



Factor Nine News

The Coalition for Hemophilia B

SPRING 2019



Topics in Hemophilia B

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THE HEMOPHILIA B COMMUNITY GATHERS FOR THREE DAYS OF LEARNING, LAUGHTER, AND LOVE

By Glenn Mones



From March 14–17, the Renaissance Orlando at SeaWorld played host to one of the largest gatherings of families and individuals affected by hemophilia B—the Coalition for Hemophilia B 2019 Annual Symposium. Attendees participated in nearly 40 educational sessions—more than we’ve ever held at our national annual meeting before—with many opportunities to share their own experiences and provide each other with mutual support.



Our Coalition members had the opportunity to hear the latest updates on treatment from some of the country’s top clinicians. Dr. Christopher Walsh, who heads the Department of Medicine, Hematology, at Mount Sinai Hospital in New York City, gave the keynote address, which focused on the latest available treatments as well as therapies in the pipeline. With so many new drugs available to people affected by hemophilia B, this topic was of great interest to all the participants who may be considering their own treatment options.

Dr. Robert Sidonio, associate director of the Hemostasis and Thrombosis Program at Children’s Healthcare of Atlanta and director of

Clinical Operations and Clinical Research, shared new insights from research on the many biological indicators other than factor levels that influence bleeding. These gleanings are helping to produce novel therapies beyond clotting factor alone that may reduce or eliminate bleeding episodes.

One of the major highlights of the symposium was the screening of the film *Bombardier Blood*, which documents the amazing journey of community member Chris Bombardier to the summit of Mount Everest. Chris is a person with hemophilia B who did not let his medical condition interfere with his life’s passion. The film tells the story of how Chris conquered not



only Everest but all “seven summits,” the highest peaks on seven continents, infusing all the way. Chris was present for the screening and made himself available for a Q&A. The screening drew a capacity crowd of symposium participants who were visibly moved and inspired by the presentation.

The program featured many sessions dedicated to specific audiences including women, men, teens, and kids. These sessions provided “safe spaces” for sharing support and personal experiences in ways that might have been more difficult in larger “mixed groups.” Other sessions focused on a variety of important topics including mental health, advocacy, maintaining access to affordable healthcare, and employment issues.





Women with hemophilia
"A woman who has less than 40 percent of the normal level of clotting factor is no different from a man with the same factor levels – she has hemophilia."





Beyond the incredible amount of learning that took place, the symposium offered lighthearted fun, too. One highlight was the Thursday night performance by the Bleeders, a band made up entirely of community members and featuring Coalition for Hemophilia B President Wayne Cook on drums. The band, performing covers of some of the greatest hits of this and earlier generations, had the crowd rocking and rolling all night.

and new friendships made, and on being newly empowered to deal with hemophilia with strength, courage, and conviction.

The Coalition for Hemophilia B wishes to express our gratitude to the many sponsors, exhibitors, speakers, volunteers, and staff team members who made the symposium possible. Please join us for our *Family Meetings* throughout the year and stay tuned for more information on our 2020 Symposium in Orlando!

By the time the symposium ended on Sunday, participants shared feedback on how much they learned, on old friendships renewed







To view more photos of Coalition events, please visit our Facebook page!
www.facebook.com/HemophiliaB/







A SPECIAL THANK YOU TO OUR 2019 SYMPOSIUM SPONSORS!

DIAMOND LEVEL



GOLD

SILVER

BRONZE



FRIENDS & EXHIBITORS





THE COALITION FOR
HEMOPHILIA B

12th ANNUAL
ETERNAL SPIRIT AWARD
GALA

*A Night of
Inspiration*

FEBRUARY 28, 2019





Members of the Hemophilia B community, family and friends gathered February 28th for the 12th Annual Eternal Spirit Award Gala.

The festive evening was held at Terrace on the Park in Flushing Meadow Park, NY. The beautiful venue, located on the site of the 1939 and 1964 World's Fairs, has breathtaking views of the surrounding area including the famed Unisphere globe.

The highlight of the evening was the presentation of the Eternal Spirit Award to Glenn Pierce, MD, PhD and Hope Woodcock-Ross, RN, BSN for their many years of service and dedication to the Hemophilia Community.

The event benefits the Dr. William N. Drohan Scholarship Fund. Bill Drohan, a well-known microbiologist and educator, lost his battle with metastatic lung cancer at the age of 60. 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. He also served as a member of the board of the Coalition for Hemophilia B and was instrumental in its creation.





The Coalition for Hemophilia B established the William N. Drohan fund to offer scholarships to children with hemophilia B and their siblings. Applicants must have applied to, or been accepted at, an accredited college or university and have need for financial assistance. Each year we award four (4) or more scholarships to students with hemophilia B or their siblings. In the past 12 years, \$220,000 was given. Two of our 2019 Scholarship award recipients were present this evening William Patsakos, Jr. and Jibin Jonhs. Additional awardees are Nitya Bhattarai and Alecia Sclafani. Congratulations to all!

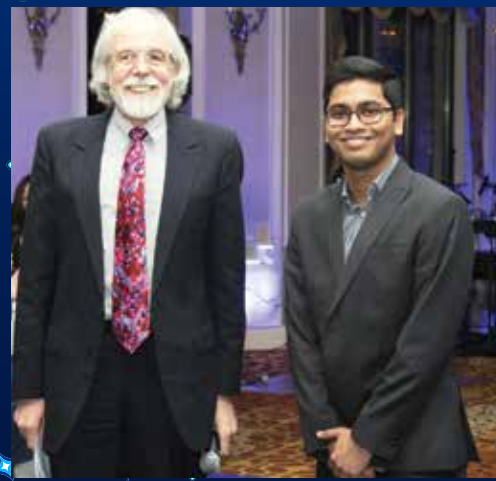


The evening also benefited our B Cares Emergency Family Assistance Fund, an important safety net helping affected families in crisis, and the B Voice Advocacy Program, ensuring that people affected by hemophilia B have access to affordable, high quality healthcare.



Attendees enjoyed cocktails and dinner, live music and entertainment, raffles, and a wonderful evening benefitting and celebrating our community. The Coalition for Hemophilia B is grateful to the many sponsors, financial donors, raffle prize donors, participants, honorees, staff members, volunteers and others who helped make the evening a great success. For more information about this and other programs, please visit www.hemob.org.





To view more photos of Coalition events, please visit our Facebook page!
www.facebook.com/HemophiliaB/





BeneFix

Coagulation Factor IX (Recombinant)

Room Temperature Storage

*BeneFix was approved February 11, 1997.

Not actual patients.

EXPERIENCE MATTERS

BeneFix: The only recombinant factor IX supporting individuals with hemophilia for more than 20 years.*

More than 20 years* of experience—you've been at the heart of all we do

- The first recombinant treatment indicated for bleed control and prevention in individuals with hemophilia B
- Designed with viral safety in mind
- More than 150 quality control tests are done on each batch of BeneFix
- The flexibility to infuse on demand or preventively based on your physical activity
- The convenience of the BeneFix Rapid Reconstitution (R2) Kit with a range of vial sizes



What Is BeneFix?

BeneFix, Coagulation Factor IX [Recombinant], is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

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

Important Safety Information

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash or hives.

