



Factor Nine News

The Coalition for Hemophilia B

Summer 2015

Topics in Hemophilia



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Generation IX Project





By **Jacose Bell**
GutMonkey

This past June 9-14th marked the second gathering of the Generation IX Project program. Situated on the beautiful Sandy River outside of Portland, Oregon and set among the vast and cooling shade of an old-growth Douglas Fir forest, it was once again a memorable success. There were several familiar faces and it was a reunion for many mentors and teens from the first retreat in October of 2014. For just as many, however, it was a new adventure and the first time they had traveled that far from home. Manuel Lopez and Orlando Correa came from as far as one could come in the United States and arrive in Oregon: Puerto Rico!

Once again, our mentors arrived a few days before the teens to spend time together sharing experiences and insights from our first retreat while preparing to receive a group of teens from all across the country. Again, everyone was affected with hemophilia B, but this year marked a new milestone as the program welcomed its first female mentor with moderate hemophilia B and its first female teen also affected by hemophilia B. Having these two young women in the mix made for an even more powerful weekend as the community grew and the new diversity was celebrated!

As always, Jim Munn, who is the nurse for the Generation IX Project in addition to his many other national and global commitments, brought some great educational content to our time together. We celebrated “New Vein Day” over the course of the weekend, and had several fantastic conversations and Q & A sessions that let participants anonymously ask any questions they had using index cards. Everyone learned a lot during these discussions and both teens and mentors commented on what a valuable portion of the retreat it was to have someone there that could speak specifically to hemophilia B.



Generation IX Project Commentary

The Generation IX project was an amazing experience! It is basically an adventurous educational program, that sometimes felt like a vacation in a rainforest. For mentors and teens with Hemophilia B. It is unlike anything else I've ever experienced. The program was designed and run by Pat 'Big Dog' Torrey through a partnership with the Coalition for Hemophilia B and the generous support of Emergent. Pat, Joe Torrey, Jacose Bell and rest of the GutMonkey crew worked really hard to make this an unforgettable experience. I flew to Portland Oregon and then we went to YMCA Camp Collins, near Mount Hood. On the first day, all of the mentors and counselors introduced ourselves to each other and divulged our guilty pleasure song (songs we love but are ashamed to admit it). I knew this was going to be fun and push a few limits. Next we went over what are the six different personality types; doer, thinker, feeler, dreamer, believer and funster. We did this to help us understand how to relate to the teens we'd be working with for camp. Then, we practiced how to encourage these different types of personality types by doing a challenge course element. We did the high ropes where we suited up in safety gear then climbed up a tree (which had climbing holds) and reached a platform. Next we jumped off to reach for trapeze. We each had a supportive relay team and a coach that helped encourage each climber to try their best. There is no pressure to do this, you just try and do as much as you can and everyone is so supportive. I liked that.

The second day the teen campers arrived. We introduced ourselves to them and asked them to tell us about themselves. We then played several games to get them feeling more comfortable and engaged. One of the most entertaining was Human Hungry, Hungry Hippos. Campers and camp counselors got into teams and used rolling boards, rope, and square hampers, we had to grab as many cage balls as possible before switching to a new rider. On the final day, we had several different activities like climbing up and rappelling down a challenge tower, a high tight rope style ropes course, shooting sling shots at metal can targets, and at the end of the day we had a camp bonfire on the beach close to the camp. We shared what we were going to take from the experience and talked about what we enjoyed about it. I learned a lot about teamwork from the experience as well as from the camp counselors and campers. Listening to their stories helped me to put my disorder in perspective. It reminded me that while we are part of a large and supportive bleeding disorder community, we are also unique. We each have something to share with the community that is relevant and our story might help someone else. If you have Hemophilia B, you must check the Generation IX Project out.

~ Mentor





We are so delighted at the success of this program, now in its second year. It has grown in numbers and unity! I love seeing the mentors and teens bond and make lifetime friendships as well as valuable lessons they will take with them to use throughout their lives. This year I made the trip out there to see firsthand what the camp was like and it was truly a beautiful place. Everyone had a bed and their own window(a nice touch) and very clean. The way Pat and his team work with the mentors and teens is just so wonderful to watch firsthand. This is one program that will continue to grow yearly in success and numbers. Bravo!

