Factor Nine News

The Coalition for Hemophilia B

WINTER 2018

Topics in Hemophilia

GENERATION IX PROJECT

LEADERSHIP THROUGH SERVICE

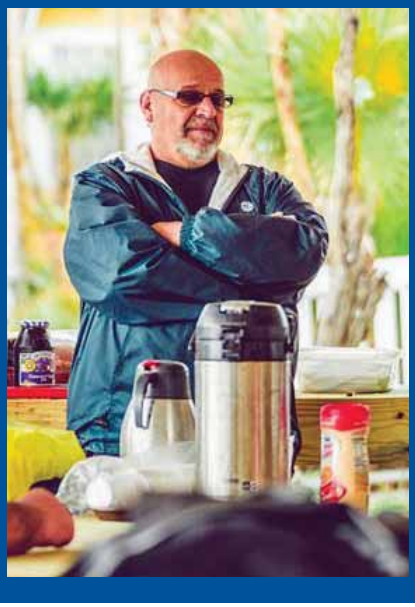
OUR EXPERIENCES AT THE PIGEON KEY GENERATION IX PROGRAM

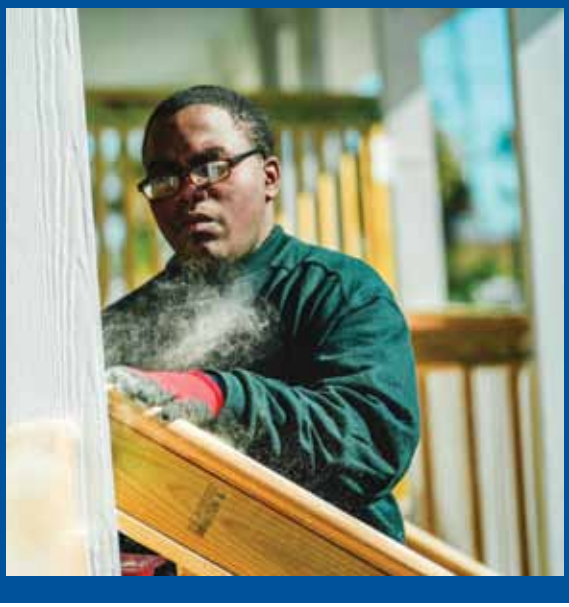
BY MANUEL A. LOPEZ SERRANO, SHAD H. TULLEDGE AND EMILI LEE

The Generation IX Project gathers community members with Hemophilia B from all over the United States and Puerto Rico. Generation IX Project Leadership was held in Pigeon Key, Florida from January 2nd to the 7th of 2018 with the main educational theme of "Service Leadership". Gutmonkey, the facilitators of the project, created a space where each of the mentors became submerged into the concept of service leadership, not just within the bleeding disorders community, but also in society in general. Embracing their roles as leaders, the Gen IX crew set out to make a difference.

Aptevo Therapeutics

Thank you to Aptevo for generously sponsoring our Gen IX Meeting!

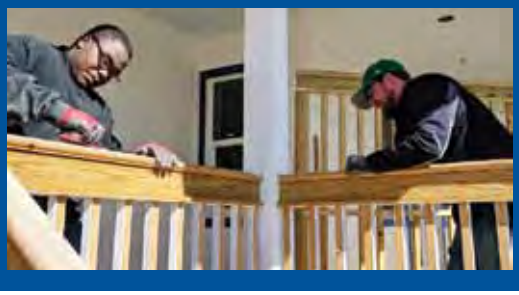




This leadership model brought the group together to learn about charity and service and that this model had three essential concepts at its core: the acts of being compelled, courageous and committed; which come into play in their advocacy and leadership efforts. During their time in the Keys, the mentors were given the opportunity to bring service leadership into practice. They first helped the Pigeon Key Foundation to restore part of the landscape of the Key. It was amazing to see the impact of what a simple act of kindness, support and service can do.

The mentors were also given the opportunity to put service leadership into practice. They made their way to Marathon Key, home of the Dolphin Research Center, to help the center in their efforts to recover from the hurricane. They were excited to receive the group of Hemophilia B mentors and were impacted to see members of a community that might normally be labeled as “physically limited,” to actually come and do the dirty work and heavy lifting, for the simple reason to give back and put service leadership into practice. The group of amazing individuals that made part of the Generation IX team, had an amazing time at this program and will most likely say, that their lives were changed for the better. Service Leadership is more than just a concept; it’s an act of love, compassion, commitment or just the simple act of being human. This group of individuals is paying forward what they have received and acting upon their commitment to make the world a better place, one act of kindness at a time.
~ Manuel

Hurricane Irma devastated the Florida coast and unfortunately had a great impact on the Florida keys in September of 2017. The Florida Pigeon Key is home to our Generation IX Leadership program and has been held there the past years. This January, we attended the program and GutMonkey’s team taught us as leaders there are many ways we can be drawn to service work. Sometimes we are compelled by an experience like Hurricane Irma, however we have



choices and so by accepting to become service leaders we are courageous enough to offer help, ask where our skills can be utilized and step up to reflect our commitment to doing work that may sometimes make us uncomfortable.

On Pigeon Key, we rebuilt the fire pit which had been destroyed by Hurricane Irma. We first had to remove large rocks and slabs of concrete. After we had removed all the rocks we started to dig a 10-foot circle to the depths of 3 feet. Once all the sand was out we started to line the hole with the rocks and slabs of concrete to keep the shape of the fire pit. This helped us put our service leadership in practice.

We spent time networking and creating connections amongst the Pigeon Key staff, the GutMonkey team and the Gen IX participants. There were times when we as a group felt uncomfortable doing certain work however, we learned to step-up by showing we are willing to do the dirty work without the reward and learning as leaders to step aside to create space for new ideas, emerging leaders, and celebrating others. Overall the Generation IX Pigeon Key Leadership Program was a great learning experience and I hope to use the skills I learned in Service Leadership to further give back in my community.
~ Shad

Being a new member in such a close knit community can be a little scary, but I also believe it can be detrimental in some ways if you don't put yourself out there to learn valuable information when it personally pertains to you and your children. So this year, as a new mom of a child with hemophilia, I decided to attend the Generation IX Program with GutMonkey on Pigeon Key. This experience was so amazing and a fantastic way to start the new year – and not just because I had freedom from my children for six days! I didn't just gain more knowledge and insight than I had prior to the event – I also gained experience, perspective, friendship and leadership skills.

