Finding “I Am”

I am a “shemophiliac,” meaning I am a female living with hemophilia B. I am 22 years old. I am a TaijiFit instructor. I am a pole-vaulter. I am a biologist. Most importantly, however, I am. I am life and I am here. The implications of this two-word phrase “I am” are infinite. There are no limits to this simple phrase. As a human, I always tried to separate different aspects of myself. By labeling each of my identities, I put them all in boxes and stored them away, easily accessible depending on the time, place and people I was around. Some I used quite often while others remained hidden. Over the last few years however, the struggles caused by my bleeding disorder allowed my perception of the universe to expand in many shapes and directions, like the roots and branches of a tree. In growing in this way, I have been able to dust off the tops of the boxes, open them up and reconnect the pieces of myself that had been separated from each other for so long. While doing so, I realized I am not just the roots and branches of one tree, but instead a whole forest. No, not just a whole forest, but a forest surrounded by a meadow, encircling a stream and beaming with life in many different shapes and forms.
Welcome!

The Coalition for Hemophilia B would like to welcome Michael Bishop to our team! Michael joins us as our Communications Director. He recently graduated with a degree in Creative Writing from the University of Findlay in Findlay, Ohio. Michael is also a wonderful artist and we can all thank him for our new and improved website as well. We are excited to have him on our team!

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Finding I Am

By Cassie Starks

Although my "I am" has been around for many years, the journey to uncovering my true self began almost four years ago when I chose to pole vault at a collegiate level. As a woman with hemophilia, competing in sports always had several challenges on its own; from learning when to recognize and treat bleeds, to dealing with things like fatigue due to excessive monthly cycles. Apparently these things were not big enough challenges for me, because I decided to pick up a sport that involved intense training practically year-round, as well as sprinting full speed down a runway carrying a giant stick just so I can fling myself as high as possible into the air.

Pole vault began for me as a side event. I was originally a short sprinter, running mostly 100- and 200-meter sprints. Picking up a pole for the first time my junior year of high school, I only cleared 8’ 6” by the time I was headed to college. During the first year of undergraduate studies, I began to see improvements in my vault. I was dedicated, hardworking and committed to perfecting every technical aspect of this unique sport. The better I got to know the sport; the more I fell in love with it. I was able to improve my personal best to 10’ 8”; just over two feet higher than I had cleared less than a year before. This improvement did not come pain free, however. By the end of my first indoor and outdoor season, I rolled and sprained both ankles, causing severe joint bleeds that forced me to sit out for at least four to six weeks each. My second year was not any better; it involved more rolled ankles, pulled hamstrings and a lot of frustration. This ended up with me giving up the open sprints to save my body for the vault.

Each time I was injured, due to my hemophilia, I was forced to sit out for at least a month. The few weeks following that month had to be taken as slow as a turtle to make sure I did not re-injure myself, or cause another bleed. Having to sit out those months was emotionally difficult for me because it set me back greatly in the technical progress I had made in the sport. Since pole vault relies so much on muscle memory, not being able to physically vault immediately slammed the brakes on all improvements and sometimes even caused me to regress. I remembered something my coach recently told me, "Light a fire and use this as fuel for your fire" and I did. I took the energy caused by my pain and frustration and redirected it to be used as motivation. I used any spare time I had to work on any low-impact drills I could and invested some serious time in physical therapy, usually spending about two to three hours a day in the training room.

By the time I reached my third year of college, I already felt as though I had grown in many ways. After reading several books trying to discover ways to heal faster and reduce injuries, I was awakened to the importance of maintaining a healthy mind and spirit. Everything I was reading in my free time seemed to have pure synchronicity with all I was learning in my biology, chemistry and physics classes. Even though the subjects differed greatly, I was able to make many connections. I began meditating daily. I started to become aware of my self and all the negative thoughts I was allowing to run wild in my mind. Suddenly, everything around me began to fall into place and it all made perfect sense. The beauty of the universe and life itself became real to me as I fell into the present moment and began to realize the true implications of how everything was possible. These were all things I was blind to by remaining trapped in my own thoughts; however, by practicing meditation and mindfulness, I was able to become aware of these thoughts, expand the silence in between them and allow a deep sense of peace and love for myself, allowing the universe to trickle in.