~ Kim Phelan, *The Coalition for Hemophilia B*



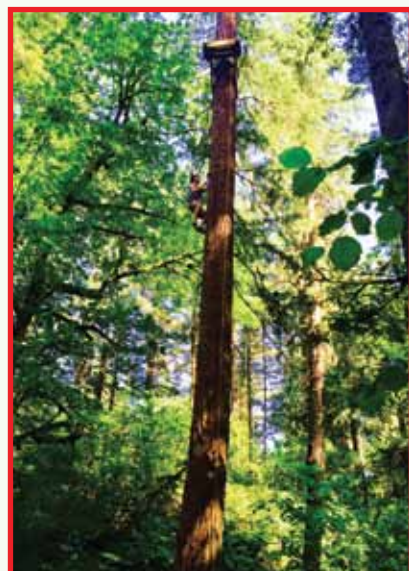
We are so proud to sponsor the Generation IX Project and to support the work the Coalition for Hemophilia B is doing to build a stronger hemophilia B community. When I sit in their circle and hear the guys talk about meeting another person with hemophilia B for the first time, or talk about how it feels to be in a group of "just Bs", I know we are creating something really special and powerful that will have a lasting impact on this community.

~ Jane Franchette, *Emergent BioSolutions*



Generation IX Project is the first national mentorship program for people living with hemophilia B where young adult mentors participate in a cutting edge leadership training before guiding teens through a weekend full of challenges and fun. The GutMonkey team, led by longtime camp superstar "Big Dog" Pat Torrey always challenges everyone yet in a safe way. You will be challenged, but you also do not have to do anything you are not comfortable with. There is great bonding and lots of belly laughing. We believe that amazing, life-long friendships are made when you get to test what you're made of and have an awesomely fun time while doing it!

~ Jacose Bell, *GutMonkey*



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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.

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.

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Is Hemophilia B Less Severe than Hemophilia A?

By Dr. David Clark

Over the past several years there has been a discussion in the medical literature about the differences between hemophilia A and hemophilia B. Hemophilia A, deficiency of factor VIII, and hemophilia B, deficiency of factor IX, are very similar diseases. Factor VIII and factor IX work together in the same step of the coagulation process, so a lack of either interferes with clotting at that point. However, some recent studies have suggested that, overall, hemophilia A might involve more severe bleeding than hemophilia B.

Why does it matter whether hemophilia B is less severe? One reason is that treatment regimens for hemophilia B are often based on those for hemophilia A. Prophylaxis is a good example. Due to the relatively small number of patients, there is a lack of good studies on prophylaxis specifically for hemophilia B. Because of this, prophylactic treatment regimens for hemophilia B have been extrapolated from those for hemophilia A. This extrapolation is based on assuming that the bleeding tendency is the same for a given factor level. If that assumption is not true, and A's bleed more severely than Bs, then it is possible that prophylaxis or the amount of factor used in prophylaxis is over-prescribed for hemophilia B. Some have even suggested that fewer hemophilia B patients might actually benefit from prophylaxis. However, now that we know that even small amounts of bleeding can cause significant joint damage over time, it is probably better to err on the side of too much rather than too little.

It is important to emphasize that we are talking about averages based on the whole populations of hemophilia A and B patients. Any individual hemophilia A patient might have more or less severe bleeding issues than any individual hemophilia B patient. There is no evidence that

the most severe hemophilia A patient has a significantly worse disease than the most severe hemophilia B patient. In fact, as discussed below, if the patients have inhibitors, the hemophilia B patient could have a more serious issue than the hemophilia A patient.

One of the biggest differences, of course, is the prevalence. Hemophilia A occurs in about one in 5000 males, while hemophilia B occurs in about one in 30,000 males (the occurrence in females has not been studied well). The reason for this difference is probably related to the size of the protein and its gene. Factor VIII is a much larger protein and thus has a much larger gene than factor IX, so it is more likely to experience a mutation.



