, possibly due to changes during joint healing. Further study is needed to understand the mechanisms behind these findings.
[Taves et al., Scientific Reports, Epub ahead of print 10/8/19]

RESEARCH UPDATE

Less Polypharmacy in Older Hemophilia Patients

9/1/19 An article from Dr. P. M. Mannucci in Italy shows that older hemophilia patients have lower rates of polypharmacy (use of multiple prescription medicines) than age-matched patients without hemophilia. Older patients tend to need more medications, but that increases the risk of side effects and drug-drug interactions, which leads to more hospital admissions and poor treatment adherence. Dr. Mannucci believes that the lower rate of polypharmacy in older hemophilia patients may be due to the fact that they are often treated at HTCs, which tend to take a more overall view of their patients' health.
[Mannucci, Mediterr. J. Hematol. Infect. Dis., Epub ahead of print 9/1/19]

Novo Sponsors Study of Brain Development in Children with Hemophilia

10/8/19 Most of what is known about brain development in children with hemophilia comes from the *Hemophilia Growth and Development Study* (HGDS) that was published in 1994. The HGDS results indicated that boys with hemophilia had substantial brain dysfunction, lower intelligence, poorer academic and adaptive skills and more behavioral/emotional problems.

Since then there have been a number of changes in the population and treatments. Most children are now on prophylactic treatment, and there are fewer patients with co-morbidities like hepatitis C and AIDS. Therefore, Novo Nordisk is beginning the *Evolving Treatment of Hemophilia's Impact on Neurodevelopment, Intelligence, and Other Cognitive Functions* (eThink) study to update the results. They expect to enroll about 510 boys ages 1 to 21 with hemophilia A or B. The study will be conducted mainly through questionnaires completed by the patient or their parents/caregivers. If you or your child is interested in participating, you can find more information and contacts on clinicaltrials.gov under study NCT03660774, or from your hemophilia treatment center.
[Buranahirun et al., Pediatric Blood & Cancer, Epub ahead of print 10/8/19]

Dutch Study on Factors Influencing Switching Treatments

11/6/19 A recent Dutch study looked at the reasons patients and caregivers give for switching hemophilia treatment products. The researchers interviewed twelve patients and two caregivers covering all hemophilia types, including different ages, infection status (HIV and HCV), and even some with fear of needles The major factors were ease-of-use, cost, fear of the unknown and healthcare provider's advice. Most subjects were comfortable with their current treatment. Cost was a huge concern, with six subjects reporting skipping infusions to save money. Eight subjects were open to switching, but did not see the need at present. Three younger participants with few bleeding problems said they didn't feel the need to switch.

All of the patients expressed a general fear of the unknown and the desire not to be guinea pigs for newer less-tested products. There is also the fear that the new treatment might not work for them. One patient reported on two friends who enrolled in a gene therapy trial. One friend was pleased because the treatment worked for him, but the other was discouraged because his treatment did not work. Overall, the advice of their hemophilia treaters was considered very influential.
[van Balen et al., The Patient, Epub ahead of print 11/6/19]

Emotional Wellness as a Mature Adult:

Discussing the Unique Challenges of Living With Hemophilia

For more information, visit b2byourvoice.com to download *Learn from Experience: A Guide for Mature Adults.*

This content is brought to you by Pfizer.

How Hemophilia Affects Mature Adults

Mature adults may look back and recognize how living with hemophilia has influenced who they are today. Persevering through the challenges of being a child diagnosed with hemophilia when less was known about the condition, and navigating the issues of being a young adult with a bleeding condition can shape one's perspective. Knowledge and wisdom are some of the benefits that accrue with age, but along with these can also come additional health concerns such as high blood pressure, diabetes, and arthritis; depression and stress; and financial planning and retirement concerns. For those who have lived with hemophilia for many decades, the task of managing these concerns of older age may seem to be less important. However, there are some key points to keep in mind when addressing the effect hemophilia can have on mental health.

The Risk of Clinical Depression

Mature adults living with hemophilia typically have experienced substantial challenges related to their disease throughout their lives. In some instances, hardships may contribute to the development of clinical depression, which is more common among people living with hemophilia than the general population. The results from one study conducted at a hemophilia treatment center showed that 37% of a sample of patients met the criteria for depression. Of that 37%, 20% had moderate to severe symptoms, and 66% reported having functional impairment due to their depressive symptoms.[1] The authors of the study concluded that the comprehensive care of adults with hemophilia should include depression screening for the potential to improve overall health outcomes.[1]

Education and support for people living with bleeding disorders and their families is one component of managing psychological wellness. Having control over life decisions and self-advocacy can also be important. For some living with hemophilia, past experiences may serve as a motivator to continue to work toward personal objectives. Others may find the journey more difficult to navigate. Self-help seminars and support groups are some of the resources that may help adults set and attain realistic goals.