This program gave me the opportunity to meet other strong and amazing people with hemophilia, both men and women, from all over the country that I would probably never have had the opportunity to cross paths with. I assure you that after those six fun filled days in the Florida Keys, I not only became a better mom to my child with hemophilia, but I also became a better leader and advocate for myself, my children and the bleeding disorders community. I highly encourage everyone to attend a GenIX program and I am already looking forward to future Generation IX events!
~ Emili

TAEKWONDO

BY TRISTAN MARTIN - 11 years-old

Taekwondo has a positive effect on my life in the following ways:

FOCUS:
Since I've started Taekondo (TKY), I am more focused when I do my homework. In school, I found that I pay more attention to the teachers too.

ENTHUSIASM:
Being part of the TKD family inspires me to help Children's of Alabama by participating in a fundraising activity and the Miracles Board Break-A-Thon. Children's of Alabama holds a special meaning for me. I was born with severe hemophilia B. That's a bleeding disorder that impacts the clotting of my blood. I don't clot like a normal child unless I get this special medicine through my port, which is under my skin (like a cancer patient). I go to Children's Hospital at least once a year for my annual check-up - I get a blood test, see the hematologist, a dentist, a physical therapist, a social worker and a nutritionist. I get to experience first-hand the hard work of our dollars from our fundraising. What we do in TKD is important, we do make a difference in the lives of children.

GOAL SETTINGS:
TKD teaches me about short term and long term goal setting. For example, a short term goal is working on the next technique, working on the forms, practicing 1-step sparring and getting the testing tip. A long term goal is getting to the next belt. TKD is teaching me that to grow, I must set goals and work hard towards them. It's not always easy, but that's what makes it even better when you achieve the goals you set for yourself.

COOPERATION:
TKD teaches me to make friends with people outside of my school or church or my neighborhood. Black belt preparation is the ultimate cooperation so far because I get to practice choreography with a partner, which is super fun.

SELF-CONTROL:
Self-control is doing the right thing, doing something good without being asked and doing it right. TKD teaches me the basis for saying *Yes, sir, Yes, ma'am*. It teaches me to stay at *attention* and not fidget.

PERSEVERANCE:
TKD helps me to never give up. Some days I don't want to go to practice, but I go anyway because I know I want to reach my goal, be reliable and not let down my partner down, and have fun.

CONFIDENCE:
TKD gives me confidence in myself. I look at my little sister and it reminds me of when I first started in TKD and how I didn't know the moves and how much I relied on my instructor. I now have confidence to show my sister and others what the moves are, answer the questions they might ask, and help if I see that their technique or posture needs to be corrected. We must always do our best.

RESPECT:
TKD constantly reminds me to not only respect our master, instructors and peers, but also our parents, grandparents, siblings, pastor, teachers, doctors, nurses, police officers and others in the community.

RESPONSIBILITY:
TKD teaches me to make sure my TKD bag has my uniform, my belt and my protective gear. It is my responsibility, not my parents' responsibility. I need to better use this lesson and apply it toward my school papers, homework and my percussion instrument so I don't accidently leave them at home.

LEADERSHIP:
TKD teaches me that in order to become a good leader, one must have all the above attributes, practice them daily and teach them to others.

This information is based on Marcus' experience. Different patients may have different results. Talk to your doctor about whether IXINITY® may be right for you.

INDICATIONS AND IMPORTANT SAFETY INFORMATION

What is IXINITY®?

IXINITY [coagulation factor IX (recombinant)] is a medicine used to replace clotting factor (factor IX) that is missing in adults and children at least 12 years of age with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Hemophilia B is an inherited bleeding disorder that prevents clotting. Your healthcare provider may give you IXINITY to control and prevent bleeding episodes or when you have surgery.

IXINITY is not indicated for induction of immune tolerance in patients with hemophilia B.

IMPORTANT SAFETY INFORMATION for IXINITY®

- You should not use IXINITY if you are allergic to hamsters or any ingredients in IXINITY.
- You should tell your healthcare provider if you have or have had 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.
- You can experience an allergic reaction to IXINITY. Contact your healthcare provider or get emergency treatment right away if you develop 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 IXINITY. An inhibitor is part of the body's defense system. If you develop inhibitors, it may prevent IXINITY from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for development of inhibitors to IXINITY.
- If you have risk factors for developing blood clots, the use of IXINITY may increase the risk of abnormal blood clots.
- Call your healthcare provider right away about any side effects that bother you or do not go away, or if your bleeding does not stop after taking IXINITY.
- The most common side effect that was reported with IXINITY during clinical trials was headache.
- These are not all the side effects possible with IXINITY. You can ask your healthcare provider for information that is written for healthcare professionals.

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

Please see accompanying brief summary of Prescribing Information on next page.

Aptevo™ Therapeutics

Aptevo BioTherapeutics LLC, Berwyn, PA 19312

IXINITY® [coagulation factor IX (recombinant)] and any and all Aptevo BioTherapeutics LLC brand, product, service and feature names, logos, and slogans are trademarks or registered trademarks of Aptevo BioTherapeutics LLC in the United States and/or other countries.

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IXINITY®
coagulation factor IX
(recombinant)

Factoring in your world™

IXINITY® [coagulation factor IX (recombinant)]

Brief Summary for the Patient

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.IXINITY.com.

Please read this Patient Information carefully before using IXINITY. This brief summary does not take the place of talking with your healthcare provider, and it does not include all of the important information about IXINITY.

What is IXINITY?

IXINITY 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 clotting. Your healthcare provider may give you IXINITY when you have surgery.

IXINITY is not indicated for induction of immune tolerance in patients with hemophilia B.

Who should not use IXINITY?

You should not use IXINITY if you:

- Are allergic to hamsters
- Are allergic to any ingredients in IXINITY

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

What should I tell my healthcare provider before using IXINITY?

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

How should I infuse IXINITY?

IXINITY is given directly into the bloodstream. IXINITY 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 IXINITY by themselves or with the help of a family member.