There are also differences in the types of gene mutations that occur in the two diseases that might account for differences in severity. Hemophilia A tends to involve large scale gene mutations that lead to the body producing no factor VIII, while hemophilia B tends to involve smaller, usually missense mutations.

Missense mutations affect a small part of the factor IX molecule, so the body produces a protein that looks something like factor IX, but which might not work properly. (Note that the missense mutations can also produce variants of factor IX with higher activity. However, individuals with those types of mutations are rarely identified since they usually don't have bleeding problems, unless the activity is so high that it produces thrombosis – too much clotting.) Thus while both A and B patients might be classified as severe because they have less than 1% factor activity, hemophilia A patients often has zero factor VIII activity, while hemophilia B patients tend to have a little factor IX activity. That can make a difference. One study that tried to address this issue by selecting

Is Hemophilia B Less Severe than Hemophilia A? *continued*

only subjects with mutations that were likely to result in no clotting factor production found equal rates of joint surgery between the two groups.

The distribution of severities for the two diseases are different. One study showed that 43.5% of hemophilia A patients had severe disease compared with 30.9% of hemophilia B patients, where severe disease is classified as a factor activity less than 1% of normal. For moderate severity (1 - 5% activity), the numbers were 17.8% for hemophilia A and 30.1% for hemophilia B. Interestingly, the combined percentages for severe plus moderate are almost equal at 61.3% for A and 61.0% for B. The percentages for mild hemophilia (5 - 40% activity) are also approximately equal at 38.7% and 39.0%, respectively.

Some of the other differences are less straightforward. One Scottish study showed that the hospital admission rate for hemophilia B patients was 2 – 3 times lower than for hemophilia A. A small Swedish study using a severity scoring system showed scores twice as high for hemophilia A, but that difference disappeared when the same scoring system was used for an equally-small group of Italian subjects. A Canadian study found almost twice the number of bleeding episodes per year for hemophilia A, 14.4 versus 8.6 bleeds/year, however, another Italian study found similar but wide ranging bleeding rates for both at 12 – 30 bleeds/year. The same Canadian study also found that A patients underwent 3.2 times more orthopedic surgeries than Bs, in agreement with a third Italian study that found three times more As having joint arthroplasty surgery than Bs. However, that was not in agreement with a Dutch study that found similar rates of arthroplasties between the two diseases, as well as similar ages at first joint bleed, similar treatment intensities and similar bleeding frequencies. A UK study found no statistically significant difference in death rate for hemophilia patients in all severity categories. There are many more studies, but one of the issues with all of them is the relatively low numbers of subjects with hemophilia B to use for comparison.

In terms of treatment, because factor VIII and factor IX have different half-lives and recoveries, there is no easy way to compare factor usage and treatment regimens. One big overall difference was found in another Canadian study that showed that 69% of hemophilia A patients were on prophylaxis compared with only 32% of hemophilia B patients. The reasons for the difference are unclear. It could be because of severity differences, but it could also be due to how physicians are used to treating hemophilia B. An earlier Dutch study had shown similar rates of prophylactic treatment for the two groups, which could just be due to different attitudes toward prescribing prophylactic treatment.

One of the biggest differences between hemophilia A and B is the incidence of inhibitor formation. Up to 30% of hemophilia A patients develop inhibitors, while less than 5% of hemophilia B patients do so. This is thought to be because hemophilia A patients often produce no factor VIII, so their immune system has never seen a protein that looks like factor VIII. Their immune system, therefore, thinks any infused factor VIII is a foreign protein that isn't supposed to be in the bloodstream, and it eliminates it. Since hemophilia B patients often produce a protein that looks a lot like factor IX, even though it doesn't work as well, their immune system is used to seeing it and doesn't look on any infused factor IX as foreign.



However, while hemophilia B patients tend to get fewer inhibitors, when they do develop an inhibitor, it can be much worse than in a hemophilia A patient. Hemophilia B inhibitors are also often associated with anaphylaxis, which is a severe allergic reaction to the protein that can even be life-threatening. Treatment of factor IX inhibitors is also more complicated. Factor VIII inhibitors can often be eliminated by immune tolerance induction (ITI) treatment, which is successful in 60 – 80% of cases. ITI works in less than 50% of hemophilia B cases. It is often complicated by anaphylactic reactions and nephrotic syndrome, which can cause serious damage to the kidneys.