Visit BeneFix.com to learn more

- Your body can make antibodies, called "inhibitors," which may stop BeneFix from working properly.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are nausea, injection site reaction, injection site pain, headache, dizziness and rash.

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

Please see the Brief Summary for BeneFix on the next page.



R_xonly

Brief Summary

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

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

What is BeneFix?

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

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

What should I tell my doctor before using BeneFix?

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

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

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

How should I infuse BeneFix?

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

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

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

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

Your doctor will prescribe the dose that you should take. Your doctor may need to test your blood from time to time. BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

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

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

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

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

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

These are not all the possible side effects of BeneFix.

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

How should I store BeneFix?

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

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

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

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

What else should I know about BeneFix?

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

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

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-10.0, revised June 2017.



The Coalition for Hemophilia B

LET'S PLAY NINE Golf Fundraiser



A DAY OF GOLF, A DAY OF GOOD!

On Wednesday, March 13, the celebrated Copperhead Course at the beautiful Innisbrook Resort in Tampa played host to our Third Annual *Let's Play Nine* golf outing. *Let's Play Nine* was formed to give young people in the hemophilia B community a chance to participate in a sport that can safely become a lifelong hobby and passion. The fundamentals of golf foster flexibility and core strength while minimizing shock and impact. Working with PGA professionals, we show young golfers the basics of the game and teach them the golf swing in a safe manner to prevent joint damage. *Let's Play Nine* also raises funds to provide young golfers with additional instruction and equipment. Proceeds also support our *B Cares* emergency assistance fund.

Participants had the rare opportunity to play on a championship golf course that six days later would host the PGA Tour. The course was in tour condition, from the manicured fairways and stadium seating to the greens running around 12 on the Stimpmeter.



The day started with a buffet breakfast and driving-range clinic hosted by the community's very own championship player, Perry Parker. Participants also enjoyed a short game clinic that included chipping, pitching, and putting, hosted by the staff at Copperhead. Our amazing day of golf was topped off with raffles, auctions, and special awards for games such as longest drive and closest to pin.

The Coalition for Hemophilia B is grateful to our event sponsors, including Aptevo, CSL Behring, CVS Specialty Pharmacy, and Novo Nordisk. We are also grateful to our staff and volunteers, and to our Let's Play Nine committee including Jim and Becky Vansant, Wayne Cook, Hope Woodcock-Ross and Jim Ross, Chad Brown, Debbi Adamkin, and Shad Tulledge.



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Gold Level



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IXINITY[®]
coagulation factor IX
(recombinant)



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GEN IX LEADERSHIP

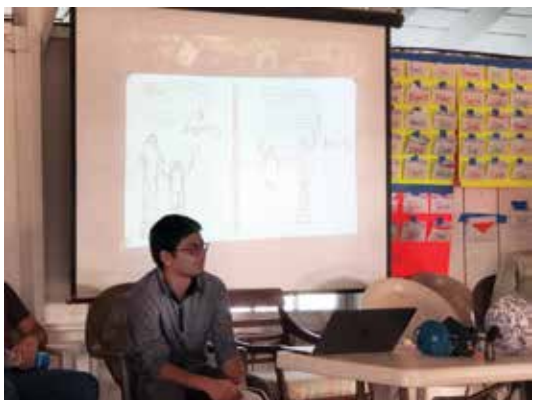
From January 9-12, historic Pigeon Key off the southernmost coast of Florida, played host to a leadership training retreat for young adults with hemophilia B. The program was the latest in the Generation IX Project. Earlier sessions focused on mentorship and advocacy.

The Pigeon Key program was designed to identify, bring together and strengthen young adult leaders and potential leaders within the hemophilia B community. The workshop focused specifically on

helping participants understand and incorporate the idea of inclusive leadership. The essentials of this style of leadership are that all voices are included in the decision-making process, all needs are considered,

and the system provides both equity and equality to all participants.

This concept was communicated though a variety of interactive sessions in which the participants





IN THE FLORIDA KEYS

Generation IX Project is a joint effort of the Coalition for Hemophilia B and Gutmonkey, with sponsorship from Aptevo Therapeutics. Gutmonkey is an experiential learning company with a long history of working with the hemophilia community.

were asked to use the knowledge and tools they were acquiring to develop solutions to real community problems.

An important feature of each day's program was a session in which two to

three participants had the opportunity to share their personal challenges and stories. One of the goals was to give the group a chance to practice open, honest communication and go outside their personal "comfort zones" in a

safe, supportive environment. Participants also had the opportunity to engage in personal physical challenges with options that included swimming and snorkeling in the beautiful natural setting of the Keys.





HERE'S WHAT A FEW OF THE PARTICIPANTS HAD TO SAY ABOUT THE PROGRAM:

“This was my first year attending the leadership GenIX program in Pigeon Key, Florida. It was an absolutely AMAZING experience! During the week we were all able to grow close to one another and to continue to grow the hemophilia B community. The opportunity to meet and talk to fellow hemophiliacs is more valuable than I can even say. Spending a week with these people really changes how you view your diagnosis and, honestly, how you view the world. Being able to spend a week on an island together really brings out the best version of yourself and you're able to carry it back into the world. In addition to that, the leadership programming itself is extremely applicable to life and helped form me into a better person and leader. I wouldn't trade the opportunity to attend Gen IX for anything.” — C.S.

“As a 35-year-old man with severe hemophilia B, and father to a six-year-

old daughter with mild hemophilia, I am thankful for the Coalition for Hemophilia B sponsoring my attendance at the Gen IX Project's leadership retreat. Due to my age, I know this will be my only opportunity to attend this event, as the program is made available to new participants each year. That said, I was able to take away a better sense of community, and pride at the accomplishments of those leading the charge with our shared condition. I met young college students pursuing degrees in medicine, nursing, and social services, as well as others who are already established in their careers, looking to advocate and mentor others within the community. The opportunity to participate in the large group exercises, as well as the more intimate story-sharing sessions, allowed me to gain a better understanding that, though we all share hemophilia, it's our personal

hardships and successes in life that are the driving factor in our shared desire to advocate and help our community.” — B. R.

“I was totally unsure of what to expect when I boarded a plane to meet with a bunch of strangers at something called the Generation IX Leadership Experience. I was very nervous to meet all of these new people, and as I'm in my 30s, I was worried I might not fit in well because the group seemed to be younger. It took all of one night to realize that fear was without merit. I have never felt so close to a group as I do this one. The weekend left me inspired to better myself and others, all while teaching me new techniques to accomplish that end. I can say honestly that it was a life-changing experience, and I will carry these new friends and lessons with me forever.” — J. S.





Generation IX Project offers young adults in our community many opportunities for learning and leadership. Please stay tuned for details about future programs and let us know if you have a personal interest in participating.

A huge thank you to Aptevo for your sponsorship, generosity and making this event possible!



MEN'S RETREAT SPRING 2019

SPONSORED BY



SPRING MEN'S RETREAT ANOTHER GREAT SUCCESS!

“The rarity of hemophilia B makes it difficult to feel connected to others with my disorder. Despite having something so personal that connects us, we are often separated by great distances.” This observation from one participant sums up why our Coalition retreats are so important for bringing members of our community together.