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

Your healthcare provider will tell you how much IXINITY 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 IXINITY to be sure that your blood level of factor IX is high enough to stop the bleeding. Call your healthcare provider right away if your bleeding does not stop after taking IXINITY.

What are the possible side effects of IXINITY?

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

- Rash
- Hives
- Itching
- Tightness of the throat
- Chest pain or tightness
- Difficulty breathing
- Lightheadedness
- Dizziness
- Nausea
- Fainting

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

The most common side effect of IXINITY in clinical trials was headache.

These are not all of the possible side effects of IXINITY. You can ask your healthcare provider for information that is written for healthcare professionals.

Call your healthcare provider for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.

How should I store IXINITY?

250 IU strength only; store at 2 to 8°C (36 to 46°F). Do not freeze.

500, 1000, 1500, 2000 and 3000 IU strengths; store at 2 to 25°C (36 to 77°F). Do not freeze.

Do not use IXINITY after the expiration date printed on the label. Throw away any unused IXINITY and diluents after it reaches this date.

Reconstituted product (after mixing dry product with Sterile Water for Injection) must be used within 3 hours and cannot be stored or refrigerated. Discard any IXINITY left in the vial at the end of your infusion.

After reconstitution of the lyophilized powder, all dosage strengths should yield a clear, colorless solution without visible particles. Discard if visible particulate matter or discoloration is observed.

What else should I know about IXINITY?

Your body may form inhibitors to factor IX. An inhibitor is part of the body's immune system. If you form inhibitors, it may stop IXINITY from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests to check for the development of inhibitors to factor IX. Consult your doctor promptly if bleeding is not controlled with IXINITY as expected.

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

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

Aptevo™ Therapeutics

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Aptevo BioTherapeutics LLC
Berwyn PA, 19312
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Part No: 1000973_1
CM-FIX-0078

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Pfizer Hemophilia

WHEN INSURANCE BECOMES A CHALLENGE

At first, when Laura received letters from her health insurance company asking for the weights of her three sons, Billy, Adam and Josh, who have severe hemophilia B, she did not find it suspicious, and she responded to their requests. However, when she told her doctor and pharmacist about the requests, they told Laura these requests were strange.

Shortly thereafter, the insurance company that has been supplying the factor for Laura's boys for over a year sent her a letter, which said that they would be lowering the factor dosages. They claimed the dosages the boys were getting were not in compliance with the factor package insert guidelines. When Laura's pharmacy preauthorized the prescriptions in the dosages they had been receiving, they were denied. Instead, the insurance company altered the scripts and cut the doses for all three boys by 80 percent.

This couldn't have come at a worse time since the family's hematologist was actually talking about raising the dosage for Laura's youngest son, Josh, who was experiencing nosebleeds. Laura was adamant to get answers from the insurance company as to why they were unlawfully changing the scripts for her family. She called them continuously and was sent from one department to another, only to having them repeat the same answer they had sent in the letter – that the dosages they had been filling for over a year were now considered by them as "experimental" and therefore not covered. After some time, the insurance company stopped taking Laura's calls altogether.

Laura had to take off from work to attend to this sudden change within her family and deal with the insurance company. Meanwhile, Josh developed a bleed in his knee, and all three boys began having daily nosebleeds, sometimes lasting hours. One night, Laura woke to find Adam in the shower with his nose bleeding profusely. "There was so much blood in the tub, it looked like there had just been a massacre," she said. Shortly thereafter, Billy developed a bleed in his elbow. Josh's knee bleed became worse and he had to use crutches. With the kids having consistent nosebleeds, they felt weak and sick because of the blood loss.

While all this was going on, Laura felt overwhelmed and alone. Living in rural Oregon, her family was a six-hour drive from their hematologist. They had to visit the emergency room frequently and bring their own factor, because the hospital did not carry it. Laura reached out to her local governmental insurance commission and they carried out several reviews of her family's situation. However after each review, they sided with the insurance company and no relief seemed in sight.

Finally, after six months Laura found someone in the insurance commission office who cared. Together, they insisted the insurance commission involve the family's hematologist in the review process. This time, after the hematologist shared all the family's records with the commission, they came out in favor of Laura and her boys. In fact, they actually recommended the doses for all three boys be increased.

When Laura reflects on the trying times she experienced not being able to prevent her kids from constant bleeds and the repercussions that came with them, she still feels afraid if such a situation should repeat itself. Since her sons began receiving their properly prescribed dosages, all the bleeds have stopped. The insurance company however, still tries to alter the scripts every now and then. When asked why she doesn't switch to a different health insurance company, she was quick to reply, "Why? So they could do the same thing to another poor family?" Additionally she stated, "The insurance company would love it if we left. As a family, we cost them millions of dollars each year."

From her story, Laura wants others in the bleeding disorders community to come away with the understanding that they should always stand up for themselves. "Don't back off!" she says. "Persistence in fighting for your rights will eventually pay off."

AIDS GROVE DEDICATES NEW SECTION TO HEMOPHILIACS KILLED BY THE DISEASE

By John King Sep 16, 2017|San Francisco Chronical
Article link: http://m.sfgate.com/bayarea/article/AIDS-Grove-dedicates-new-section-to-hemophiliacs-12203299.php#photo-1809232?cmpid=email-mobile

There's a new place of solace within the National AIDS Memorial Grove: a memorial circle devoted to victims of the disease who were hemophiliacs.

About 300 people attended a ceremony Saturday morning in Golden Gate Park to dedicate the space, which has several benches inscribed with the names of victims, most of whom died between 1983 and the mid-'90s. Shrubs were planted along the circle and then family members read the names of victims, each name accompanied by the ringing of a Tibetan bell.
The victims had been infected in the early 1980s by the injection of blood-clotting products for hemophiliacs that were contaminated by plasma tainted with HIV.

The best-known victim was Ryan White, an Indiana middle-school student who at first was banned from his school after being diagnosed. His case received international attention and the teenager eventually was befriended by celebrities including President Ronald Reagan, basketball star Kareem Abdul-Jabbar and singer Elton John.

"It took the innocence of a young boy to begin to shake the bigotry and the hatred" directed by many people toward many of the men, women and children affected by the disease, said John Cunningham, executive director of the National AIDS Memorial Grove. An estimated 10,000 hemophiliacs eventually died, Cunningham said.