Overall, there are probably more studies suggesting that hemophilia A is more severe, but there is still no conclusive answer. For the individual patient, it does not really matter. Each individual patient has to be treated based on his or her own characteristics and symptoms. It would be great if hemophilia B wasn't as severe, but unfortunately the answer is not any clearer than many other questions concerning hemophilia B.

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*The pharmacokinetics of IXINITY have been evaluated in 32 previously treated patients ≥12 years of age with severe to moderately severe hemophilia B.¹

†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.²

¹The 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.¹

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 brief summary of Prescribing Information on next page.

Reference: 1. IXINITY [coagulation factor IX (recombinant)] prescribing information. Winnipeg, MB, Canada: Emergent BioSolutions Inc.; April 2015. 2. National Hemophilia Foundation Medical and Scientific Advisory Council (MASAC). MASAC recommendations concerning products licensed for the treatment of hemophilia and other bleeding disorders. MASAC Document #230. September 2014.

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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?

Store IXINITY 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.

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

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.

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.

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Life Begins Where Your Comfort Zone Ends

By Collin Johnson, GA (age 16)

It really hit me a while back that you're never going to be your best you if you're not willing to push yourself to the limits. After camp, I'm always worn out and don't want to do anything, but it's always the highlight of my summer because I get to try new things and learn new skills. It's extremely hard to find a limit in my comfort zone because I'm comfortable doing just about anything. However, there are things I'm not comfortable doing, like public speaking. I've learned that I have to push past that and be uncomfortable to become a better me and to be a better leader. For instance, heights don't bother me; actually, at times, I feel more comfortable in the air than I do on the ground; however, I've realized I need to work on internal fears like speaking to a lot of people or talking to people I don't know. Honestly, that is my greatest fear; not knowing what to say or how to fix a problem. I work well in stressful situations, and I can handle the pressures, but when it comes to talking to groups of people, I feel very small and useless. I know I need to work on that.



Camp is a great place to work on these skills. I meet new people and try new things that I may not have done before. Camp pushes me to be my best me and teaches me the skills I need in order to be successful in life because, not all things about life are taught in school. We learn math, science and other things in school, but if you haven't learned to speak with people and be social, then it's all for nothing; you have no voice to state your opinion. Camp teaches me to be independent

while helping me to fix problems when I can't solve them on my own. All kids need a sense of independence. While at camp I definitely get that, I know that I'm not alone when I have a problem and that there is always someone willing to help me when I mess up. Camp connects me with people I wouldn't normally be friends with; I gain brothers in a bond that nobody on the outside will understand. When I'm at camp I feel like I'm at home with family. The little kids are the younger siblings and I help them if they need it, while the older kids or counselors and doing the same in return for me. I connect on a level that's deeper than just friends.



All in all, camp is an amazing experience that every kid needs to experience. Camp for children with bleeding disorders is an even better experience for some, because it's the only week they get where they don't have to worry about their disorder. There's nobody telling them they can't do something because they have a bleeding disorder. They are given the freedom to be "normal", even if they at times, feel they are not. I would never trade my bleeding disorder for anything; it's taught me how strong I can be and how to handle stressful situations calmly. It's also led me to meet great people and form some amazing friendships. I went to Washington D.C. with Hemophilia of Georgia to work on getting laws passed and budgeting set up to help the bleeding disorder community, as well as many other things. If it wasn't for my disorder, I would have never been able to do some of the amazing things I have done alongside these remarkable people. Overall, I want to say thank you to all who stand behind me as I grow and learn at camp and through other programs that support children with bleeding disorders!



Jay lives with severe hemophilia B with inhibitors.



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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.



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Industry News

Baxalta

Baxalta Spins Off from Baxter

On July 1, 2015, Baxter International spun off its BioScience Division as a stand-alone company named Baxalta. Baxalta includes all of Baxter's former hemophilia treatment business.