For four sunny days in April, we hosted men with hemophilia B at the Hilton Grand Vacations in Orlando, Florida, for our annual spring retreat.

The dads gathered to learn about infusion techniques and challenges for caregivers, while the other men with hemophilia B had a session on nutrition, focusing on the dangers of sugar, diabetes, and daily nutrition tips they can easily incorporate into their daily lives.

A favorite part of the retreat is the ice breaker and opening rap session. This is where the bonding happens, where everyone has a chance to meet peers, open up and share personal stories and experiences, and offer each other emotional support in a safe space.

One of the featured speakers was lawyer and longtime community activist Donnie Akers. Donnie led two important sessions, one on informed consent and ER standards of care

and the second on understanding the complex area of insurance advocacy.

Dr. David Clark, Chairman of the Coalition, gave an overview of hemophilia B.

Robert Friedman, well-known for his use of drumming to release stress and promote health and well-being, held a session titled *“What’s So Funny?”* He encouraged participants to gain optimism and be willing to laugh





more. The use of wordplay, physical humor, and vocalizations created a positive and laughter-filled environment.

There were other opportunities for camaraderie and fun at a gangster-themed murder mystery dinner and the annual Bleeder Olympics, which included a variety of backyard games. The retreat ended with participants feeling energized and empowered, and with many new and renewed friendships.

Many thanks to our sponsor, Pfizer



We look forward to seeing everyone at another Coalition for Hemophilia B event soon!



To view more photos of Coalition events, please visit our Facebook page!
www.facebook.com/HemophiliaB/



COMMENTS

“The rarity of hemophilia B makes it difficult to feel connected to other people with my disorder. Despite having something so personal that connects us, we are often separated by great distances. It is such an amazing opportunity, for which I am so grateful, to get to better know my far-flung brothers in hemophilia. The retreat and events like it are a great joy to attend and I always leave having learned a lot and also feeling more connected and alive.” — A.J.

“As a father of a hemophilia B child, I didn’t know exactly what to expect coming into the retreat weekend. My goal was to walk away with knowledge to better raise my son with this disease. After being there less than an hour, I felt that my trip was already worth it! All of the guys were so welcoming and open to talking about their personal experiences with hemophilia B. I left with an overwhelming amount of appreciation for those who came before my son to ultimately give him a better life.” — C.P.



WOMEN'S RETREAT SPRING 2019

SPONSORED BY



Women from all walks of life across the USA gathered for an event-filled weekend retreat at the Hilton Grand Vacations Tuscany Village in Orlando, Florida.





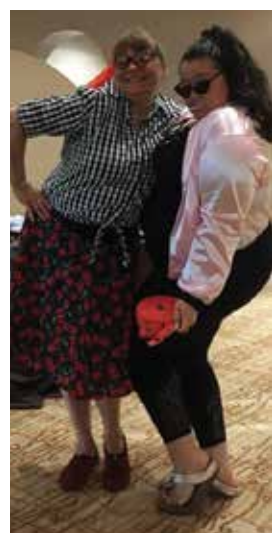
Our first session began with *Slow Flow Yoga* featuring Savasana Storytelling with Dalia Stoffer. Through meditation and relaxation, the ladies found pure tranquility with gentle guidance and meditative flow to calm their mind, body, and souls. They ended by embarking on a journey into Savasana - a peaceful and quiet mental state in yoga - to learn a new way to nurture their true selves in serenity.

There were two activities to choose from Friday and Saturday mornings after breakfast; a tranquil nature walk or *Aqua Aerobics* with a professional instructor. An interactive session began with Amy Brock on healing beliefs and learning how to retrain the mind to choose new

thoughts processes to replace the negative repetitive one and empower them.

After lunch, we had a session on *Self-Talk* and the *Power of Words* workshop engaged the ladies into speaking truths to ones self and how practicing forgiveness can simplify life and affect you and your relationships in positive ways. The ladies were able to open up to each other and were provided with a safe platform to express their feelings and learn their commonalities. Afterward, all gathered for a networking break to continue bonding. A lovely dinner was enjoyed Friday evening and we ended the day with *Art Therapy*. Each and every canvas painted reflected their true unique selves.





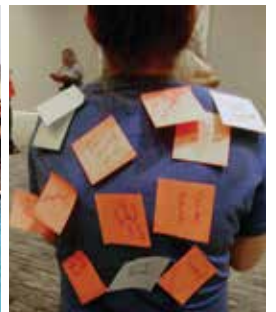


On Saturday, Daysi Fardales presented on *Adversity, Strength, and Resilience*, where the ladies learned essential patient information on various topics like coping with chronic illness, how to communicate with health care teams, learning how to advocate for themselves and loved ones, financial considerations of health care through different life stages, and the importance of staying physically active with appropriate

activities. Donnie Akers followed next with *Medicare & Medicaid 101*, where Donnie spoke on the different options for families looking for information on Social Security Disability (SSD/SSD) & Supplemental Security Insurance (SSI). Equipped with new tools and information to take home, the ladies enjoyed a healthy lunch and then nutritionist, Sheri Johnson presented on why sugar and processed foods are so addictive and dangerous for

the body and options for healthy alternative choices.

In the evening, caregivers Sherry Stacey, Milinda DiGiovanni, and woman bleeder, Stormy Johnson, openly shared their stories during the women's last session of the day. In their sharing, others felt safe to open up. Chit-chat and chocolate has always been one of our most powerful bonding experiences.





With laughter, tears and chocolate, the women wrapped up their self-awareness weekend by sharing stories and connecting with their best selves and each other. This was a very empowering and emotionally bonding experience. Our final night needed to bring joy and laughter and a '50s themed 'Prom Sock Hop' Murder Mystery Dinner was just the ticket. Dressed up in their finest fifties, the women danced, laughed, and solved the murder mystery!

yoga class and breakfast! We know lifelong friends were made and the women went home more relaxed, empowered with information and insight, and a strong network of support.

Thank you to our sponsor, Pfizer, for supporting our 2019 Women's Spring Program.



On Sunday morning we held a light



WOMEN'S RETREAT COMMENTS

"This retreat meant getting to take time for me and connecting with other amazing women to share the same struggles and triumphs as myself. Our self-care is just as important as our roles as caregivers and the Coalition goes above and beyond to make that happen for us."
— N.S.

"The women's retreat helps me understand factors with hemophilia better by hearing other stories from moms that have been in the same situation as me with their sons' bleeds. Overall just knowing we are not alone. I'm very grateful to meet so many special moms and make friends. I know I have blood sisters just a phone call away! Thanks again for the retreat!"
— M.D.

"It was a wonderful weekend, with women that have been through so much more than I have. Was so very interesting to hear their stories, and see how they were not afraid to share, or give input when questioned. I loved every moment that I got to spend at the women's retreat. Thank you so much for letting me be a part of this."— E.T.

"I really look forward to this event, thank you for the Sponsoring this women's retreat. This is such a refreshing way for me to spend a weekend with a group of women who can totally understand this huge part of your life that most people can't relate to. To be with my blood sisters and friends that I only see once or twice a year is the best way to learn and rejuvenate. You guys are amazing and I appreciate all that you do for our community." — T.M.