The grove's first redwood was planted in 1991, and it was declared a national memorial in 1996. There are several circles of contemplation, but this is the first one dedicated to a specific set of victims.

National AIDS Memorial Grove volunteer, Jesse Dimond arranges flowers while preparing for the December 1st 20th anniversary of the Grove. In Golden Gate Park, Nov. 28, 2011.

"The hemophiliac victims are such a unique community, in that they were affected by the tainted blood supply," Cunningham said. "It's important that we all unite" in overall remembrance of the roughly 750,000 Americans who are estimated to have died of AIDS.

John King is a San Francisco Chronicle staff writer.
Email: jking@sfchronicle.com
Twitter: johnkingsfchron

A COMMUNITY OF CARE

BY JILL LATHROP

In June 2016, my son suffered a psychotic break resulting in an inpatient hospitalization. As I sat on the floor in the hallway of the emergency department, I felt helpless, hopeless and terrified; the feeling was terribly familiar. I was taken back to our very first emergency room visit for an infusion of clotting factor. I remember feeling overwhelmed, even leaving the room as the nurses struggled to find a vein in the arms of my screaming six-week old baby. That was also the night I realized my husband and I were going to have to be our kids' strongest advocates. I couldn't run away from hemophilia; I certainly couldn't run away from my 18-year old's psychosis.

Looking back, I can see there were many subtle things that led up to my son's psychosis. We noticed changes in his routine, but attributed them to excitement and stress surrounding his high school graduation.

The morning I took my son to the hospital began uneventfully. I think I asked him if he had breakfast and his response was along the lines of "I don't need to eat anymore." Something about the way he made this statement made me stop and really look at him. He was disheveled and a little disoriented…something was definitely off. Reassuring my son I wanted to help him and that I thought he needed to see a doctor, we headed for the hospital.

The decision to take him to the emergency department at our local hospital turned into a 6-day stay in the emergency room and another 4 days in a psychiatric unit 200 miles from our home. Despite our efforts to explain hemophilia, our son injured himself early in his stay. We had difficulty accessing medical attention for the hemophilia related injury. We were also dismayed to learn virtually all the mental health facilities available to help my son refused to accept him because of his bleeding disorder.

Once our hematologist was notified, he was able to assist in finding an suitable placement. An appropriate course of treatment was initiated and my son was discharged 3 days later. His recovery from psychosis hasn't been easy; however, the tools that helped us navigate hemophilia are the same tools we're relying upon now.

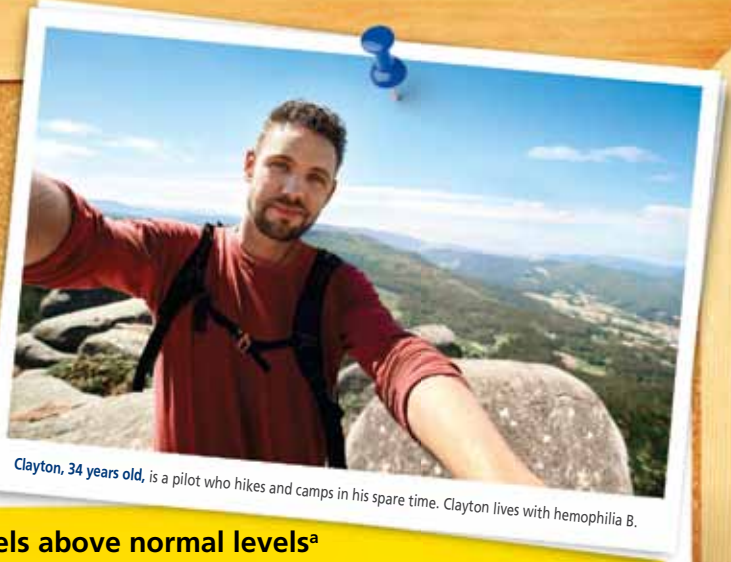
When I needed help 20 years ago, the bleeding disorders community stood shoulder to shoulder with me, encouraging and teaching me. 20 years later, my blood brothers and sisters have been a source of comfort and encouragement through this leg of our journey.

Truly, though, if you're reading this, you're stronger than you know and you are not alone.

In hemophilia B

TAKE CONTROL TO A HIGH LEVEL WITH REBINYN®

NOW AVAILABLE



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 ≤2% FIX levels[a]

[a]In 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.

[b]Based 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

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rebinyn®
Coagulation Factor IX (Recombinant), GlycoPEGylated

rebinyn®

Coagulation Factor IX (Recombinant), GlycoPEGylated

Brief Summary Information about:

REBINYN® Coagulation Factor IX (Recombinant), GlycoPEGylated

Rx Only

This information is not comprehensive.

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

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

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

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

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

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

What is REBINYN®?

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

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

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

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

Who should not use REBINYN®?

You should not use REBINYN® if you

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

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

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

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

You should tell your healthcare provider if you

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

How should I use REBINYN®?

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

REBINYN® is given as an infusion into the vein.

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

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

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

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

Use in children

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

If you forget to use REBINYN®

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

If you stop using REBINYN®

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

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

What if I take too much REBINYN®?

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

What are the possible side effects of REBINYN®?

Common Side Effects Include:

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

Other Possible Side Effects:

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

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

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

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

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

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

What are the REBINYN® dosage strengths?

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

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

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

How should I store REBINYN®?

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

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

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

If you choose to store REBINYN® at room temperature:

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

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

After Reconstitution:

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

The reconstituted REBINYN® should be used immediately.

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

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

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

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

More detailed information is available upon request.

Available by prescription only.

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

Revised: 11/2017

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

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

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

For information about REBINYN® contact:
Novo Nordisk Inc.
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novo nordisk®

AFTER HURRICANE MARIA

PUERTO RICO HUMANITARIAN MISSION

On September 20, 2017, Hurricane Maria, a powerful Category 4 hurricane with winds in excess of 15O mph made direct landfall on Puerto Rico. From south to north, with drenching rain, storm surges and devastating winds, the storm ripped through the island resulting in complete power outage, total loss of communications and no running water.

To aid in the humanitarian efforts for hurricane ravaged Puerto Rico, The Coalition received over $35,000 of medical supplies in just 8 days from community members and organizations across the USA. Bill Patsakos departed on October 8th to deliver the much-needed supplies to the Puerto Rico Chapter of NHF.