As I began my third year of track I was extremely motivated and became known as what’s called a “vault-nut” someone who is very addicted to pole vaulting. I was determined to stay healthy for an entire year; however, this did not last long. At the start of the year, I pulled a hamstring, immediately causing a bleed. The physical pain of the needles from infusing and the injury itself were not even remotely close to the emotional pain and frustration I felt for not being able to work towards my goal. This time though, because of my practicing mindfulness, I was more aware of my pain. I could feel it. The more I breathed into feeling the pain rather than simply thinking about it, the more it began to change and form into a new shape - a bright red-orange flame in the pit of my stomach. I remembered to feed the fire. As I sat on the bench watching my teammates take up vault after vault, I could feel it growing and burning brightly. I allowed it to continue to grow and once again used it to take every opportunity I could to work on drills that were not too stressful on my hamstring.

By the time my leg was healed enough to step back on the runway, I was hungry to vault and hungry for success.
qualified for the NAIA indoor national meet during the third meet of the season. When March finally rolled around, I was ready to compete in the biggest meet I had ever been in. I was excited and felt ready with high hopes and expectations. I cleared every height on my first attempt up until 11’9”, which I cleared on my second attempt. During my first attempt at 11’9”, I pulled my hamstring. The meet officials told me I finished 8th place, which I was content with because top eight finishes are considered All-American. As I was receiving treatment on the training table later on, I found out that they made a mistake - my one miss from earlier put me in 9th place - one spot away from All-American status.

I did not suffer much from this defeat. Instead I used it to fuel the fire. That summer I worked harder than ever. One of my older brothers, Zane, designed a lifting program for me and trained me four days a week, making sure I knew exactly how to execute the techniques properly to prevent any future injuries. I also worked on more drills, trying to improve my technique and explosiveness in the vault. I had big goals for my senior year. Wanting to win both conference and national competitions, as well as break both the college’s indoor and outdoor school records, I knew it would take a lot of physical and mental practice. With my classes settling down, I had even more time to dedicate towards weight lifting, drills and mindfulness practices. I made it through the entire first semester without any injuries. I cleared 12’ for the first time at a practice competition while I was halfway blind from the aura of a migraine. Once January came around, I qualified for indoor nationals during the first meet of the season by clearing 11’9”.

On a visit back to my parent’s house, I decided to join in on one of my dad’s TaijiFit classes. I read quite a bit about Tai Chi and the studied health benefits, but never tried it other than maybe one or two random YouTube videos. During the session, I quickly entered what is known as the flow state and time seemed to slow down. I fell out of my thoughts and back into my body; my arms seemed to just float through the air, guided by my breath. My senses began to sharpen and I became aware of all that was around me. My mind cleared. It was as if the different streams of thoughts that are normally rushing through, twisting and tangling in multiple directions, were all at once smoothed out and merged into one gentle, steady slow-moving river. I was in the present, the now, and it was beautiful. I felt a sense of power and freedom in removing all the thoughts that were creating limitations on my mind. I fell in love. Too quickly, my first session ended. I decided to sign up for online classes to become a TaijiFit instructor. I started waking up earlier as well, just so I could attend two, sometimes three sessions a day.

The first week I began attending classes, my confidence in pole vault competition skyrocketed. I cleared a new personal best of 12’ 5.5”, earning me the number two spot in the nation. My hard work was finally starting to pay off. The following week was our conference meet and I accomplished two goals in one, both winning the competition and setting a new school record at 12’ 6.25”. I continued taking the online TaijiFit classes and felt all aspects of my life begin to unfold. My quality of life was higher and every aspect within this universe began to have meaning, and not just because of my success in the sport.

Even though I did not accomplish my goal of winning a national meet, I am still deeply grateful for my successes and all the many individuals who helped me along the way. I am deeply grateful for the journey that allowed me to overcome many challenges as a hemophiliac, as well as learning how to lead others into the flow state for the first time. I am deeply grateful for this journey that awakened my true self - the “I am” with no limitations, no negativities, no judgments or separations. This is the “I am” that I truly am, and the “I am” that is within everyone. I am light. I am love. I am peace. I am bliss. I am gratitude. I am compassion. Simply, I AM.

A failure is only a failure if you do not learn and grow from it. Following your dreams is not about success. It is not about the end result. Following your dreams is about surrendering to your heart, opening up to the willingness and strength to try.
REAL PEOPLE, REAL EXPERIENCES

"My coordinator helped me get ALPROLIX at no charge while my insurance was being processed."

- RICK  MyALPROLIX Peer™

"My MyALPROLIX™ Coordinator got me 30 days of free factor and was able to reduce my copay to zero."

- BRIAN  MyALPROLIX Peer™

These MyALPROLIX Peers are real people living with hemophilia B who want to share their experiences with ALPROLIX.