> "[A reminder to] older adults that there is always somewhere to turn, even in times of immense hardship. All you need to do is ask, and you should never feel ashamed for doing so."
>
> — Judy Bagato
>
> RN, BSN, Hemophilia Specialist

Finding Support for Complex Issues

For people who acquired human immunodeficiency virus (HIV) and/or hepatitis C (HCV) from virally contaminated blood products, there may be feelings of anger and resentment. The adversity caused by a lack of family or social support during younger years or changes later in life, such as changes in one's capacity for employment or altered family dynamics, may also contribute to these feelings. Learning effective ways to cope with the stresses of living with hemophilia in older age may help an individual to be resilient to these challenges. If you are experiencing stress that is affecting your day-to-day outlook, it is important to seek help. Reach out to your treatment team to discuss your situation and learn about what help and support may be available.

Reference: 1. Iannone M, Pennick L, Tom A, et al. Prevalence of depression in adults with haemophilia. *Haemophilia*. 2012;18:868-874. doi: 10.1111/j.1365-2516.2012.02863.x.

PATIENT AFFAIRS LIAISONS

Patient Affairs Liaisons are Pfizer employees who are dedicated solely to providing support to the community. Your Pfizer Patient Affairs Liaison is available to help you access the support and information you need. To find your Patient Affairs Liaison, go to hemophiliavillage.com/support/patient-affairs-liaison-finder or call Pfizer Hemophilia Connect at 1.844.989.HEMO (4366).

PP-HEM-USA-1237

September 2019

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THE COALITION FOR HEMOPHILIA B

ON THE ROAD AGAIN

Our members tell us on a regular basis that one of their favorite things about the Coalition for Hemophilia B is the broad selection of opportunities we provide for them to meet other community members, spend time together and learn in a safe, supportive environment. For many, the highlight of these programs is our Annual Symposium, which will take place next from Thursday, March 19 to Sunday, March 22, 2020, at the Renaissance Orlando at SeaWorld in Orlando, FL.

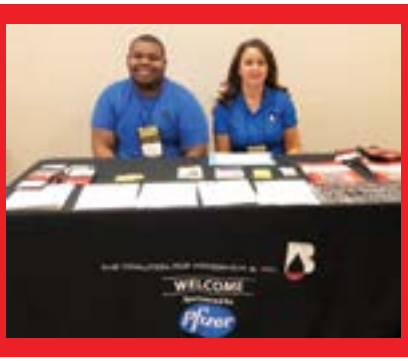
For more information or to register, visit: https://www.hemob.org/symposium2020).

However, not every family can travel that far or for that many days. That's why we also host a year-long series of programs called "Family Meetings On the Road." This series of one-day free family programs are held in cities across the country throughout the year, with a record 13 meetings held in 2019.

In our Summer edition of Factor Nine News (https://www.hemob.org/newsletter), we reported on the first series of meetings which took place in Minneapolis, MN, St. Louis, MO, Boston, MA, Dallas, TX, and Ann Arbor, MI. After a short break, meetings resumed in October, ultimately bringing the "On the Road MAGIC" to families in eight more cities.

Read on!

ON THE ROAD — COLUMBUS, OHIO — OCTOBER 19, 2019



Our final series of *On the Road Family Meetings* kicked off October 19 at the Columbus Marriott Northwest in Dublin, OH. The meeting featured a variety of informative sessions led by top professionals from around the country. The object is to equip participants with practical tools and information they can use to improve the care they provide to themselves and to family members.

In a session called *Learning from Experience: Living with Hemophilia B*, Dr. Michael F. Guerrera, Director of the Comprehensive Hemostasis and Thrombosis Program at Children's National Health System in Washington, D.C., provided an update on the findings of the *B-HERO-S Study* (*Bridging Hemophilia B Experiences, Results and Opportunities into Solutions*), conducted by Novo Nordisk. The study featured survey responses by 290 hemophilia B patients and 150 caregivers. One of the most significant findings was the fact that most respondents, including those with mild or moderate hemophilia B, report a negative impact on things like education, work life, and participation in recreational activities.

In another session called *Constructive Conversations*, participants learned and practiced techniques for maximizing the effectiveness of their communications with healthcare providers to achieve better outcomes. The workshop was led by Michael Sager, a Patient Affairs Liaison with Pfizer Rare Disease.