BY KIM PHELAN

In October, William Patsakos - a father of 4 sons, 3 with hemophilia B, a pharmacist and an FDNY Lieutenant began his two-week journey to deliver much needed relief to the bleeding disorder community of Puerto Rico.

Before his departure, a conference call was had,which included William and myself, Puerto Rico HTC's Dr. Enid Riviera, Dr. Van Heise, Dr. Peter Kourides, Erik Iglwski of Rochester Regional, Joe Pugliese of Hemophilia Alliance, Dominick Dellamarco of the New York Blood Center, Dawn Rotellini of NHF, Dr. Christopher Walsh and Susan Miller both of Mt. Sinai Hospital, Laurie Kelley of Kelley Communications, Linda Mugford of Hemophilia Association of New York (HANY), Brett Spitale of NHF, Mariam Voutsis with The Consortium, Kathryn McLaughlin of HRSA, Becky Dudley of American and Thrombosis and Hemostasis Network (ATHN), Dr. David De Angel Sola, and many additional bleeding disorder treatment center, chapter and organization representatives – all working together to assist the people in Puerto Rico.

From medical supplies to beneficial funding, generosity came in many forms from many HTCs, hemophilia organizations, homecare companies throughout the bleeding disorders community. Over $35,000 of supplies were donated and sent to the Coalition for Hemophilia B for William to take to assist the people in Puerto Rico.

In addition to collecting medical supplies, The Coalition donated monies to several relief organizations. The Hemophilia

Alliance donated monies to HFA's (Hemophilia Federation of America) Disaster Relief for proper allocation. Funds donated to HFA's program were administered by their Helping Hands team. Many Puerto Rican bleeding disorder community members have come to stay with family in the continental United States on a permanent basis or until the situation in Puerto Rico improves. These donated funds also assisted the families in these circumstances; and NHF and HFA are matching donations while the Hemophilia Association of New York is standing by to help relocated families. HFA continues to accept donations for this worthy cause on their website.

The outstanding response from pharmaceutical companies has also been impressive: Aptevo has made available 10 percent of the units they sell for access by US-based HTC blood banks managed by AmeriCares. Bioverativ delivered emergency supplies to hemophilia patients through Direct Relief and sent a two-week supply of factor for 70 hemophilia patients. CSL Behring provided coagulation therapies while Novo Nordisk donated money and life-saving medicine through AmeriCares. Pfizer activated a comprehensive and coordinated plan at a company-wide level to provide cash donations through the Pfizer Foundation. They shipped thousands of factor doses through Direct Relief and AmeriCares, a special one-to-one match for donations made by Pfizer colleagues, activated disaster relief protocols for its Patient Assistance Program waiving financial documentation requirements and allowing existing patients early refills and created voluntary events across the company for colleagues to assemble hygiene kits for people in hurricane affected areas. Shire donated financially to hurricane-related medical relief efforts through their NGO partners Direct relief and AmeriCares, donating over $155K, which includes matching their employees' $55,000 generous donation.

This is a small list of a lot of very good people from all organizations doing their part! There are many more wonderful people from great organizations not listed here working just as hard! The tireless efforts of our community and their giving of time and resources is outstanding. It is times like this we are able to see how fortunate we are to be part such a strong community.

PUERTO RICO HUMANITARIAN MISSION A RECAP

BY WILLIAM PATSAKOS

Puerto Rico's Governor Ricardo Rosselló said, "Make no mistake - this is a humanitarian disaster involving 3.4 million US citizens."

San Juan Mayor, Carmen Yulin Cruz proclaimed, "Help us! Without a robust and consistent help, we will die."

The official death toll of Hurricane Maria is 64, though within 42 days following the hurricane, 1052 more lives than usual life were claimed* with many more at risk due to disease, lack of infrastructure and access to basic necessities and medical care and medicines.

The tragedy in Puerto Rico has deeply affected many Americans both on the island and in the continental United States. In a 2012 census, there are approximately 5.4 million Puerto Ricans living in the US and approximately 120,000 veterans and many living in the NYC area. I am one of those American citizens with family living in Puerto Rico.

I am writing this article to detail the conditions encountered during my humanitarian trip to Puerto Rico and to plead for continued support from our fellow citizens by personal volunteerism, both monetarily and with reconstruction aid workers. I want to convey messages from residents, first responders, and members of the medical community. I am an Army Veteran Medical Service Corp officer, CVS Pharmacist, and FDNY Lieutenant. In addition, I have 3 sons with hemophilia. I have worked in Puerto Rico with the bleeding disorder community in the past and have a connection to patients in hematology and oncology, their families, and members of the medical community. The hurricane generated great concern from the bleeding disorder community to act immediately and decisively. I want to highlight several groups that acted swiftly and generously to serve the people of Puerto Rico and share my profound gratitude and respect for the following groups:

- New York City Fire Department Incident Management Team (Commissioner Nigro and Chief of Department James Leonard)
- NYC Urban Search and Rescue Task Force (Fire and Police Departments)
- The FDNY Disaster Assistance Response Team
- Puerto Rico Fire and Police Departments
- Centro Medico Medical Center P.R. HTC and Mount Sinai HTC-Zone 2(Dr.Rivera, Dr. Walsh, Susan Miller and staff)
- CVS Heath Specialty Pharmacy (Nicole Jackson P.R. and Joel Helle)
- The Coalition for Hemophilia B (Kim Phelan)
- National Hemophilia Foundation (Val Bias, Puerto Rico chapter and NYC chapter Jeremy Griffin)
- Hemophilia Federation of America (Kimberly Haugstad)
- LA Kelley Communications and "Save one Life" (Laurie Kelley)
- Heart 911 (Numerous members of the first responder community)

There have been so many other agencies local and federal under FEMA and the military that have worked tirelessly to rescue people and clear roads for access, restore power and communications, deliver supplies and medicine and volunteered.

It is important to note that prior to Hurricane Maria, the economic situation on Puerto Rico was dire. Prior to the hurricane, Puerto Rico filed for the equivalent of federal bankruptcy protection in May 2017, for 74 billion in debt and 53 billion in unfunded pensions. Maria has escalated Puerto Rico's financial chaos and damage with estimates ranging from 40 to 80 billion. The electric grid, and water and sewer infrastructure were experiencing daily interruptions in service.