To learn more about how a MyALPROLIX Coordinator can assist you, call 1-855-MyALPROLIX (692-5776) Monday–Friday, 8 AM – 8 PM ET, or visit MyALPROLIXresources.com

INDICATIONS AND IMPORTANT SAFETY INFORMATION

Indications
ALPROLIX, Coagulation Factor IX (Recombinant), Fc Fusion Protein, is a recombinant DNA derived, coagulation factor IX concentrate indicated in adults and children with hemophilia B for:
- Control and prevention of bleeding episodes
- Perioperative management
- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes

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

Important Safety Information
Do not use ALPROLIX if you are allergic to ALPROLIX or any of the other ingredients in ALPROLIX.

Tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines, supplements, or herbal medicines, have any allergies and all your medical conditions, including if you are pregnant or planning to become pregnant, are breastfeeding, or have been told you have inhibitors (antibodies) to factor IX.

Allergic reactions may occur with ALPROLIX. Call your healthcare provider or get emergency treatment right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash, or hives.

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

ALPROLIX may increase the risk of formation of abnormal blood clots in your body, especially if you have risk factors for developing clots.

Common side effects of ALPROLIX include headache and abnormal sensation of the mouth. These are not all the possible side effects of ALPROLIX. Talk to your healthcare provider right away about any side effect that bothers you or does not go away, and if bleeding is not controlled using ALPROLIX.

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 full Prescribing Information on the next page. This information is not intended to replace discussions with your healthcare provider.

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ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein], Lyophilized Powder for Solution For Intravenous Injection.

FDA Approved Patient Information

ALPROLIX® /all' pro liks/ [Coagulation Factor IX (Recombinant), Fc Fusion Protein]

Please read this Patient Information carefully before using ALPROLIX® and each time you get a refill, as there may be new information. This Patient Information does not take the place of talking with your healthcare provider about your medical condition or your treatment.

What is ALPROLIX®?

ALPROLIX® 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.

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

Who should not use ALPROLIX®?

You should not use ALPROLIX® if you are allergic to ALPROLIX® or any of the other ingredients in ALPROLIX®. Tell your healthcare provider if you have had an allergic reaction to any Factor IX product prior to using ALPROLIX®.

What should I tell my healthcare provider before using ALPROLIX®?

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

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

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

How should I use ALPROLIX®?

ALPROLIX® should be administered as ordered by your healthcare provider. You should be trained on how to do infusions by your healthcare provider. Many people with hemophilia B learn to infuse their ALPROLIX® by themselves or with the help of a family member.

See the Instructions for Use for directions on infusing ALPROLIX®. The steps in the Instructions for Use are general guidelines for using ALPROLIX®. Always follow any specific instructions from your healthcare provider. If you are unsure of the procedure, please ask your healthcare provider. Do not use ALPROLIX® as a continuous intravenous infusion.

Contact your healthcare provider immediately if bleeding is not controlled after using ALPROLIX®.

What are the possible side effects of ALPROLIX®?

Common side effects of ALPROLIX® include headache and abnormal sensation in the mouth.

Allergic reactions may occur. Call your healthcare provider or get emergency treatment right away if you have any of the following symptoms: hives, chest tightness, wheezing, difficulty breathing, or swelling of the face.

ALPROLIX® may increase the risk of forming abnormal blood clots in your body, especially if you have risk factors for developing blood clots.

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

These are not all the possible side effects of ALPROLIX®.
Talk to your healthcare provider about any side effect that bothers you or that does not go away.

How should I store ALPROLIX®?

Store ALPROLIX® vials at 2°C to 8°C (36°F to 46°F). Do not freeze.

ALPROLIX® vials may also be stored at room temperature up to 30°C (86°F) for a single 6 month period.

If you choose to store ALPROLIX® at room temperature:

- Note on the carton the date on which the product was removed from refrigeration.
- Use the product before the end of this 6 month period or discard it.
- Do not return the product to the refrigerator.

Do not use product or diluent after the expiration date printed on the carton, vial or syringe.

After Reconstitution:

- Use the reconstituted product as soon as possible; however, you may store the reconstituted product at room temperature up to 30°C (86°F) for up to 3 hours. Protect the reconstituted product from direct sunlight. Discard any product not used within 3 hours after reconstitution.
- Do not use ALPROLIX® if the reconstituted solution is cloudy, contains particles or is not colorless.

What else should I know about ALPROLIX®?