To view more photos of Coalition events, please visit our Facebook page! www.facebook.com/HemophiliaB/



In Memoriam

Kevin Scates

In March 2019, we lost one of our hemophilia B brothers, Kevin Scates. He was a kind and loving person who cared very deeply for his family and community. Our thoughts and prayers go out to his loved ones.

WASHINGTON DAYS



Representatives of The Coalition for Hemophilia B team joined members of the New York bleeding disorders community for NHF's Washington Days March 28-29, 2019. Among other elected officials and staff on Capitol Hill, our group met with Congresswoman Grace Meng, who represents New York's 6th congressional district in the borough of Queens. This is a critical year to be in Washington as the gains of the Affordable Care Act are again threatened by judicial and legislative activities.



HFA's 25TH ANNUAL SYMPOSIUM



The Coalition for Hemophilia B had a wonderful time at the Hemophilia Federation of America's 25th Annual Symposium in San Diego April 4-7, 2019. We were glad to see so many of our Factor 9 friends there!



Knowing her son is protected from bleeds gives Milinda peace of mind

When Milinda's son Andrew was younger, his frequent bleeds made him miss out on a lot. Their family life was hectic with his many doctor appointments and hospital visits. Now Milinda and Andrew have more free time to spend with family and friends.



#1 FACTOR IX CHOICE

When Changing Therapy*

Find out more about powerful bleed protection at **IDELVION.com**

“I’m a mom, I’m going to worry no matter what. But with IDELVION, we both feel more confident in his bleed protection.”

—Milinda, whose son Andrew switched to IDELVION in 2016

Important Safety Information

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

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

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

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

IDELVION is manufactured by CSL Behring GmbH and distributed by CSL Behring LLC.

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www.CSLBehring.com www.IDELVION.com IDL-0278-JAN19

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

Important Safety Information (cont'd)

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

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

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)

IDELVION is manufactured by CSL Behring GmbH and distributed by CSL Behring LLC.

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www.CSLBehring.com www.IDELVION.com IDL-0278-JAN19

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.

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.



B VOICE

ADVOCACY UPDATE

Advocacy is the way our hemophilia B community voices our concerns in asking elected officials and other leaders for the things we need to live happy, healthy lives. That's why we call our advocacy program the "B Voice."

By Glenn Mones

There are many ways you can get involved and express YOUR B Voice, and staying informed on key issues is the first step. The Coalition for Hemophilia B is monitoring challenges to your access to care and efforts to surmount those challenges. This B Voice Advocacy Update will appear as a regular feature in Factor Nine News. It will be your place to keep yourself up to date on issues that affect our entire community, including you and your family.

In between our quarterly newsletter, we issue email, web, and social media alerts—which we encourage you to follow. Remember that you can always find more information on our website at: www.hemob.org.

ISSUE 1: ACCUMULATOR ADJUSTOR PROGRAMS

Last fall we reported on a growing trend of insurance companies refusing to count third-party co-pay assistance payments against the insured's annual out-of-pocket maximum. These programs, which essentially amount to "double-dipping" by the insurers, have the potential to dramatically increase the cost of medications for people with hemophilia and other high-cost conditions.

The hemophilia community, with the National Hemophilia Foundation (NHF) taking the lead, has been working to eliminate or minimize the impact of these programs. On April 18, 2019, these efforts bore fruit as the Centers for Medicare and Medicaid Services (CMS) released the final Notice of Benefit and Payment Parameters Rule for 2020.

This rule, released annually, sets limits on what private insurance plans may or may not do. Although the new rule does not ban accumulator programs completely, it does limit their application to brand-name drugs that have equally effective generic alternatives. Because there are no generic drugs for hemophilia, all of the currently available drugs used to treat it should be exempt.

Although the new CMS rule does not ban accumulator programs completely, it does limit their application to brand-name drugs with equally effective generic alternatives. Because there are no generic drugs for hemophilia, all currently available drugs used to treat it should be exempt.

This is a very important win for the entire hemophilia community. However, it is likely that insurers will continue to look for other ways to limit what they pay while forcing patients to pay more. That's why it is vital for us to consistently track any challenges community members face in getting their treatments covered.

If you or someone you know encounter such challenges, please inform us at The Coalition for Hemophilia B, as well as the folks at your local chapter and other national organizations.



ISSUE 2: CHALLENGES TO THE AFFORDABLE CARE ACT

The Affordable Care Act (ACA), also known as “Obamacare,” brought many improvements to the way healthcare is reimbursed in the United States. It eliminated lifetime insurance caps under most policies, banned discrimination in insurance based on preexisting conditions, expanded Medicaid and other coverage options in many states, and allowed young people to stay on their parent’s insurance policies for longer periods of time. These reforms have been of immense benefit to people affected by hemophilia and other chronic, high-cost conditions.

The ACA, which for now is still in effect, has survived many attacks. Unfortunately, it is being threatened like never before.

The ACA, which for now is still in effect, has survived many attacks. Unfortunately, it is now being threatened like never before. You may have read that last December, a federal district court ruled in *Texas v. United States* that the Affordable Care Act is unconstitutional in its entirety. To make matters worse, on March 25, 2019, the Department of Justice (DOJ) decided in an unusual measure that it

would not defend the ACA, essentially taking the position that the entire law and all its provisions should be struck down.

So, what happens now? First, it is important to know that for now, the ACA and its provisions remain in effect. Your Obamacare or expanded Medicaid coverage (should you have one of those) will not just disappear tomorrow.

Elected officials who support protecting patient access to care are working to find ways to counter these attacks. H.R.1884—the Protecting Pre-Existing Conditions and Making Health Care More Affordable Act of 2019—was introduced recently by Representative Frank Pallone Jr. The bill is now in committee and has 146 co-sponsors. The legislation is designed to protect the gains made by the ACA while making health insurance even more accessible and affordable for all Americans. It is not a bipartisan bill, so while it could pass in the Democrat-controlled House, it is unlikely that it would garner enough support in the Republican-controlled Senate. Therefore, while it represents a strong “return volley” in the latest battle over healthcare, it is likely to be subsumed at some point by a broader piece of legislation that will be the product of cross-aisle compromises.

The bottom line is that we can anticipate a lot more activity on healthcare in the next weeks and months. We will continue to monitor these issues, keep you informed, and ask your help in making sure we keep our access to affordable healthcare.

DON'T STAND ON THE SIDELINES

LEARN IF RIXUBIS®
MAY BE RIGHT FOR YOU



Visit RIXUBIS.com to learn more

Indications for RIXUBIS [Coagulation Factor IX (Recombinant)]

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

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

You should tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies, have any allergies, including allergies to hamsters, are nursing, are pregnant or planning to become pregnant, or have been told that you have inhibitors to factor IX.

Allergic reactions have been reported 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.

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.

If you have risk factors for developing blood clots, the use of factor IX products may increase the risk of abnormal blood clots.

Common side effects that have been reported with RIXUBIS include: unusual taste in the mouth, limb pain, and atypical blood test results.

Call your healthcare provider right away about any side effects that bother you or if your bleeding does not stop after taking RIXUBIS.

Please see following page for RIXUBIS Important Facts.

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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RIXUBIS
[COAGULATION FACTOR IX
(RECOMBINANT)]

MOVING FORWARD

RIXUBIS

[COAGULATION FACTOR IX (RECOMBINANT)]

Important facts about

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, and it does not include all of the important information about RIXUBIS. If you have any questions after reading this, ask your healthcare provider.