The focus has been initially to restore power and communication capabilities to critical facilities - firehouses, police stations and hospitals. I have been able to assist and coordinate medical shipments to 4 strategic pods located around the island and link up with medical volunteers to triage and deliver care to rural areas that are heavily affected.

We have been able to visit patients that doctors were concerned about and deliver medications. To date, we were able to collect medical supplies, surgical supplies and over the counter meds and approximately $350,000 in factor product and injectable drugs, refrigeration, and storage. We have been able to assist major distributors and pharmacies in gaining assistance from FEMA for fuel and delivery logistics. In addition we have communicated needs to volunteer organizations in obtaining

tarps for temporary roofing and, in some cases, replace roofs of some residences.

We visited many hospitals and in particular Centro Medico San Juan University, which is the major medical institution on the island. I had the opportunity to round with attending physicians and department head, Dr. Enid Rivera, and CVS territory executive, Nicole Jackson. We worked with CVS Health Specialty to assist pharmacists with shipments for all complicated and chronic disease states. There is a rare genetic disease that afflicts Puerto Ricans in greater incidence than any other population in the world. This condition is known as Hermanski-Pudlak syndrome which is an oculocutaneous albinism with bleeding problems and platelet abnormalities. There are approximately 100 hemophiliacs and another 150 von Willebrand's Disease patients. These and other oncology patients rely on continuous infusions and access, which is complicated by no electricity or clean running water.

Together with Lilly Gomez and Nicole Jackson, we are compiling testimonials and patient stories to share with the international community for the purpose of awareness and fundraising, and will bring to you in a future article. I would like to share 3 such cases we encountered and, with their permission, share their stories and pictures in order to raise awareness and funds to help Puerto Ricans in their hour of greatest need.

Our first is Osman - a beautiful ten month old baby boy with hemophilia A. His 21-year-old mother and grandmother are bleeders, but were never officially diagnosed. Osman has a newborn baby sister, who has been diagnosed with a bleeding disorder. Osman's mom, Maria, had to stop working as a security guard since she is frequently taking her kids to doctor's appointments. The children's father currently works daily12-hour shifts, also as a security guard, to provide at least the basic needs for the family. During the hurricane, their house was completely flooded. All their furniture, clothes, and babies articles were damaged, and three weeks after the hurricane, only had water a couple of hours a day with no electric power. Maria had been struggling to find access to the medication Amicar. The pharmacies contracted with Medicaid didn't have it available. Maria was very thankful she would receive it as part of donations to the Pediatric Hospital. It's heartbreaking how Osman's mom says they are okay while everything is going so bad for them.

We also met Joanne, a 20-year-old that had been diagnosed with severe Factor 5 deficiency (with a prevalence of about one in a million) since she was 11-months-old. She was being infused factor while we listened to her story. Before the hurricane, Joanne lived with her stepmother, aunt, uncle and grandmother in Ponce. Since their house was near the beach, they were evacuated from their home the day before the hurricane. They stayed in a refuge center for 5 days before they were able to return home. They found their home completely flooded. Her cousin's home just near hers was completely destroyed. Now 8 members of the family had to stay in a space of a living room/bedroom with only 2 beds, no water and no electric power.

Joanne sleeps in one bed with her grandmother, who had open heart surgery, is insulin dependent and has asthma. They have a very small generator to refrigerate the insulin, but with the gas shortage, they have to wake at 4 am to wait in line to buy gas. It's been very difficult to purchase drinking water, and in their area, there is almost no food available in stores.

Communications are very difficult, which worries Joanne in the event of a health emergency. She tried to smile, but seemed very sad and hopeless. Receiving their blessings for our help, we left Dr. Rivera, Dr. Amy Lee Cabrera (Hema-Oncology fellow), and Yue Sai Jao (4th year med student), and traveled to the town of Canovanas looking for what used to be the home of Victoria Pereira, a patient who left the island and is currently receiving help from the HFA. As we arrived to the address provided, it was sad to see the house completely destroyed.

Hurricane Maria was a very powerful hurricane that left no part of the island untouched. The people of Puerto Rico are very strong, resilient and with tremendous dignity. They are Americans living in third world conditions. We must work to restore Puerto Rico's infrastructure while simultaneously assisting their economy and jobs, or the island will face a mass exodus to the continental U.S. Since Hurricane Maria, 40,000 people have moved from the island

Please donate your time and money. If asked to help, respond with "I am here!"

* https://www.nytimes.com/interactive/2017/12/08/us/puerto-rico-hurricane-maria-death-toll.html

INDUSTRY NEWS

BY DR. DAVID CLARK

Alnylam Receives FDA Approval to Re-Start Clinical Studies

Alnylam Pharmaceuticals has received FDA approval to resume its clinical studies of fitusiran for treatment of hemophilia A and B. The studies were put on hold earlier after the death of a subject. The death was later judged to not have been caused by fitusiran but rather to a misdiagnosis that lead to dosing the hemophilia A subject with high doses of factor VIII. That apparently lead to fatal blood clots in his brain. Alnylam will resume the clinical study including new risk-mitigation measures to better control the concurrent use of other hemophilia medications.

The fitusiran death and the death of a subject in a trial of Hemlibra, a hemophilia A drug that was recently licensed, show that the community needs to develop a new awareness of the potential dangers when manipulating the clotting system. Many of the new hemophilia drugs being developed are not just new versions of factor VIII or IX. They exploit other parts of the clotting system to try to shift the balance back toward being able to efficiently form clots. In doing so, they are potentially interfering with the regulation and control naturally occurring in the system, which can lead to thrombosis, dangerous unneeded clotting. Products like factor VIII and IX concentrates and inhibitor-bypassing agents that seem very safe in normal use can have unexpected consequences when other parts of the clotting system have been modified.

Alnylam Restructures Agreement with Sanofi Genzyme

Alnylam has restructured their development agreement with Sanofi Genzyme for fitusiran. Under the new agreement, Sanofi Genzyme will take over all development activities and conduct of the clinical studies. Sanofi will have global commercialization rights. This is the next step in Sanofi becoming a major hemophilia treatment organization. They recently also obtained FDA approval of Hemlibra, an innovative treatment for hemophilia A.