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

Manufactured by
Biogen
14 Cambridge Center
Cambridge, MA 02142
U.S. License #1697
My name is Manuel Lopez. I'm 26 years old and have severe Factor IX deficiency. My brother, Angel Jr., and I were born in Puerto Rico, but raised in the United States with our parents Angel Lopez and Ana Serrano. My family and I lived in a beautiful small town called Utuado, just north of the center of Puerto Rico in one of the mountainous regions of the island.

When I was born, I was a normally active baby, but at nine months old my parents started to notice random bruises on my body. Concerned about my health, they visited our local pediatrician, who then referred us to Centro Médico de Puerto Rico, a treatment center in Puerto Rico. In the hospital, a team of oncologists and hematologists took on my case and it was there where I was diagnosed with severe hemophilia B. The news was heartbreaking to my parents and family. For my family, this was something new, as no one in our family had this disorder.

Before home treatment was available or even an option, we would constantly have to visit the treatment center in San Juan, which due to old and dangerous roadways, was about two hours away from home.

We were a low income family and the financial struggles became evident rapidly. We did not have a car and my father could barely work due to my bleeding episodes. Mom was my constant caretaker so that dad could work as much as possible. Family members helped when they were able and took care of my older brother while my parents and I spent long days at the hospital. There were times when we would drive three or even four times a week for my bleeding episodes; at times we spent weeks there. I still remember the times when Mom and Dad would do the impossible to get me to the hospital. There were many great people who supported us by donating money, the use of their vehicles as well as their homes; this gave us the ability to spend all the time we needed at the hospital.

So my parents would be able to take me to the hospital whenever I needed, a friend donated his car to my family after seeing us struggle so much. Even though we now had a car, I can still remember the times it broke down at two or three in the morning while coming back home from the hospital. One night, our car broke down; my father's friend, who was coming home from work, recognized us and stopped.
A Man Living with Hemophilia B and his Journey

He brought my mom, brother and I home, leaving the car behind with my father. His car was small so my father had to wait on a dangerous road until his friend went back to get him. My parents gave their all for me and I will forever be grateful.

My life, as many patients’ lives, was and is by no means, easy. I was limited in doing most things when I was younger. I was not able to play sports or be very physically active because of a bad right ankle. I was forced to use ankle braces to protect my joints. Prior to 1999, no prophylaxis treatment was available; I would have to infuse on an “on demand basis.” Being a very energetic kid, it was hard for me to adjust my life to the demands of hemophilia B and what I could or could not do to avoid bleeds and injuries.

In 1998, with the exception of our car, we lost everything during Hurricane George and moved from Puerto Rico to Erie, Pennsylvania. Once in Erie, I visited the Hemophilia Center of Western Pennsylvania in Pittsburgh, where I was offered better treatment options. Unfortunately, prior to arriving in Erie, I was exposed to Hepatitis C through previous treatment options. Thankfully, even though exposed, Hepatitis C is dormant in my body and has not caused any health concerns and I have been able to lead a normal life.

In 1999, I started using Benefix Recombinant IX to treat my Factor IX deficiency on a prophylaxis treatment basis and it changed my life. I became more active and had a sense of a more “normal” life. My family had an amazing opportunity to have a better quality of life in every aspect as well.

Now, the struggles are still there, which provoke some worries. I still have a bad right ankle so I use prophylaxis as my main treatment. I must take the precautions any hemophilia B patient has to as well as routinely visit my hematologist. None of those things have changed, but I still have some worries that are clearly present: acquiring an inhibitor and having to replace a joint. Although there is no evidence that switching products can actually cause an inhibitor, it is something that I still think about. Since having a bad ankle, the thoughts of getting a replacement also comes to mind, but I’m also looking for other options to avoid such an invasive procedure. Even though treatment for hemophilia has advanced in the United States, in Puerto Rico and in the rest of the world, as a patient, I still think about what comes next. Hemophilia B is gaining more attention because there are now more options for factor treatment. I cannot deny it though; life with hemophilia is challenging; but I like challenges - they make us who we are.

I’ve grown up, matured and achieved great things. In 2007, I graduated high school and moved back to Puerto Rico to attend college. While in college, I advocated for hemophilia. I acquired a bachelor’s degree in Business Administration and had a normal life with hemophilia during my years in college; I even obtained a minister’s permit. With life’s struggles, I pulled through and succeeded. Hemophilia B has not stopped or limited me. It has not divided my family, but rather it has united my family. Having hemophilia B has connected me with people I would have never met if I did not have it. As the saying goes, “If life gives you lemons, make lemonade,” and that is exactly what my family and I, with God’s help, have done.