What is RIXUBIS?

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

Who should not use RIXUBIS?

You should not use RIXUBIS if you

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

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

What should I tell my healthcare provider before using RIXUBIS?

You should tell your healthcare provider if you

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

How should I infuse RIXUBIS?

RIXUBIS is given directly into the bloodstream. RIXUBIS should be administered as ordered by your healthcare provider. 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.

Your healthcare provider will tell you how much RIXUBIS to use based on your weight, the severity of your hemophilia B, and where you are bleeding. You may have to have blood tests done after getting RIXUBIS to be sure that your blood level of factor IX is high enough to clot your blood. Call your healthcare provider right away if your bleeding does not stop after taking RIXUBIS.

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. 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?

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.

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.

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.

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 http://www.shirecontent.com/PI/PDFs/RIXUBIS_USA_ENG.pdf or by calling 1-800-FDA-1088.

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HEMOPHILIA UPDATE

By Dr. David Clark

RATE OF JOINT REPLACEMENT SIMILAR IN HEMOPHILIA A AND B

There has been a lot of discussion in the research community about whether hemophilia A or B is more severe. Some studies have suggested that hemophilia A patients may suffer worse joint damage over time, but the



problem has always been that there have been too few Bs in the studies to make a definitive conclusion. Now a group in Taiwan has performed a large population-based study. They looked at the incidence of total joint replacement (TJR: hip, shoulder, knee, elbow, and ankle) in 782 males with hemophilia A and 153 males with hemophilia B. Data were collected from 1997 to 2013 from the Taiwanese National Health Insurance Database, which covers essentially 100% of Taiwanese citizens.

The results showed no statistical difference, with 10.6% of A patients receiving TJR compared to 10.5% of Bs. The study excluded inhibitor patients, but not patients with comorbidities like HIV or hepatitis C. The average age of study patients was 35.1 for the As and 31.6 for the Bs. The most common replaced joint was the knee (72.3% in A; 68.8% in B), followed by the hip (20.5% in A; 18.8% in B). [Lin W-Y et al., *Clinical and Applied Thrombosis/Hemostasis* (2018) published online 9/13/18]

PREVALENCE OF AUTISM SPECTRUM DISORDER IN CHILDREN WITH HEMOPHILIA

A recent British study has shown that the prevalence of autism spectrum disorder (ASD) is higher in boys with hemophilia than in non-hemophiliac controls. In the general population, about 10/1000 have ASD, with a 3:1 prevalence in boys over girls. There is no known connection between hemophilia and ASD, but the researchers had noticed a high ASD prevalence at their hemophilia center. They found a



prevalence of 74.8/1000, much higher than in the general population. The study included 11 As (9 severe) and 5 Bs (3 severe). Three patients (23%) had a history of intracranial hemorrhage (bleeding in the brain). Their average age at diagnosis of ASD was 6.46 years. Further research is needed to explain the findings and explore risk factors for ASD in hemophilia.

[Bladen M et al., presented at the European Association for Haemophilia and Allied Disorders (EAHAD) Congress, February 6–8, 2019, Prague, Czech Republic, Abstract P210]

ERECTILE DYSFUNCTION IS MORE PREVALENT WITH AGE IN MEN WITH HEMOPHILIA

As people with hemophilia live longer, we are finding more age-related disorders in that population. One of the conditions that is rarely discussed, despite its impact on quality of life, is erectile dysfunction (ED) in older men. A group of Canadian researchers looked at 44 male subjects (27 As; 17 Bs). Using a standardized ED questionnaire, as well as clinical information and lab tests, they found that 39% of the subjects had symptoms of ED. The risk appeared to increase with age. Other risk factors were smoking, high blood pressure, larger hip-to-waist ratio, and higher homocysteine levels in the blood (a marker of blood vessel inflammation). The results were not compared with non-hemophiliac controls. More research is necessary before any connection between ED and hemophilia is proposed. If you are concerned about ED, talk to your doctor. There are a number of medications that work extremely well, and several have recently gone generic, so the prices have come down.

[Yang M et al., *Haemophilia*, 25(2), 283–288, 2019]

ENDOSCOPIC BLEEDING RISK NOT HIGHER IN HEMOPHILIA PATIENTS ON PROPHYLAXIS

Endoscopic procedures have become common in modern medicine. An endoscope is a long narrow tube that doctors can use to look into the body and also perform many surgical procedures. A group of Canadian researchers, concerned about whether endoscope use might pose an increased bleeding risk for patients with bleeding disorders, conducted a study of 48 adults at their hospital receiving gastrointestinal endoscopies (esophagus, stomach, or colon, including colon polyp removal). The subjects had hemophilia A or B, von Willebrand disease, or factor VII or XI deficiency. In 104 endoscopies overall, they found no excess bleeding risk, as long as the patients were on preoperative prophylaxis.

[Tomaszewski M et al., *Haemophilia*, 25(2) 289–295, 2019]

GETTIN' IN THE GAME 2019 ESSAY CONTEST

The Coalition for Hemophilia B will once again nominate two (2) winners of this annual essay contest to attend the 2019 Junior National Championships Gettin' in the Game competition, which will be held November 8–10 in Phoenix, Arizona.

DEADLINE TO ENTER: JULY 22

The essay contest is for Coalition members age 7–18 with hemophilia B. To enter, submit an essay on “**Why I Love Baseball, Swimming, or Golf.**”

Essay length should be age-appropriate. Include your name, age, diagnosis, address, phone, and your parent's name. (Parents: Be sure to have your child write it! It's about the passion for the sport, not perfection!)

Submit your essays by email, fax, or regular mail:

Email: karenb@hemob.org

Fax: 212-520-8501

Mail to:

The Coalition for Hemophilia B
757 Third Avenue, 20th Floor
New York, NY 10017

Deadline is July 22, 2019.

Questions? Please call 212-520-8272. We will notify winners by July 26!

ABOUT THE JNC

The Junior National Championships is a competition expressly for children with bleeding disorders. During the program, our nominees will have the opportunity to participate in baseball, golf, and swimming activities to learn the fundamentals, improve

their skills, compete with their peers, and learn about the importance of physical fitness in managing a bleeding disorder. Accomplished athletes, who themselves have been diagnosed with a bleeding disorder, are part of the program to offer pro tips.

Each participant must be accompanied by one adult (a parent or caregiver).

CSL Behring will sponsor each contest winner and accompanying adult. Sponsorship covers coordination and cost of airfare, ground transportation in Phoenix, and hotel arrangements. (You will be responsible for transportation to and from your hometown airport.)