A highlight of every *Family Meeting* is a comprehensive update on the latest innovations in treatments for hemophilia B. Participants learn not only about improvements to clotting factor therapies but also about newer technologies like subcutaneous injections and gene therapy. In Columbus, the session was presented by David Cohenour, who teaches at Montana State University. David was a founding Board Member and Chair of the Rocky Mountain Hemophilia and Bleeding Disorders Association, and a former Board Member of the National Hemophilia Foundation.

On the Road meetings always include workshops that address the needs of the heart, mind and body. In Columbus, these included a session on the ancient Chinese martial art of Tai Chi, known for its slow, gentle movements that foster deep relaxation, increased strength and flexibility, and even lower blood pressure and decreased pain. The session in Columbus was led by Rick Starks, a man with severe hemophilia B who has studied and taught martial arts for more than forty years.

In a workshop about *Kinesiology and Joint Support*, participants learned how to apply Kinesio tape to assist in pain reduction in all joints. The workshop was conducted by Dr. Michael Zolotnitsky, PT, DPT, an orthopedic and neurological physical therapist with severe hemophilia A.

Perhaps most important were the many opportunities for families to spend time together, sharing stories, experiences and strength. Many families discover "the hemophilia B family next door" that they never knew about, and many make new lifelong friends.

Special thanks to Pfizer for their generous sponsorship of our Columbus Ohio event!

While one group of families gathered in Columbus, another group came together for the same purpose at the Holiday Inn World's Fair Park in Knoxville, TN. Highlights of the Knoxville meeting included several sessions focusing on parenting issues. In a workshop called *The Sky's the Limit: Encouraging your Children Responsibly*, participants empowered to encourage their children to "just be kids" while keeping them safe and healthy. The session was led by John Vieke, a man with hemophilia who, despite many obstacles and efforts by some to discourage him, fulfilled his dream of a career in law enforcement.

In another parent-oriented session called *Spotlight on Unaffected Siblings*, attendees discussed the impact a child with hemophilia may have on the lives of brothers or sisters who do not have a chronic condition. Participants were empowered with tools to help them ensure that the needs of all their children are being met. The session was led by Tanya Stephenson, a carrier of hemophilia who works as a Community Relations and Education Manager with Sanofi Genzyme.

Several workshops addressed topics similar to sessions offered in Columbus, with other presenters of course. The *B-HERO-S Survey* update was given by Cathy Tiggs-Johnson, MSSA, a medical social worker experienced with diverse patient populations. The *Kinesiology and Joint Support Workshop* was given by Dr. Douglas Stringham. Doug is an expert in sports medicine and physical rehabilitation.

Finally, the *Factor Nine Family Meeting and Hemophilia B Treatment Update* was led by Coalition for Hemophilia B Chair Dr. David Clark. Dave has a Ph.D. in chemical engineering from Cornell University and more than 35 years of experience doing scientific research in hemophilia.

We would like to express our great thanks to CSL Behring for their generous support of the Knoxville meeting.

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A week later, it was time to "hit the road" again as the Coalition for Hemophilia B came to Atlanta, GA and Seattle, WA. The Georgia meeting was held at the Atlanta Airport Marriott Gateway. John Vieke, community member speaking to parents in Knoxville, led a session this time called *Hiding Behind "Normal Opening Up About Your Diagnosis*. John shared his personal experience of deciding to be more open about his hemophilia, including with his coworkers in law enforcement.

At this meeting, the findings of the *B-HERO-S Survey* were again given by medical social worker Cathy Tiggs-Johnson. The popular *Factor Nine Family Meeting and Hemophilia B Treatment Update* were given by Dr. Lisa Hensley, who has a Master of Science in Public Health and a Ph.D. in Epidemiology. She is also a hemophilia B carrier and has a son with hemophilia B.

Rick Starks led his popular *Tai Chi Movements and Meditation* workshop, and the *Kinesiology and Joint Support Workshop* was again led by Dr. Douglas Stringham. As always, the meeting featured many opportunities for families to interact, make friends and share their strength.

Our gratitude to CSL Behring for providing the funding for the Atlanta meeting.

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On the same day, more hemophilia B families gathered in Seattle, WA for another *Family Meeting On the Road*. The event was hosted at the Hyatt Regency Lake Washington.

In a first-time session called *F.O.C.U.S: Strategies for Keeping a Positive Perspective*, parent advocate Shonda Joshua shared strategies she has developed as a hemophilia mom for maintaining a good attitude while successfully parenting a child with a bleeding disorder.

