



Factor Nine News

The Coalition for Hemophilia B Fall 2012

Topics in Hemophilia

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The Coalition for Hemophilia B Factor Nine Family Breakfast Meeting was held in conjunction with the National Hemophilia Foundation Annual Meeting in Orlando Florida in November 2012

We had a great turn-out at our Factor Nine Family meeting! Nayan Heath gave a great talk on *Overcoming Challenges*. Nayan's presentation was followed by our group session and an updated presentation by Dr. Dave Clark on current research. Since our program ran overtime a bit, many folks did not make the picture we took at the end because they had to leave! It was wonderful to see you all! 🇺🇸



Season Highlights!



Fall Fishing

Hunter (15), **Coy** (10) and **Montana** (8) love to fish on the ranch. These three brothers can't wait for the irrigation canal to shut down each fall. The irrigation canal water is shut off creating shallow pools of water that fish can't swim out of (if not caught, the fish will die).

Due to the freezing temperatures, ice had formed on the pools of water. The boys spent an entire day fishing in the canal. Their technique was to first "stomp on the ice" to break it open, then using nets and bare hands, they caught the fish. This fishing extravaganza is an annual event for the boys!

Inhibitors in Hemophilia B

By Dr. David Clark

Inhibitor formation is one of the most significant problems in hemophilia treatment today. Although it is much less common in hemophilia B than in hemophilia A, the consequences for patients with hemophilia B can be much worse and the treatment options more limited. Inhibitors are antibodies that neutralize clotting factors and prevent them from working. Only about 3% of hemophilia B patients develop inhibitors against factor IX. However, factor IX inhibitors are more difficult to eradicate and may be accompanied by severe complications. In addition, because of the relatively small number of hemophilia B inhibitor patients, even including all patients worldwide, much less is known about treating them.

Inhibitors are often detected because of the failure of a patient to respond to infused factor. They usually develop early in life after only a few factor treatments. Regular screening for inhibitors and analysis of a patient's genetic defects are recommended. Inhibitor development does not appear to be associated with the specific factor product used, although that has happened and is always a concern when a new product becomes available. The risk of inhibitor formation appears to be the same for recombinant and plasma-derived products.

Antibodies generally protect us from disease—when the immune system discovers a bacteria or protein in the bloodstream that it doesn't recognize, it forms antibodies to remove or destroy the intruder. Although the reasons for inhibitor formation in hemophilia are still not well understood, the immune system may see infused factor IX as something it doesn't recognize, so it tries to get rid of it.

Inhibitors occur more often in patients with large factor IX gene defects or defects that result in little or no protein production. People with smaller gene defects often still have a protein circulating in their bloodstream that looks like factor IX, even if it doesn't produce clotting. The additional factor IX that they infuse doesn't look much different, so the immune system leaves it alone. However, people with large gene defects may not produce anything that looks like factor IX, so infused factor IX looks to the immune system like something foreign that needs to be removed.

The activity of inhibitors is measured in Bethesda Units. A "low responding" inhibitor is one that has an activity of less than 5 Bethesda Units (< 5 BU). Patients with low responding inhibitors can often be treated by simply infusing a larger amount of factor, enough to overwhelm the inhibitor and also provide additional activity to produce clotting. Low responding inhibitors sometimes disappear on their own.

Most factor IX inhibitors are high responding (≥ 5 BU). High responding inhibitors usually persist and often get stronger. They can't be overwhelmed with larger amounts of factor - it would take too much to be practical or affordable. Patients with high responding inhibitors are usually treated either with recombinant activated factor VII (NovoSeven, Novo Nordisk) or a plasma-derived activated prothrombin complex (FEIBA, Baxter).



These products are called bypassing agents because they work via other parts of the clotting system, bypassing the factor VIII/factor IX step. NovoSeven is preferred for hemophilia B patients with inhibitors because FEIBA also contains factor IX, which can in some cases make the inhibitor worse and is a problem for inhibitor patients who develop allergic reactions to factor IX. However, some patients respond better to one product than the other. A number of additional bypassing agents are currently under development.

The cost of these products is high, but it is still important to treat bleeds quickly to prevent subsequent joint damage - the cost to repair damaged joints can be even higher, not to mention the potential decrease in quality of life. Recent studies have shown success with both NovoSeven and FEIBA for prophylactic use. However, patients receiving prophylactic treatment with bypassing agents must be monitored carefully because of the risk of thrombosis, too much clotting, which can be dangerous. The bypassing agents have also made surgery possible for inhibitor patients, although it is still challenging, and should preferably be undertaken in consultation with an experienced treatment center.