Catalyst Biosciences Merges with Targacept

Catalyst Biosciences, Inc. has merged with Targacept, Inc. The new company, retaining the Catalyst Biosciences name, is working on more potent, longer acting versions of factor VIIa for inhibitor treatment, factor IX for treatment of hemophilia B and factor Xa for treatment of general bleeding disorders. Catalyst has a proprietary method for generating large numbers of mutated or variant factor proteins, which they then screen to find ones with longer half lives and higher activities.

CSL Behring

CSL Behring Begins Enrollment in Phase II/III Trial of rVIIa-FP

CSL Behring has begun enrollment for its Phase II/III trial of rVIIa-FP, its longer-acting factor VIIa product for treatment of hemophilia patients with inhibitors. In rVIIa-FP, recombinant factor VIIa is fused to recombinant albumin to give it a longer half life in circulation. The goal is to recruit about 54 hemophilia A or B inhibitor patients. The study will evaluate pharmacokinetics, efficacy and safety of rVIIa-FP.



FDA Approves Two New Treatments for Hepatitis C Genotypes 3 and 4

On July 24, 2015, the FDA approved two new oral drugs for treatment of hepatitis C. Bristol-Myers Squibb's Daklinza™ (daclatasvir) in combination with Gilead's Sovaldi® (sofosbuvir) was approved for patients with genotype 3, and AbbVie's Technivie™ in combination with ribavirin was approved for some patients with genotype 4.

Daklinza was approved based on a clinical study in 152 subjects, both treatment-naïve (never treated for hepatitis C) and treatment-experienced (previously treated, but not cured) participants with genotype 3 infection. The participants were given Daklinza and sofosbuvir once a day for 12 weeks and then followed for 24 weeks after treatment. Of the treatment-naïve subjects, 98% of the subjects who did not have cirrhosis and 58% of the subjects with cirrhosis showed a sustained virologic response (SVR), which means they no longer had detectable virus in their blood. Of the treatment-experienced subjects, 92% of the subjects who did not have cirrhosis and 69% of the subjects with cirrhosis showed an SVR.

Technivie is a combination of three drugs, ombitasvir, paritaprevir and ritonavir. The clinical study included 135 subjects with genotype 4 hepatitis C but without cirrhosis. Participants received Technivie once a day for 12 weeks, either with or without ribavirin. 100% of the subjects who received Technivie with ribavirin achieved an SVR, while 91% of those treated without ribavirin achieved an SVR. These approvals further expand the options for treatment of hepatitis C without the use of interferon.



NHF Nurse's Guide to Bleeding Disorders

Since 1995, the National Hemophilia Foundation (NHF) has published the Nurses' Guide to Bleeding Disorders, which is available on the NHF web site at <http://www.hemophilia.org/Researchers-Healthcare-Providers/NHF-Provider-Working-Groups/Nursing-Working-Group/Resources-for-Nurses/Nurses-Guide-to-Bleeding-Disorders>. This comprehensive guide is updated periodically and provides a wealth of information on the care and treatment of patients with hemophilia and other bleeding disorders. Although it is intended for nurses, it is also a valuable resource for patients and others who need a good, accurate overview of hemophilia treatment.

COALITION B ON THE MOVE

**June 6, 2015 Coalition for Hemophilia B Meeting on the Road
Albuquerque, New Mexico**

We had a wonderful meeting which was well attended and it was so nice to see our members in person! We had good speakers and Rick Starks did his famous Taji Fit Class! The children attended the Albuquerque Zoo and later made a big splash swimming! WE love our meetings on the road! Special thanks to our sponsor:





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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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R_x only

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 infuse BeneFix?

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

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

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

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

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

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

wheezing
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 blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

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

How should I store BeneFix?

DO NOT FREEZE BeneFix. 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 kit 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 product 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.

**June 9, 2015 Generation IX Project
Portland, Oregon**

Christian and I stopped by the Camp to say hello and see the beautiful facilities and meet some of the attendees! Now we understand why the teens and mentors are willing to travel



so far to attend! It is just one of the most beautiful camps I have ever seen! Pat and his team from GutMonkey put together an amazing program! Special thanks to our sponsor:



Special CONGRATULATIONS to a beautiful couple and members of our Coalition B Family, Brittany Peterson and Rocky Williams, on their engagement! We are so very happy for you!