Bioverativ Receives Updated Labeling Approval for Alprolix

Bioverativ continues to obtain clinical data to support the use of Alprolix in treatment of hemophilia B. FDA has approved updated labeling including addition of pediatric data showing that treatment with Alprolix results in effective bleeding protection with extended dosing intervals. The new labeling includes additional clinical trial experience in 93 subjects treated prophylactically for more than two years. The new data shows median spontaneous annualized bleeding rates of zero for children and 1.04 for adults and adolescents when treated weekly.

Bioverativ Presents New Clinical Data for 14-Day Dosing with Alprolix

At the recent American Society of Hematology (ASH) meeting, Bioverativ presented additional clinical data showing that weekly, 10-day or even 14-day or longer dosing with Alprolix can deliver optimal protection against bleeds. Study participants who received Alprolix prophylactically every 14 days or longer had a median spontaneous annualized bleeding rate of 0.7 over three years of observation.

Bioverativ Presents Data on the Tissue Distribution of Alprolix

Bioverativ in collaboration with Invicro, LLC, a leading imaging company, performed a study of the distribution of Alprolix and other factor IX products in the body. Normally, only about half of the factor IX made in the liver or infused by a patient remains in the blood stream. The other half binds to the endothelial cells that line the blood vessels or distributes into the extravascular space, the space in the body outside the blood vessels. One idea is that this provides a reservoir of extra factor IX in case of severe bleeding. One concern is whether the longer-acting factor IXs will also perform the same way.

Bioverativ and Invicro radio-labeled Alprolix, a conventional factor IX product and a glycol-pegylated longer-acting factor IX and infused them into animals. (Radio-labeling is attaching a radioactive tag to a molecule. The radioactivity can then be detected by imaging to determine where the labeled compound is in the body.) The results indicate that Alprolix showed the greatest distribution to the extravascular spaces, including the joints. The conventional factor IX was next, and the glyco-pegylated product showed the least amount. The meaning of these results for hemophilia treatment and joint issues is currently unknown, but Bioverativ plans to do more work in this area.

Catalyst Presents Data from Phase I/II Trial of Variant Factor IX

Catalyst Biosciences announced promising results from their Phase I/II trial of their factor IX variant CB 2679d. CB 2679d is a variant of normal factor IX that has only three different amino acids in its structure, but demonstrates much higher activity and longer half-life than normal factor IX. The clinical results show that CB 2679d exhibits a half-life almost five times the half-life of normal factor IX when dosed subcutaneously once-daily. The Phase I/II study is expected to be completed in the first quarter of 2018.

Catalyst Starts Phase II/III Study of Variant Factor VIIa

Catalyst Biosciences is initiating a Phase II/III study of their variant form of factor VIIa for treatment of hemophilia A and B patients with inhibitors. Factor VIIa is commonly used to treat inhibitor patients because it can bypass the step in the clotting cascade that involves factors VIII and IX. However, the current product has a relatively short half-life and therefore must often be administered once or more a day. Like Catalyst's variant factor IX above, the variant FVIIa, called marzeptacog alfa (activated) or MarzAA, has only small changes to its structure, but those changes have large effects on potency and half-life. It is also administered subcutaneously. In the Phase I study MarzAA showed a six- to nine-fold improvement in potency and half-life compared to the current FVIIa product with excellent safety and tolerability.

Seattle Researchers Engineer B Cells to Secrete Factor IX for Gene Therapy

Researchers at the Seattle Children's Research Institute (SCRI) are developing a method to insert factor IX genes into B cells for production of factor IX. B cells, also known as B lymphocytes, are part of the immune system. They are white blood cells that circulate in the bloodstream and secrete antibodies. Previous attempts to genetically engineer B cells to secrete proteins failed to produce enough expression for adequate gene therapy. The SCRI scientists used CRISPR/Cas9 methods to edit the B cells' genomes to introduce the factor IX gene in a specific location and to cause the B cells to mature into a specific type known as plasma cells. Plasma cells reside for long periods, even decades, in the bone marrow where they normally secrete antibodies, but could potentially secrete proteins like factor IX.

Spark and Pfizer Announce Results from Gene Therapy Study

Spark Therapeutics announced promising results for their hemophilia B gene therapy treatment, SPK 9001. The ten patients who took part in the Phase I/II study achieved an average factor IX level of 34% of normal (range 15 - 78%). The annualized bleeding rate of the group fell from about 11.0 before treatment to 0.4 afterward. Eight of the patients were able to completely stop prophylaxis with clotting factor. There were no severe adverse events, although two patients had temporary increases in liver enzymes that were resolved with corticosteroids.

Meanwhile, in November, Spark and Pfizer announced that they had amended their license agreement for SPK-9001. They have modified the manufacturing process and are transferring it to a Pfizer facility for future production. They will continue the Phase I/II study with up to five additional patients using product made by the new method for comparability. Pfizer will take over development of the product and perform a Phase III study.

Tremeau Developing Pain Treatment for Hemophilic Arthropathy

Tremeau Pharmaceuticals, a new pharmaceutical company focused on developing non-opioid pain treatments for rare diseases, has been granted orphan drug designation for their TRM-201 under development for treatment of hemophilic arthropathy (joint disease). TRM-201 is rofecoxib, which was previously marketed by Merck & Co. as Vioxx. Rofecoxib is a COX-2 non-steroidal anti-inflamatory drug (NSAID), which reportedly has no effect on bleeding times and a reduced risk of gastrointestinal bleeding. Vioxx was voluntarily taken off the market by Merck in 2004 after studies showed that it increased the risk of cardiovascular events, such as heart attacks and strokes. Tremeau believes that the risks can be adequately managed within small specific indications like hemophilic arthropathy and other niche applications. TRM-201 is currently planning a Phase III clinical study.

uniQure Presents Gene Therapy Data for AMT-061

uniQure has replaced the conventional factor IX gene in its AMT-060 gene therapy treatment with the highly-active FIX-Padua gene, hoping to achieve higher factor levels in patients. The new treatment is designated AMT-061. FIX-Padua is a variant of factor IX that is approximately eight times more active than conventional factor IX. In studies in non-human primates, treatment with AMT-061 produced about 6.5 times higher factor IX levels than treatment with AMT-060. Safety and toxicity measures showed no significant difference between the two. Both FDA and the European Union have tentatively given uniQure approval to move forward substituting AMT 061 in the ongoing clinical studies. uniQure is currently preparing the IND amendment for the study, which it plans to submit to FDA in the first quarter of 2018. The pivotal Phase III study is expected to start during the third quarter of 2018.