While writing this brief part of my story, I reflected and could not avoid the tears of joy, sadness and gratitude. Many years and many experiences have passed, which have made me who I am today. I live in complete gratitude to God for all He has done in my life. I honestly could not have asked for anything better.

Even though I have hemophilia B, I have access to treatments, while unfortunately many people around the world do not. I met wonderful people during this journey that have helped and blessed me. To those people - you know who you are - I honor you and ask God to bless you. I’d like to honor my family: my father Angel Lopez, my mother Ana Serrano and my brother “Little” Angel Lopez, for giving me their all and for never abandoning me. I do not know what I would have done if it wasn’t for my family and friends. My life is what it is because of every one of you. I love you and ask God to bless you dearly. May He give you many years of life and happiness, as you have given me.
NOW APPROVED FOR HEMOPHILIA B

With IXINITY®

You define the IXperience™

• 98% average incremental recovery. Higher recovery may allow lower doses.†
• Third-generation factor IX with no inhibitors developed in a clinical study.†
• 24-hour half-life for peak factor levels when you need them.†

Learn about a FREE Trial at IXINITY.com

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, diziness, 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.

†The pharmacokinetics of IXINITY have been evaluated in 32 previously treated patients ≥12 years of age with severe to moderately severe hemophilia B.

‡Third-generation product is defined by the National Hemophilia Foundation Medical and Scientific Advisory Council as recombinant factor IX (rFIX) produced in Chinese hamster ovary cells; no human or animal plasma-derived proteins are used in the manufacturing process.

The efficacy (n=68) and safety (n=77) of IXINITY has been evaluated in a prospective, open-label, uncontrolled, multicenter trial in which previously treated patients (PTPs) between 7 and 64 years of age received IXINITY in either a routine or on-demand regimen.

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 brief summary of Prescribing Information on next page.


Manufactured by Cangene Corporation, a subsidiary of Emergent BioSolutions Inc. and distributed by Cangene BioPharma Inc., a subsidiary of Emergent BioSolutions Inc.

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IXI 107-0415
IXIVITY® (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.IXIVITY.com.

Please read this Patient Information carefully before using IXIVITY. 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 IXIVITY.

What is IXIVITY?
IXIVITY 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 IXIVITY when you have surgery.

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

Who should not use IXIVITY?
You should not use IXIVITY if you:
- Are allergic to hamsters
- Are allergic to any ingredients in IXIVITY

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

What should I tell my healthcare provider before using IXIVITY?
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 IXIVITY passes into your milk and if it can harm your baby
- Are pregnant or planning to become pregnant. It is not known if IXIVITY may harm your baby
- Have been told that you have inhibitors to factor IX (because IXIVITY may not work for you)

How should I use IXIVITY?
IXIVITY is given directly into the bloodstream. IXIVITY 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 IXIVITY 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 IXIVITY 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 IXIVITY 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 IXIVITY.

What are the possible side effects of IXIVITY?
Allergic reactions may occur with IXIVITY. 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 IXIVITY in clinical trials was headache.
These are not all of the possible side effects of IXIVITY. 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 IXIVITY?
Store IXIVITY at 2 to 25°C (36 to 77°F). Do not freeze.
Do not use IXIVITY after the expiration date printed on the label. Throw away any unused IXIVITY 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 IXIVITY left in the vial at the end of your infusion.
Do not use IXIVITY if the reconstituted solution is not clear and colorless.

What else should I know about IXIVITY?
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 IXIVITY 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.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use IXIVITY for a condition for which it is not prescribed. Do not share IXIVITY 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.

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U.S. License No. 1201 Issued April 2015 01 035-0814
The bleeding tendency in hemophilia is usually categorized according to the amount of factor VIII or factor IX activity in the bloodstream. A factor level of less than 1% of normal (<1 IU/dl or <0.01 IU/ml) indicates severe hemophilia. A level of 1 - 5% indicates moderate hemophilia, and a level of 5 - 40% indicates mild hemophilia. This classification system was first proposed in the 1950’s and still remains in use today. However, there have always been some people with hemophilia who do not fit the classification. About 10% of those classified as severe bleed more like milds or moderates. Similarly, there are milds and moderates who bleed more severely than expected. This happens in both hemophilia A and B.