One significant issue for hemophilia B patients is that inhibitor formation is accompanied by an allergic reaction

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to factor IX in about half of patients, something that doesn't happen in hemophilia A. An allergic reaction may occur before, at the same time or later than the first occurrence of an inhibitor. Allergic reactions can develop suddenly and can be severe, even life-threatening, so the World Federation of Hemophilia (WFH) recommends that newly-diagnosed patients who have a family history of inhibitors or who are considered likely to develop inhibitors have their first 10 - 20 infusions in a hospital or clinic equipped to deal quickly with a severe reaction.

Immune Tolerance Induction (ITI) is the one method that has had some success in eliminating inhibitors. It is often successful in hemophilia A patients but only in about one-third of hemophilia B patients. ITI involves frequent or daily infusions of factor over a period of time until the

inhibitor disappears. Because of the danger of allergic reactions, hemophilia B inhibitor patients undergoing ITI are sometimes also given immune system-suppressing drugs. Hemophilia B ITI patients may also develop nephrotic syndrome, kidney damage that can sometimes be permanent. Because of these risks, WFH recommends that ITI for hemophilia B inhibitor patients only be undertaken in consultation with an experienced treatment center.

Inhibitor development adds additional complexity to the already challenging task of dealing with hemophilia. However, with proper treatment, inhibitor patients can lead relatively normal lives. The important points are to treat bleeds quickly but otherwise to proceed cautiously because of the risk of associated complications like allergic reactions and nephrotic syndrome. 🐾

Messages From Kim...

Hello All!

We have started working on our Winter Edition of Factor Nine News and would be delighted if you would send us photos and a brief write-up about your talents in the field of Music, Dancing and Singing! Please send to me directly athemob@ix.netcom.com

Also, we are putting together a band called the B-FLATS (not B-Sharps!). The band will perform one or two songs at our Fundraising dinner each year. If we have someone who sings, plays cello and piano they would perform one song, another group that plays guitar and drums, etc. would perform a second song. Please send us your videos or a write-up if you would like to join the group. Each year we will have a certain number of members from the B-FLATS come to New York with their family as our guests and perform! We want this to be an enjoyable experience so have fun with it and come play with your peers!

If you have any questions please call me at 917 582-9077 or email hemob@ix.netcom.com
I am excited to hear from you! Have a wonderful day!

Over the past several months we have received many requests for assistance from families in our community. We believe it takes a village, even \$5.00 will make a tremendous difference in the quality of life for people with hemophilia in need. We thank you for all your love, kindness and generosity to help families in need! And to make things easier, we have exciting news! We now have a PayPal account to make it easier to make donations! Just visit our website coalitionforhemophiliab.org and click on **donate**, which will bring you directly to our PayPal site, or go to PayPal and use our email (hemob@ix.netcom.com) address to donate.

Thank you! Thank you! **Thank you!!!**

Best,
Kim

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Hemophilia and Inhibitors

How It Changed My Life

By Michelle A. Brabon

I was first introduced to the word *hemophilia* when I was 8 years old. To me, hemophilia was just a word that caused my little brother to be in the hospital ICU - a place where I was unable to visit my baby brother. Back then in 1983, I didn't know there were different severities of hemophilia. I didn't know there was A, B, and other bleeding disorders so very similar. All I knew was my little brother was in pain for days and would constantly cry. Mom had to catch a ride 22 miles to the nearest hospital and from there be flown to Phoenix. My baby brother was bleeding in the head, for no known reason. He fell into a coma. We moved to Phoenix to be close to the hospital. Mom and Dad would be at the hospital all day, for months. My brother passed away three months after his first birthday.



I remember telling people at school about my brother and that he had hemophilia. I remember it wasn't questioned and I didn't have to explain what it was. I would just get a nod, hugs and apologies. I thought everybody knew what it was.

No one else in my family had hemophilia. Then, nine years later, my sister was pregnant and going into labor. My parents called to tell me they had to go to the hospital. They didn't sound happy for the new baby boy. They told me my sister had complications, the baby was bleeding in his head and that he had hemophilia. Why, how? My other sister has two kids and they are fine. After a long period of being in the hospital, I finally got to see my nephew. I remember his big black eyes looking at me. While my sister was going to give him a bath, I saw he had a tube coming out of his chest - a port-a-cath.