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

He's free to infuse only once every 14 days. **Are you?**

The only FDA-approved treatment for hemophilia B with up to 14-day dosing.* **Visit us at IDELVION.com.**

14-DAY DOSING WITH HIGH LEVEL PROTECTION†
Dosing schedule that fits into your lifestyle

21% FIX LEVELS WITH PROPHYLACTIC USE
High and sustained Factor IX levels at steady state[1,†]

ZERO BLEEDS MEDIAN AsBR
Zero median annualized spontaneous bleeding rate (AsBR) when dosed at 7 or 14 days in clinical trials

Protection with peace of mind—**low incidence of side effects**

*Appropriate people 12 years and older may be eligible for 14-day dosing. Talk with your doctor.
†Average FIX levels with 7-day dosing over 92 weeks in clinical trials.

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.

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.

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 brief summary of prescribing information for IDELVION on next page.

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 ingredients in 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.

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

Based on November 2016 PI revision.

References: 1. Data on file. Available from CSL Behring as DOF IDL-002.

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

GETTIN' IN THE GAME

SWIM STYLE

BY MELISSA FRANZEN

My son, Charlie, who is 12 years old, was lucky to attend CSL Behring's *16th Annual 2017 Gettin' in the Game Junior National Championship* in Phoenix, Arizona last November. Charlie loves to swim and was excited that they added swimming to their sports activities line up this year.

Charlie has been playing club water polo for the last year and a half. To be considered for this opportunity, he submitted an essay he wrote on how much he loves swimming and water polo. In his essay, he expressed that when he's swimming, he doesn't feel as though he has hemophilia. Being in the water is his form of meditation. When he's having a bad or stressful day, getting in the water to swim, surf or playing water polo helps him refocus. He comes out of the water feeling instantly better. I am glad that he's found something to help him cope.

The Getting' in the Game fun-filled weekend included a stay in a hotel with other kids from the bleeding disorder community from all over the country. We don't live near other children with a bleeding disorder, so spending time with community families is always special to us. The weekend was packed with fun activities with a focus on swimming, golf, basketball; as well as being healthy, and ended with an award ceremony.

On the first day, time was spent going over a variety of stretches and learning about sports safety as well as going through an assortment of drills and swimming exercises. The second day was a competition day where, through a variety of games, the boys competed to see who had the best swim times, diving and endurance.

Charlie is pretty competitive, so he really enjoyed this part of the weekend. He tried his hardest, competed well and was very pleased that he came in third place. He won his first big trophy! It was too big to pack, so he had to carry it on the plane home. We are so proud of Charlie and all his accomplishments in finding his passion! Thank you, CSL Behring, for giving my son this opportunity!

UPCOMING EVENTS 2018!

MARCH 1, 2018
ETERNAL SPIRIT AWARD DINNER
New York, New York

MARCH 22, 2018
GOLF FORELIFE OUTING
Ponte Vedra Beach, Florida

MARCH 23-25, 2018
12TH ANNUAL SYMPOSIUM
Ponte Vedra Beach, Florida

APRIL 14, 2018
MEETINGS ON THE ROAD
Maine

APRIL 19-22, 2018
WOMEN'S RETREAT
Florida

MAY 3-6, 2018
MEN'S RETREAT
Florida

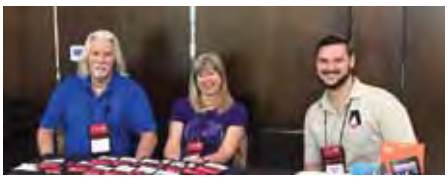
MAY 19, 2018
MEETINGS ON THE ROAD
Alabama

SEPTEMBER 13-16, 2018
MEN'S RETREAT
Arizona

SEPTEMBER 22, 2018
MEETINGS ON THE ROAD
Pennsylvania

SEPTEMBER 27-30, 2018
WOMEN'S RETREAT
Arizona

OCTOBER 6, 2018
MEETINGS ON THE ROAD
New Mexico and North Carolina



NOVEMBER 10, 2018
MEETINGS ON THE ROAD
Virginia and Colorado

NOVEMBER 17, 2018
MEETINGS ON THE ROAD
California

ForeLife

THE COALITION FOR HEMOPHILIA B

2nd Annual Golf ForeLife Outing and Fundraiser

Thursday – March 22, 2018

TPC Sawgrass; Ponte Vedra Beach, Florida

THE COALITION FOR
HEMOPHILIA B, INC.

Keynote Speaker:

Christopher Walsh, MD, PhD

Assoc. Professor Medicine, Hematology and Medical Oncology

Mt. Sinai Hospital New York, NY

Childcare Provided
Tween Program
Teen Program

12th Annual Symposium

Friday – Sunday
March 23 – 25, 2018

Sawgrass Marriott Hotel
1000 PGA Tour Blvd.
Ponte Vedra Beach, FL 32082

Attendee and Exhibitor Registration:
www.hemob.org

Registration is free for people with diagnosed with hemophilia B and their families;
$350 for healthcare professionals, industry representatives and other interested parties.

For more information, please contact Kim Phelan, 917-582-9077

BCONNECTED

THE COALITION FOR HEMOPHILIA B

YOU ASKED
WE LISTENED

COMING SOON!

PEERS | FAMILIES | EXPERTS

Kidz Korner!

Winter Word Find

Z	C	H	R	N	V	F	G	E	M	I	T	T	E	N	S	T	L
P	S	L	F	P	B	N	R	T	X	Y	M	M	M	U	O	S	U
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COATS
COLD
FROST
GLOVES
HATS
HOTCHOCOLATE
ICICLES
MITTENS
SCARF
SKATING
SLEDDING
SNOW

The Coalition For Hemophilia B

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Website: www.hemob.org
Facebook: www.facebook.com/HemophiliaB/
Twitter: https://twitter.com/coalitionhemob

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