The *B-HERO-S Study* update was given by Penni Smith, RN, an RN Case Manager, at the Intermountain Hemophilia and Thrombosis Center in Salt Lake City, UT and a board member at the Utah Hemophilia Foundation. The *Hemophilia B Treatment Update* was again given by Coalition Chair Dr. David Clark.

A session called *Mindful Movement* was led by Chris Brain Maloney, founder of yoga and meditation studio Stressless in Seattle. The techniques she shared in the workshop represented a fusion of traditional yoga poses and contemporary movement designed to release tightness and tension from the body. Participants also had the opportunity to learn about the benefits of *Kinesiology Taping* with Dr. Michael Zolotnitsky.

Many thanks to CSL Behring for sponsoring the Seattle meeting!

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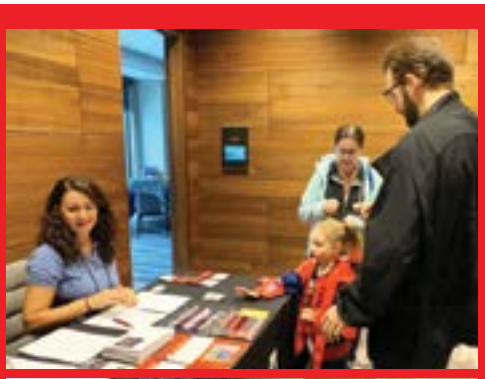











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ON THE ROAD BATON ROUGE, LOUISIANA NOVEMBER 2, 2019

On November 2, the Coalition headed to the heart of Cajun country for our next meeting in Baton Rouge, LA. The meeting was held at the Renaissance Baton Rouge Hotel.

In a session called *Be the Best Patient Advocate*, attorney and long-time Coalition Advisor Donnie Akers taught participants about navigating the ER, medical and clinical trial worlds. The presentation placed an emphasis on the legal requirement of *Informed Consent* and your rights in the doctor-patient relationship, including the right to refuse treatment.

At this meeting, the *B-HERO-S Survey* findings were presented by Angela Forsyth, PT, DPT, a physical therapist with an extensive background in hemophilia, including at the Penn Hemophilia and Thrombosis Center. The *Hemophilia B Treatment Update* was given by Dr. Lisa Hensley, hemophilia mom Shonda Joshua gave her *Positive Parenting* talk, Rick Starks led his popular *TaiChi Movements and Meditation* workshop, and Dr. Douglas Stringham led the *Kinesiology and Joint Support Workshop*.

In a session called *What's So Funny*, psychotherapist, author, professional speaker and musician Robert Lawrence Friedman, MA focused on the physical, mental and emotional benefits that humor brings, and showed participants how to incorporate these benefits in their daily lives.

Our deep gratitude to CSL Behring for their support of the Baton Rouge meeting.

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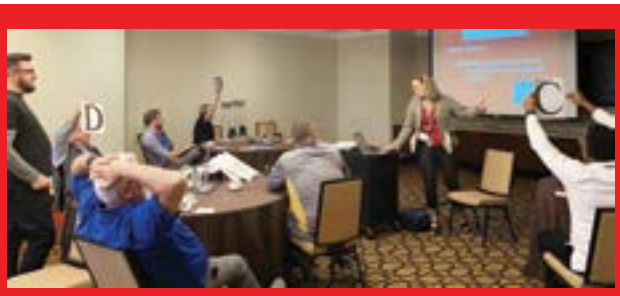









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On the same day, more families from the community came together at the DoubleTree by Hilton Fort Smith City Center for our Arkansas *On the Road*.

Arkansas attendees were treated to a workshop led by educator Ben Shuldiner. Ben was the founder and principal of the High School for Public Service in Brooklyn, NY. Currently, Ben is the Distinguished Lecturer of Education Leadership and Program Coordinator of the School and District Leadership Program at Hunter College in New York City. Ben also has hemophilia B. Ben's workshop was called *Advocating for Your Child: 504 Plans and IEPs*. The presentation offered ways for students and families affected by bleeding disorders to receive needed accommodations at school. The discussion included an explanation of the laws that guarantee and protect the right to these accommodations.

In another session new for Arkansas, Jeffery McFall led a discussion called *Empowered: Tools for Self-Advocacy*. The workshop highlighted some of the unique challenges of life with hemophilia and provided practical tips and resources to become a self-advocate in all areas of life. Jeff is a Patient Affairs Liaison at Pfizer with more than thirty years of healthcare experience.

At the Arkansas *On the Road*, the *Factor Nine Family Meeting* featuring the topic *What's New in Hemophilia B* was once again led by Coalition Chair Dr. David Clark.