The characteristics of a disease, in this case the bleeding tendency, are called the phenotype (FEE-na-type). Scientists and physicians would like to figure out what causes the difference between the factor level and the bleeding phenotype in some patients for two reasons: first, to better understand the disease itself and second to be able to make better treatment choices for the patients affected. Unfortunately, the reasons that some patients fall outside the normal classifications are not yet well understood. There are probably a number of causes. The human body is incredibly complex and everyone is a little different. Just because a person has hemophilia, doesn’t mean that everything else in his body is completely normal. Everyone has many differences from what is considered “normal”. Some of these other differences could affect various aspects of hemophilia.

The obvious first place to look for an answer is in the

How Does Factor IX Level Correspond to the Severity of Bleeding Symptoms?

By Dr. David Clark

www.coalitionforhemophiliab.org Factor Nine News Winter 2016 11
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How Does Factor IX Level Correspond to the Severity of Bleeding Symptoms?

Coagulation cascade (the body’s clotting system) is a complex arrangement of many proteins and other factors, some of which promote clotting and some of which inhibit clotting. They are normally in a delicate balance so the blood clots when it needs to and doesn’t clot when it shouldn’t. A deficiency of factor IX upsets the balance so the blood doesn’t clot normally, but what would happen if some of the other proteins, especially the anticoagulants, were also defective? That could shift the balance back toward clotting. Alternatively, mutations in some of the other clotting factors could also make them more active and shift the balance more toward clotting. (We think of mutations as interfering with the ability of the clotting factors to perform, as in hemophilia, but some mutations can actually make proteins more active.) Researchers have looked at this and found some evidence that changes in the other clotting factors or in the anticoagulant proteins could possibly improve the bleeding phenotype. So far, however, they have found no definitive answers.

Another system that could affect the severity of hemophilia is the fibrinolytic system. The fibrinolytic system’s job is to break down clots after an injury has healed and they are no longer needed. It is also a complex system of many factors. If the fibrinolytic system is more active than normal, that could interfere with the ability to produce stable clots. There is some evidence that changes in the fibrinolytic system can affect the severity of hemophilia, but again, there are no definitive answers.

Platelets are something we tend to overlook because we are focused on the part of the clotting system that involves soluble proteins like factor IX. Platelets are the reason that bleeding eventually stops in people with severe hemophilia. Platelets are small cells that circulate in the bloodstream. When a blood vessel is injured, platelets become activated and stick together to form a plug in the damaged wall of the vessel. The platelet plug is normally stabilized by clotting of the blood, but even without that, it can often do the job itself. Variations in the platelets could lead to increased or decreased bleeding independent of the factor IX level.

The major issue in hemophilia is bleeding into the joints, and we often use the degree of joint bleeding as an indication of the bleeding phenotype. Unless they are on prophylaxis, people with severe hemophilia usually suffer from significant joint bleeding and long-term joint damage. However, it appears that some people’s joints are naturally more resistant to bleeding and/or long-term damage. That may make them appear to have a milder phenotype than is indicated by their factor level.

Now that gene sequences are available for many people with hemophilia, researchers have been able to look at how the specific mutation in the factor IX gene affects the phenotype. One significant observation is that people with the same gene defect can have quite different bleeding phenotypes. This suggests that in at least some cases there must be other things besides the factor IX level that affect the phenotype. Researchers have shown that people whose gene defect leads to absolutely no production of factor IX do tend to have severe disease. The situation for others, who produce only a small amount of factor IX or a defective factor IX protein, is less clear.

Several major questions in this area involve assays. Since the classification system depends on the factor IX level, it is important to be able to measure it accurately. Most commonly used clinical assays for factor IX have a detection limit of about 1% of normal. That means that the assay can’t accurately determine whether a person has only a little factor IX, for example 0.8%, or absolutely no factor IX, and that can make a difference. The presence of even a little factor IX can have a significant effect on bleeding.

This also raises the question of whether a factor IX assay is really the best type to use to determine the bleeding tendency. In the past several years there has been a lot of work on other assays that give a more global picture of the ability of a given patient’s blood to clot. That type of assay might be better able to determine the clotability of the blood and might correlate better with the observed phenotype. Some of the more promising assays are done in whole blood, rather than just plasma, and therefore include the effects of platelets on the blood’s ability to clot.