**GET WRITING AND
GOOD LUCK!**

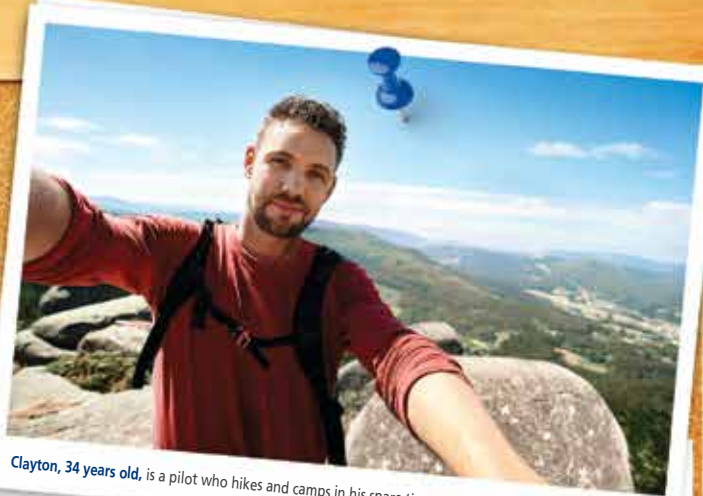
SAVE THE DATE

**2019 Gettin' in the GameSM
Junior National
Championship (JNC)**

**Friday, Nov. 8th –
Sunday, Nov. 10th
Phoenix, Arizona**



In hemophilia B
**TAKE CONTROL TO A
HIGH LEVEL
WITH REBINYN®**



Clayton, 34 years old, is a pilot who hikes and camps in his spare time. Clayton lives with hemophilia B.

Rebinyn® elevates factor levels above normal levels^a

+94% Factor IX (FIX) levels achieved immediately after an infusion^b

17% FIX levels sustained after 7 days^a

With a single dose of Rebinyn® 40 IU/kg in adults with $\leq 2\%$ FIX levels^a

^aIn two phase 3 studies, factor levels were evaluated for 1 week after the first dose of Rebinyn® 40 IU/kg. The average levels after 7 days were 16.8% in 6 adults, 14.6% in 3 adolescents, 10.9% in 13 children ages 7 to 12 years, and 8.4% in 12 children up to age 6 years.

^bBased upon a 2.34% increase in factor levels per IU/kg infused in adults.

Image of hemophilia B patient shown is for illustrative purposes only.

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



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

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rebinyn®
Coagulation Factor IX
(Recombinant), GlycoPEGylated

rebinyn®

Coagulation Factor IX (Recombinant), GlycoPEGylated

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

Rx Only

This information is not comprehensive.

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

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

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

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

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

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

What is REBINYN®?

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

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

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

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

Who should not use REBINYN®?

You should not use REBINYN® if you

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

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

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

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

You should tell your healthcare provider if you

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

How should I use REBINYN®?

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

REBINYN® is given as an infusion into the vein.

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

infuse the medicine by themselves or with the help of a family member.

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

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

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

Use in children

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

If you forget to use REBINYN®

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

If you stop using REBINYN®

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

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

What if I take too much REBINYN®?

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

What are the possible side effects of REBINYN®?

Common Side Effects Include:

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

Other Possible Side Effects:

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

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

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

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

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

More detailed information is available upon request.

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

Revised: 11/2017

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

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TREATMENT NEWS

By Dr. David Clark

APTEVO APPROVED FOR 3000 IU IXINITY VIALS. ALSO PLANS PEDIATRIC STUDY

4/15/19 Aptevo Therapeutics announced that they have received FDA approval to produce 3000 IU vials of Ixinity, their recombinant factor IX product. They plan to launch the new size in mid-2019. Aptevo is also planning to launch a Phase IV (post-licensure) study of Ixinity for use in the pediatric population in the 3rd quarter of 2019. [Aptevo Press Release]



BIOVERATIV/CSL PATENT DISPUTE UPDATE

1/6/19 Bioverativ (now part of Sanofi) filed a suit against CSL Behring in 2017 claiming that CSL's Idelvion infringes several of Bioverativ's patents for extended half-life products. CSL counter sued claiming that Bioverativ's patent was invalid. The Patent Trial and Appeal Board (PTAB) denied CSL's challenge (a patent challenge is called an inter partes review or IPR) to Bioverativ's patent. They said CSL did not provide sufficient explanation why Bioverativ's technology would have been obvious to a "person of ordinary skill in the art." Bioverativ's original suit against CSL is now set for a jury trial starting 3/30/20. The legal system moves slowly. (The Coalition, NHF and HFA have all filed comments with the court objecting to Idelvion's possible removal from the U.S. market, if Bioverativ wins the suit.) [https://www.biosimilarsip.com/2019/01/31/the-ptab-denies-institution-of-two-iprs-involving-a-patent-directed-to-the-treatment-of-hemophilia-b/]



CATALYST ANNOUNCES UPDATE ON VARIANT FACTOR IX

12/18/19 Catalyst Biosciences is developing DalcA (dalcinonacog alfa), a variant factor IX that has an extended half-life and can be injected subcutaneously. DalcA is 22X more active than normal factor IX. Catalyst (not FDA) had stopped their Phase I/II clinical study of DalcA after two patients developed antibodies (inhibitors) to the product. One inhibitor was



temporary and one longer-lasting. Both only reacted to DalcA and not normal factor IX. Catalyst has done an impressive amount of research and determined that DalcA is no more immunogenic (prone to immune reactions and inhibitor development) than normal factor IX. That is, patients have an equal chance of developing inhibitors to normal factor IX products and to DalcA. This finding led FDA to grant permission to proceed to Phase IIb.

Interestingly, the fact that the inhibitors did not react to normal factor IX, might mean that DalcA could be used as an alternate treatment by factor IX inhibitor patients. There is no word whether Catalyst will pursue that.

4/2/19 Catalyst Biosciences announced the beginning of enrollment for their Phase IIb study of DalcA. The study will involve six patients receiving DalcA for 28 days with the aim of keeping their factor IX levels above 12%. The study is expected to be complete in late 2019. [Catalyst Press Releases]

(Note: This is Catalyst Biosciences, not Catalyst Pharmaceuticals. Catalyst Pharmaceuticals, which is not in the hemophilia market, was recently targeted for increasing the cost of a previously free drug to \$375,000.)

CATALYST ANNOUNCES UPDATED RESULTS FOR MARZAA

2/8/19 Catalyst Biosciences announced updated results for marzeptacog alfa (activated) (MarzAA), their daily subcutaneous activated factor VII product for inhibitor treatment. MarzAA is a variant FVIIa currently in Phase II/III clinical studies. Results have shown a marked reduction in bleeding with no significant adverse events and no evidence of inhibitor formation against MarzAA. The study is also investigating the quality of life (QoL) of patients with inhibitors. After only 50 days of treatment, subjects showed improvement in QoL as measured by the HAL and Haem-A-QOL assessment tools. They have completed enrollment and expect to have results in Q3/2019. [Presented at the European Association for Haemophilia and Allied Disorders (EAHAD) Congress, February 6-8, 2019 in Prague, Czech Republic]



Hemophilia B Connected is an online discussion board where patients, caretakers, and their loved ones have the opportunity to ask questions, share experiences, and connect with one another on a wide range of peer support issues: Advocacy, Psychosocial issues (depression, pain management, unaffected siblings), Physical Therapy, Nutrition & Exercise, Inhibitors, New Family Support, Aging with Hemophilia, and much more! Users can customize and control the content they receive. To request access to Hemophilia B Connected, contact administrators Rick Starks and Shad Tullidge at: bconnected@hemob.org.

Note that Treatment News is now being updated regularly on B Connected. Look for the Treatment News channel. News will be posted online soon after it is announced.