Physical therapist Dr. Michael Zolotnitsky joined us again to lead his *Kinesiology and Joint Support Workshop*. Michael also co-led a workshop called *Using Fitness to Fight Back*, joined by Derek Houser. Derek is a man with hemophilia b who is very involved in athletics and physical fitness. The workshop explored how physical fitness can help defend against injury for people with hemophilia.

The Coalition for Hemophilia B is grateful to Pfizer for their generous support of the Arkansas meeting.


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ON THE ROAD PHOENIX, ARIZONA NOVEMBER 9, 2019

On November 9th, families came together in Phoenix, AZ, and Schaumburg, IL for the last two *On the Road Family Meetings* of the year. The Arizona meeting was held at the Sheraton Phoenix Hotel Tempe.

A new face in Phoenix was Grace Hernandez who presented the findings of the *B-HERO-S Survey*. Grace is a physical therapist at the Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders in Orange, CA.

Yet another new face for this season belonged to Myles Ganley, a community member with hemophilia B who has overcome multiple obstacles and challenges to live his fullest life. Myles gave a talk on the topic *Using Fitness to Fight Back*. Participants learned how to safely utilize fitness components like flexibility, core stability, strength training, and body movements to help prepare for a healthy, active lifestyle.

Returning speakers included hemophilia mom Shonda Joshua on *Strategies for Keeping a Positive Perspective*, Dr. Douglas Stringham on *Kinesiology Taping and Joint Support*, and Robert Lawrence Friedman on *What's So Funny?* The *Family Meeting and Hemophilia B Update* was led by Coalition Chair Dr. David Clark.

Many thanks to CSL Behring for their sponsorship of the Phoenix meeting!

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ON THE ROAD — SCHAUMBURG, ILLINOIS — NOVEMBER 9, 2019




On the same day – and last, but not least – a final group of families gathered In Schaumburg, IL to close out our wonderful series of *Family Meetings on the Road* for the year. The meeting was held at the Chicago Marriott Schaumburg.

New for the series but certainly not to the community, the meeting featured a rap session led by long-time community member Rocky Williams. The session was an open forum for participants to discuss timely issues that are going on in their lives and within the community.

Also new for this season, the *B-Hero-S Survey* results were presented by Dr. Sandeep Rajang. Dr. Rajan is the medical director of the Hemophilia and Thrombosis Treatment Center in East Memphis, TN.

Presenters returning to popular topics from other cities included Rick Starks on *TaiChi Movements and Meditation* and Dr. Michael Zolotnitsky on *Kinesiology Taping and Joint Support.* The *Factor Nine Family Meeting and Hemophilia B Update* was given by Dr. Lisa Hensley.

The Coalition for Hemophilia B would like to express our deep gratitude to Novo Nordisk for sponsoring the Schaumburg meeting.

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THANK YOU...

We are thankful for the many speakers, volunteers, staff members and attendees who made this incredible program series possible.

Although our *Meetings on the Road* are completed for 2019, there are many more coming in 2020 so check our website www.hemob.org for more information. Please remember that our Annual Symposium will take place Thursday, March 19 to Sunday, March 22, 2020 in Orlando, FL. (For more information or to register, visit https://www.hemob.org/symposium2020.



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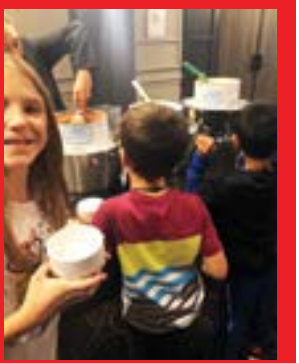




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2020 SCHOLARSHIP APPLICATION PROCESS NOW OPEN!

THE WILLIAM N. DROHAN SCHOLARSHIP

FOR STUDENTS WITH HEMOPHILIA B AND THEIR SIBLINGS

DEADLINE FEBRUARY 20, 2020

The 2020 application for the William N. Drohan Scholarship is now available!

The William N. Drohan Scholarship was created in memory of Dr. William N. Drohan who passed away in 2007. Dr. Drohan was a well-known microbiologist and educator who will long be remembered for his many contributions to science. He was a pioneer in using molecular biology to produce recombinant proteins and a visionary scientist who dedicated his life to improving the safety of blood and blood products.

This scholarship is for college and graduate students with hemophilia B and their siblings. Please visit our website www.hemob.org to download the application. The application includes eligibility information and instructions. Completed applications must be received by end of business day February 20, 2020 to be considered.

For questions, please contact Kim Phelan (212) 520-8272.

The Coalition for Hemophilia B

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