**August 13-15 , 2015 NHF Annual Meeting
Dallas, Texas**

The NHF was held at the beautiful Gaylord Texas Resort this summer. The programs were excellent and the final night event was filled with entertainment and a rodeo. Everyone learned a lot and also had a good time. It was a pleasure to attend and we were happy to see so many members stop by our booth!



Sponsored by a generous grant from



The 3rd BScene Video is now up on our Facebook Site Coalition for Hemophilia B Featuring Elliot Goldrick! We are happy to have members in our community share their story with you!





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The Coalition for Hemophilia B understands there are families within our bleeding disorder community who are feeling the effects of the current economic situation. We thought it would be a nice idea to ask our more fortunate Factor Nine Families to make a financial donation to the *Factor Nine "Holiday Fund"* to help buy gifts for children with hemophilia this holiday season. The Coalition for Hemophilia B will also contribute to this fund.

If you wish to make a donation, please send a check payable to:
The Coalition for Hemophilia B "Holiday Fund"
 825 Third Avenue, Suite 226; New York, NY 10022

Please respond by **December 12, 2015** so that the Factor Nine Santa can load his sleigh with holiday gifts for all good girls and boys!

For those families in our community in need of a little Holiday Cheer, we would like to help put something under the tree for your children! Just fill out this form and send it to Santa's special elf, Kim at the "East" Pole. Since the Factor Nine Santa has such a busy schedule, please send it to us ***no later than December 12, 2015.***

(Your name and information will be kept *strictly* confidential.)

Please send this form to:

The Coalition for Hemophilia B Holiday Cheer
Attention: Special Elf Kim
 825 Third Avenue, Suite 226; New York, NY 10022

We wish you all a beautiful season filled with love, happiness and good health!

Name: _____

Phone: _____

Address: _____

Please give us an exact description of the item your child is wishing for.
 If we have any questions, we will contact you directly.
 Holiday gifts will be purchased by The Coalition and sent to your home.



Child's Name and Age:

Child's Name and Age:

Child's Name and Age:

Wish List:

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Wish List:

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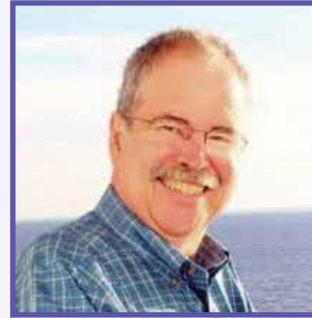
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 **COAGULATION**

In Memorium



Joe Caronna



George Price

On Thursday, September 10, 2015, we lost a dear member of our community, Joe Caronna of Inalex Communications. Joe was 55 years old. Joe had the most wonderful sense of humor and would always put a big smile on your face when you saw him coming your way! As founder and president of Inalex Communications, Joe truly made a difference by hosting fantastic programs to help improve the lives of people with hemophilia. Joe helped so many in our tight knit community and we are all better people for having known him. Our heartfelt prayers go out to Joe's wife, Cathy, his children, Alex and Christina, and to his family and friends, especially his dear friend, Sal. We will all miss him dearly.

Godspeed, Joe.



We are very saddened to hear of the passing of George Walter Price on Saturday, August 15, 2015. He was 66 years old. Throughout his government career and 26 years as a Navy reserve officer, George proudly served our country. He also had a strong history of volunteering in the bleeding disorder community and was especially proud of his accomplishments as president of the Hemophilia Association of the Capital Area. The most important part of George's life was his family. Our thoughts and prayers are with his wife, Linda, sons Greg and Jeffrey, and his large, extended family. George was a wonderful man and will be greatly missed by those who were fortunate to have known him.

Rest in peace, George.





SAVE THE DATES

**The Coalition for Hemophilia B
9th Annual Fundraising Dinner**

Thursday, March 3, 2016
New York, New York

**The Coalition for Hemophilia B
10th Annual Symposium**

Friday, March 4-6, 2016
New York, New York

Join us as we celebrate our 25th Anniversary!

For more information, please email: hemob@ix.netcom.com or call Kim Phelan at 917-582-9077



We wish you all a very happy Fall season!