CATALYST ENTERS THE GENE THERAPY RACE

2/6/19 Catalyst Biosciences presented preclinical results

for their CB 2679d-GT gene therapy for hemophilia B. CB 2679d-GT uses the gene for their factor IX variant DalcA, which is being developed for hemophilia B treatment (see above). In hemophilia B mice, CB 2679d-GT achieved a 4X reduction in bleeding time and a 3X increase in clotting activity, compared to FIX-Padua. FIX-Padua is already about nine times more active than normal factor IX. FIX-Padua is being used by uniQure and Pfizer in their gene therapy treatments.

[Presented at the European Association for Haemophilia and Allied Disorders (EAHAD) Congress, February 6–8, 2019 in Prague, Czech Republic]



safety or efficacy of the product. [NCT02484638 at clinicaltrials.gov]

FDA LICENSES BIOSIMILAR RITUXIMAB

12/10/18 FDA has approved Truxima (rituximab-abbs), a biosimilar to Roche's Rituxan (rituximab). Rituximab is an anti-cancer agent that is sometimes used to treat hemophilia inhibitor patients. It leads to the destruction of immune system B cells, which are implicated in the production of inhibitors.



A biosimilar is the generic equivalent of a biological drug. When a small-molecule drug loses its patent protection after a number of years, the FDA encourages other companies to produce the same drug as a generic. This is seen as a way to increase competition, lower prices and prevent market shortages. Since the brand-name drug has already been tested extensively, the generic producers don't have to repeat the same expensive studies. They just have to show that their product is equivalent to the brand-name product.

Biosimilars work the same way. Now that Roche's Rituxan is off-patent, other companies can produce it, as Teva and Celltrion, the manufacturers of Truxima have done. This may help reduce the cost for inhibitor patients using rituximab.

[<http://www.biopharminternational.com/fda-approves-celltrion-and-teva-s-biosimilar-rituxan-0>]

12/2/18 Pfizer is also developing a biosimilar rituximab. They are halfway through their clinical comparability study of PF-05280586, their candidate. [Pfizer press release]

Note that neither of these rituximab biosimilars will be indicated for treatment of hemophilia inhibitors, but physicians may use them off-label as they do now for Rituximab.

CSL STUDIES HEALTH-RELATED QUALITY OF LIFE IN KIDS ON IDELVION

11/14/18 CSL Behring published a study of health-related quality of life (HRQoL) in pediatric patients on Idelvion, their extended half-life factor IX. Twenty patients on prophylaxis were studied in two groups: twelve patients aged 4–7 and eight patients aged 8–12. Using validated questionnaires for both the patients and their caregivers, CSL found that the younger group showed significant improvement in "physical health." The older group showed improvement in physical health and many other areas. CSL's conclusion is that HRQoL is substantially improved in patients on prophylaxis with Idelvion and caregivers are more satisfied. It's not clear whether the findings would also apply to other extended half-life products.

[von Mackensen S et al., Haemophilia 25(1), 45-53, 2019]



CSL TERMINATES CLINICAL STUDIES FOR LONGER-ACTING FVIIA

1/4/19 CSL Behring has terminated clinical study NCT02484638 for their longer-acting factor VIIa product for treatment of inhibitor patients. The product, CSL 689 or rFVIIa-FP, was a recombinant activated factor VII fused to albumin to give it a longer half-life. The project was terminated due to business decisions, not because of the



“One of the most important things you can do on this earth is to let people know they are not alone.”

— Shannon L. Alder



THE COALITION FOR HEMOPHILIA B PATIENT ASSISTANCE PROGRAM

The high cost of medical care is often a challenge for people with hemophilia B. Fortunately, insurance coverage, government programs and other forms of patient assistance cover much of that cost. Unfortunately, these programs do not cover the cost of non-medical emergencies, which may interfere with a family or individual's ability to deal with day-to-day life with a bleeding disorder. These emergencies may involve struggling to having enough resources for housing, food, transportation, or a range of other necessary and critical needs.

When these needs are not met, the health and well-being of the patient as well as the entire family can be negatively affected. Often, assisting a person in an immediate circumstance is all that's needed to keep the situation from spiraling out of control.

The Coalition for Hemophilia B deeply cares about families and individuals, and the urgent needs they may face. Several years ago, because of this and in order to live true to our mission statement, we established a patient assistance program for hemophilia B patients and families. We reintroduce our program as **BCares**.

BCares operates with funding generously donated by pharmaceutical manufacturers, homecare companies, business partners, and other interested supporters.

Those donating share our belief - in the case of an urgent situation, we can all do more to help. It is our obligation as a community to lend a hand and assist those in short-term, dire straits.

The Coalition for Hemophilia B is able to offer a limited amount of financial aid to our factor 9 community members who face a financial emergency. Those requesting assistance can submit a simple, confidential application. Each application will be reviewed thoroughly by a committee, who will determine and prioritize grants based on the request and level of urgency.

How you can help: We are exceedingly grateful to the donors whose charity and compassion have made this critical program possible. Please consider becoming involved by offering additional funds so we may help more hemophilia B patients through challenging times.

For more information, please contact:

Farrah Muratovic
farrahm@hemob.org
The Coalition for Hemophilia B

Tel: 212-520-8272
hemob.org



TREATMENT NEWS

GC PHARMA DEVELOPING A TFPI INHIBITOR



Undated. GC Pharma, formerly Korean Green Cross Corporation, is developing an inhibitor to tissue factor pathway inhibitor (TFPI), an anti-coagulant. GC's MG1113A is a monoclonal antibody for subcutaneous injection that has a long half-life. Several other companies are also developing anti-TFPI products. The idea is that reducing the amount of TFPI will restore the balance in the clotting system so it will form clots more easily in hemophilia patients. These products should work for both hemophilia A and B.

[GC Pharma website]

HEMA BIOLOGICS ANNOUNCES FDA RESPONSES TO FVIIA LICENSE APPLICATION



4/10/18 HEMA Biologics reported that FDA has no efficacy or safety concerns with HEMA's license application for their LR769 Eptacog Beta Activated factor VIIa treatment for treatment of hemophilia A and B patients with inhibitors. Eptacog Beta Activated is produced in the milk of transgenic rabbits, from which it is purified to produce the final product.

[HEMA Biologics Press Release]

INTELLIA PRESENTS RESULTS FOR CRISPR/CAS9 FACTOR IX GENE THERAPY



4/29/19 Intellia Therapeutics in collaboration with Regeneron Pharmaceuticals presented results for a factor IX gene therapy employing CRISPR/Cas9 gene editing with delivery by both lipid nanoparticles (LNPs) and AAV virus vectors. In studies in mice and non-human primates Intellia obtained factor IX levels in the normal range. The method uses LNPs to deliver the CRISPR/Cas9 gene editing molecules and a guide RNA to liver cells at the same time that a factor IX gene is delivered to the cells using an AAV virus vector. The CRISPR/Cas9 system inserts the factor IX gene into the genome under the control of the albumin promoter.

[Intellia Press Release and Presentation]

ROCHE PURCHASE OF SPARK THERAPEUTICS DELAYED



4/26/19 Roche has withdrawn its notification to U.S. regulators that it plans a \$4.8 billion acquisition of Spark Therapeutics, a leading gene

therapy company. The regulators need more time for an antitrust review because both companies are active in the hemophilia A space. Note that this does not affect Spark's SPK-9001 gene therapy for hemophilia B, which has already been transferred to Pfizer. SPK 9001 is currently in Phase III clinical studies under Pfizer.