There are also a number of external aspects that could affect the bleeding phenotype independently of the factor IX level. Differences in activity level, weight, physical condition and stress level, for instance, can affect bleeding and joint damage. Researchers have considered all of these, but the reason that phenotype and factor level do not always match is still not clear. Thus, this is another one of those questions that just doesn’t have a good answer at the present time.
Change the way you picture living with a rare bleeding disorder. Novo Nordisk is helping people like Jay write his story. That’s why we are continuously seeking new ways to advance science, support, and education for the bleeding disorders community. Because at Novo Nordisk, we’re always committed to helping you make your potential possible.

Jay lives with severe hemophilia B with inhibitors.
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Sangamo BioSciences has announced that they have received FDA clearance to start a clinical trial of their SB-FIX gene therapy treatment for hemophilia B. Sangamo will initiate a Phase 1/2 trial to look at safety, tolerability and potential efficacy. Sangamo’s approach uses zinc finger nucleases to insert a normal factor IX gene into liver cells.

Researchers Developing Method to Produce FIX in Lettuce Plants for Oral Treatment and Elimination of Inhibitors

Researchers at the University of Pennsylvania and the University of Florida are working on a project that has three potential benefits for hemophilia treatment. They are developing a method for oral administration of factor IX, a method for producing factor IX in lettuce plants for oral administration and a method for eliminating inhibitors in hemophilia patients.

Oral administration of factor IX has been discussed for years, but has always been problematic. Factor IX tends to break down in the stomach, and the small amount that survives to reach the intestines has a very difficult time crossing the intestinal wall to enter into the bloodstream. Therefore, massive amounts would need to be consumed to produce a sufficient level of factor IX in the blood. With the current factor IX products, the cost would be prohibitive.

The Penn/Florida researchers have developed a recombinant factor IX fused to a carrier protein (FIX-CTB) that can cross the intestinal wall easily. The CTB portion interacts with receptors on the intestinal wall to pull the FIX-CTB across. Once in the body’s tissues, normal body enzymes cut a special site engineered into the protein and release the factor IX from the CTB portion. The researchers have shown in mice that significant amounts of factor IX can enter the bloodstream this way.

The researchers have also genetically engineered lettuce plants to produce FIX-CTB. The plants can produce as much as 70% of their total weight as FIX-CTB. After harvest, the plants are freeze-dried, which encapsulates the FIX-CTB in the cells of the plants and has been shown to keep it stable for up to two years. They found that when the lettuce is fed to mice, the cell walls encapsulating the FIX-CTB protect the product from digestion while it passes through the stomach. The microbes in the intestines break down the plant cell walls and release the FIX-CTB, which can then cross the intestinal walls allowing factor IX to enter the bloodstream.

The researchers are developing a large scale production method for the factor IX-producing lettuce. They believe that this will be a much more cost-effective way to produce recombinant factor IX than the currently-used cell culture methods. Additional cost is avoided because the FIX-CTB protein does not have to be recovered and purified — ultimately the patient would just consume the lettuce. The lettuce would probably be freeze-dried, crushed and placed in capsules. Both the less expensive production method and oral administration could make this a viable product for treatment of hemophilia patients in less developed countries that cannot afford the current factor products.

Another possible benefit of oral administration being investigated is oral tolerization to eliminate inhibitors. Immune tolerance treatments, which can be effective in removing hemophilia A inhibitors, often do not work in hemophilia B. Studies have suggested that oral administration of large amounts of factor IX over a period of time may help to eliminate inhibitors. The Penn/Florida researchers have shown that feeding lettuce-produced FIX-CTB to inhibitor-prone hemophilic mice suppresses and reverses inhibitor development and eliminates anaphylaxis (a severe allergic reaction) against factor IX.

These studies are still in the R&D stage, but the results so far look promising. If even part of this project pans out, it could be a significant improvement for hemophilia treatment.
Inhibitors are antibodies that some patients make in response to infused factor IX. Inhibitors develop in about 5% of hemophilia B patients. In these patients, the immune system thinks factor IX is a foreign protein that shouldn’t be there, so it produces antibodies to neutralize and remove it. Inhibitors significantly complicate therapy since they prevent any infused factor IX from working. Inhibitor development in hemophilia B can also be associated with anaphylactic reactions, severe allergic reactions that can even be life-threatening.

The U.S. Centers for Disease Control and Prevention (CDC) recently completed their Hemophilia Inhibitor Research Study, which was designed to assess the feasibility and usefulness of conducting national monitoring for hemophilia inhibitors. The study had the following conclusions:

• Everyone with hemophilia, regardless of age, is at risk for inhibitor development
• One-third of newly-developed inhibitors were found in people with non-severe hemophilia
• One-half of those who developed inhibitors were over 5 years of age

In response to the CDC study findings, MASAC, NHF’s Medical and Scientific Advisory Committee, has issued recommendations including yearly inhibitor testing for all individuals with hemophilia. They also made recommendations on improvements to inhibitor assays, on furthering inhibitor research and in support of the development of inhibitor prevention programs.

Many THANKS to ALL of you who donated to our Factor Nine Santa Holiday Fund! WE helped over 60 children have a very Happy Holiday by donating toys, food, booths, coats and food baskets!

Special thanks to David Dorian-Ross for holding a special fundraising for The Coalition! The donation is much appreciated and we are grateful for your belief in our good work! David is the TaiChi teacher of Rick and Cassie Starks. Rick has been teaching Taiji fit to our community the past two years and had been at all of our on the road meetings, Symposium and Men’s Retreats.

Thank You!
IMPORTANT SAFETY INFORMATION FOR BeneFix®

- 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.
- Your body can make antibodies, called “inhibitors,” which may interfere with the effectiveness of BeneFix.
- 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.

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.

Please see brief summary of Prescribing Information on next page.

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.

*BeneFix was approved February 11, 1997.

**IMS National Prescription data October 2013.

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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 1-800-934-5556.

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 store 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
- difficulty breathing
- chest tightness
- turning blue (look at lips and gums)
- fast heartbeat
- swelling of the face
- faintness
- 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 venous catheter through which BeneFix is given by continuous infusion. 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. BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Store the diluent syringe at 36° to 86°F (2° to 30°C). 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.

Different storage conditions are described below.

Product labeled for Room Temperature Storage

Store at 2° to 30°C (36° to 86°F).

If you have the product kits labeled for room temperature storage, it can be stored at room temperature (below 30°C or 86°F) or in the refrigerator (2° to 8°C or 36° to 46°F).

Product labeled for Refrigerator Storage

Continuous refrigeration

(2° to 8°C (36° to 46°F))

If you have the product labeled for storage in the refrigerator (2° to 8°C or 36° to 46°F) and you have not taken the kit out of the refrigerator, then the expiration date printed on the package still applies. You can store the product at room temperature (below 30°C or 86°F) for up to 6 months or until it has reached its expiration date, whichever comes first.

If you have taken the kit labeled for storage in the refrigerator out of the refrigerator and stored it at room temperature (below 30°C or 86°F), then use the product within 6 months from the time you took the product out of the refrigerator or until it has reached its expiration date, whichever comes first. If you cannot remember when you took it out of the refrigerator, then subtract one year (12 months) from the date that is printed on the end flap of the carton package. The date you get is your new expiration date. Throw away any product that has gone over the new expiration date.

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-0464-8.0, revised November 2011.
The Coalition was on the road on Saturday, September 19th in Pittsburgh at the Pittsburgh Marriott City Center. The children went on a day trip to the Pittsburgh Zoo chaperoned by Christian Viallarreal.

Nayan Heath held a lively interactive talk called “Step it Up!” followed by “Laughing through Stress with Lori Kunkel. Rick Starks taught the Taiji Fit Class. The afternoon was filled with hot topic discussions and concerns of the community, product updates and what’s in the pipeline with Dr. Dave Clark and our Factor Nine Family meeting.
Rixubis
[Coagulation Factor IX (Recombinant)]

For more information, contact your Baxalta representative today:

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On Saturday October 3rd, we were in Anaheim, California at the Marriott Suites. Speakers included Dr. Diane Dimon on Meditation for Anxiety & Stress and Angelica Flores on how to stay more active and reach your personal goals. The children went to Disneyland for the day trip.

Our last trip of the season was Saturday, November 7th in San Ramon at the San Ramon Marriott. The meeting was filled with many lively discussions such as how to be more active with hemophilia. We were very happy to see so many of our members this year and we look forward to seeing more of you next year on the road! We thank Pfizer for their support and wish you all a very happy and healthy New Year!
The Coalition for Hemophilia B 9th Annual Fundraising Dinner
Thursday, March 3, 2016
Terrace on The Park; New York, New York

The Coalition for Hemophilia B 10th Annual Symposium
Friday-Sunday, March 4-6, 2016
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Registration available online at hemob.org

Join us as we celebrate our 25th Anniversary!

For more information, please contact Kim Phelan at
Kimbo1217@live.com or call 917-582-9077

SCHOLARSHIP
The William N. Drohan Scholarship Fund is Available online:
www.coalitionforhemophiliab.org

Have a safe, happy and healthy New Year 2016.
See you soon!