[Roche Press Release]

ST. JUDE AND WFH COLLABORATE ON GENE THERAPY



WORLD FEDERATION OF HEMOPHILIA

4/17/19 St. Jude Children's Research Hospital and the World Federation of Hemophilia (WFH) announced a collaboration to further develop St. Jude's gene therapy treatment with the aim of treating patients in low- and middle-income countries. St. Jude, along with University College London (UCL) pioneered one of the first successful gene therapy treatments, which was described in a 2011 article in the New England Journal of Medicine (NEJM). A 2014 follow-up article in NEJM reported that the study patients were still benefiting from the treatment. Note that the St. Jude/UCL method is also being developed further by the commercial company Freeline Therapeutics, a spin-off from UCL.

[St. Jude Press Release]

SANGAMO BEGINS AND ENDS CLINICAL STUDY FOR FIRST "GENE EDITING" TREATMENT FOR HEMOPHILIA B



12/17/18 Sangamo Therapeutics has announced the treatment of the first patient in their Phase I/II study evaluating SB-FIX, their gene therapy for hemophilia B. Sangamo uses the zinc finger nuclease (ZFN) gene editing method to integrate a factor IX gene directly into the genome of liver cells. Most of the previous gene therapy treatments have used a non-integrating gene construct - the existing genes are not edited (modified), instead a new gene is just delivered into the nucleus of a liver cell, but it remains a free-floating piece of DNA.

Sangamo's method is really the first "gene editing" method tried for hemophilia B. The ZFNs actually modify the existing genes, placing a new factor IX gene into the genome under the control of the existing albumin gene. Albumin is the most prevalent protein in plasma, the liquid part of the blood. Under the control of the albumin gene, Sangamo expects the new gene to produce plenty of factor IX.

Continued on page 49.



FREE for individuals and families with HEMOPHILIA B. To join B Connected, contact administrators Rick Starks and Shad Tullidge at: bconnected@hemob.org

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- » DEBBIE DE LA RIVA, LPC: MENTAL HEALTH & BLEEDING DISORDERS



Why B Connected?

New therapies are flooding the market. It's more important than ever that everyone in the Hemophilia B community has a way to:

- » Get critical information in a timely way.
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- » Stay engaged with the community virtually even if your hemophilia limits your mobility.
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Through B Connected you can also digitally join online **Ask the Expert** sessions—hour-long discussions on topics such as advocacy, depression, pain management, unaffected siblings, physical therapy and how to cut down on joint bleeds, nutrition and exercise, inhibitors, new family support, aging with hemophilia, and much more!

JOIN TODAY!

Hemophilia B Connected online discussion board is hosted on Slack and is 100% HIPAA compliant.



TREATMENT NEWS

4/2/19 Sangamo Therapeutics obtained poor results (low factor levels) in their Phase II study of SB-FIX, a gene therapy treatment for hemophilia B. The study is now on hold and they have gone back to preclinical studies using a second-generation ZFN (ZFN 2.0) that is five to twenty-fold more. No further information is available at this time. [Sangamo Press Release and 4/2/19 email]

SANOI ANNOUNCES LESS-IMMUNOGENIC GENE THERAPY VECTORS FOR FACTOR IX

5/22/19 Sanofi (Bioverativ) and the San Raffaele Telethon Institute for Gene Therapy (SR-TIGET) in Italy published the results of a study looking at ways of making the viral vectors used for gene therapy less likely to trigger an immune reaction. One issue with using viral vectors is that they are often attacked by the immune system, which can decrease the effectiveness of the treatment. The researchers found that by engineering the surface of the vectors to contain the protein CD47, the vectors become less susceptible to detection by the cells of the immune system. They showed in non-human primates that CD47-modified lentiviral vectors showed enhanced transfer of factor IX genes to liver cells, producing higher factor levels than with non-modified vectors. [Milani et al., Sci. Transl. Med., 11, eaav7325 (2019)]



SHIRE BECOMES PART OF TAKEDA

1/8/19 Shire plc, which had purchased Baxalta after it had been spun-off from Baxter International, became part of Takeda Pharmaceutical Company Limited on 1/8/19. Takeda is a global biopharmaceutical company based in Japan. Takeda focuses on four areas: Oncology, Gastroenterology, Neuroscience and Rare Diseases. They also have a vaccines business, and as part of the Shire purchase, they will also have a major stake in the plasma-derived pharmaceuticals market. The combined annual revenue of the companies exceeds \$30 billion.



Also, Shire/now Takeda won an International Society of Pharmaceutical Engineers (ISPE) 2018 Facility of the Year award for the upgrade of their Los Angeles facility, which originally was Baxter's plasma fractionation plant. [Shire and ISPE Press Releases]

SIGILON AND MIT COLLABORATE ON GENE THERAPY FOR HEMOPHILIA A AND B

1/7/19 Sigilon Therapeutics and Massachusetts Institute of Technology (MIT) are collaborating on a gene therapy approach for both hemophilia A (SIG-001) and B (SIG-003). Sigilon has developed novel engineered human cells that have a synthetic biomaterial covering called Afibromer. The Afibromer coating keeps the cells from being seen by the immune system, thus preventing immune reactions. The cells can be programmed to produce factor VIII or IX. They would be implanted in the body to produce the missing factor in hemophilia patients. [Sigilon press release]



UNIQUIRE ANNOUNCES UPDATED CLINICAL DATA ON HEMOPHILIA B GENE THERAPY

2/8/19 uniQure N.V. announced results from their Phase IIb study of AMT-061, their gene therapy candidate for hemophilia B. The Phase IIb study was conducted to determine the best dose for the product after uniQure changed to the factor IX Padua gene variant. The three severe B patients in the study achieved an average factor IX level of 38%. All three patients had low levels of pre-existing antibodies against AAV5, the vector, but were able to be treated successfully. In addition, two of the patients had been excluded from a different gene therapy study because they had pre-existing antibodies to the AAV vector used in that study. The treatment was well-tolerated with no serious adverse reactions. None of the patients required immunosuppression. [Presented at the European Association for Haemophilia and Allied Disorders (EAHAD) Congress, February 6-8, 2019 in Prague, Czech Republic]



5/10/19 uniQure NV announced additional results from their Phase IIb study of AMT-061, their gene therapy treatment for hemophilia B. Six months after treatment administration of AMT-061, the factor IX levels in the three patients enrolled in the Phase IIb study have increased to 33%, 51% and 57% of normal. [uniQure Press Release]

2/28/19 uniQure N.V. is enrolling patients for their Phase III study of their AMT-061 hemophilia B treatment with the first patient treated in early February. They plan to enroll approximately 50 patients by the end of 2019. [uniQure Press Release]

2019

Meetings on the Road

Bringing education and support to families living with hemophilia B. We look forward to seeing you soon!

THE COALITION FOR
HEMOPHILIA



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Registration on our website
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V	E	O	K	I	W	C	L	A	N
O	O	V	G	H	A	X	Q	B	I
O	X	A	F	E	T	X	Y	Z	D



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