During that time, I had my own apartment and every time my nephew was in the hospital, I would go and sit with

him. That helped my sister and brother-in-law get rest, go to work or go home for a little bit. I learned quickly the procedures of treatment and what the nurses and doctors looked for when they came in and out of the hospital room. Although I was just a teenager back then, I treated my nephew like my own. My sister went on to have two more sons and they were also diagnosed with hemophilia. We finally were all tested. Of the six of us, my three sisters and I were all diagnosed as carriers of hemophilia.

From then on, I wanted to know everything I could about it. I attended a tech school to work in a hospital since I was so familiar with the hospital scene. All my research papers, oral presentations and reports were on hemophilia. I decided I wanted to be a nurse so I started college for my pre nursing requirements in hope of being admitted to the nursing program. When I was almost finished with school, I applied and took the entrance exam for the nursing program.

I was very excited that I passed the exam - I just had to wait to turn in my final grades. Unfortunately, one of my required classes didn't get the grade I needed and around the same time, I found out I was pregnant! No problem, I knew I could take the class again and some intro to parenting, and infant and toddler childcare classes with it. I also began talking with the HTC to discuss hemophilia. I was ready and I was going to be the best mother ever!



Dylan was born and was diagnosed with severe hemophilia B. I was so happy to be a mom. Having your own son with hemophilia is very different

than just taking care of your nephews. You are in charge of everything: all the information, insurance, medication, emotions, stress and physical wear down. My classes didn't prepare me for the extreme. I didn't learn about all this at any of my schools. My sister never told me all this.

I have gone through the rough times - the emotional and physical stress of when my son gets hurt; hitting his head and waiting for the CAT scan results. I have had financial stresses from missing work and getting fired from a job due to my hurt child. Sometimes I lacked sleep because I needed to care for my baby in pain from an injury or had spent long nights at the hospital ER advocating for my son. In addition to surgery, infections, prophylaxis, inhibitors and dose changes, I've been through the stresses of insurance, reaching lifetime caps and being without insurance. Additionally this year, we moved from the comfy environment of a special needs preschool that Dylan has been part of for two years to a regular kindergarten.

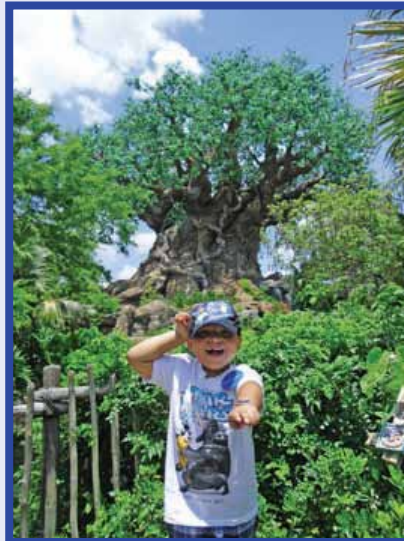
Luckily, there is support. My support systems are the Arizona Hemophilia Association, The Coalition for Hemophilia B, the HTC, and many other resources. The bleeding disorder community is there for us. I would have never have gotten through my rough times without these people being an active part of my life. They have taught me what I needed to know, they have given me a whole new education. I have met so many new friends, not only in my state, but all across the country. I have compared stories with parents and families from all over. I strongly urge people to become active in their community.

I try my best to be involved with the community. I recently, volunteered for the *My Nana's Best Tasting Salsa Challenge* for the first time and had a blast! Being Dylan's mother has changed my life. I have done things I most likely would not have done without having hemophilia in my life. I am captain of my own hemophilia walk team - I never have been captain of anything in my prior life. I never liked to speak in front of people unless I absolutely had to. Now, I am a volunteer speaker for the AZ Hemophilia Association. I help spread awareness about bleeding disorders. I have traveled with my family to places I never thought I would ever go.

My biggest feeling of accomplishment was joining *Cruisers for Bruisers* in January. I never would have seen myself participate in the AZ PF Chang's Rock N' Roll Marathon. I decided to do the half marathon, which was 13 miles long. The marathon started downtown Phoenix and ended at the Arizona State University Stadium in Tempe. I didn't train as much as I wanted to, but definitely tried to be more active. I met up with friends to walk around the canal, with my niece to jog around the canal. I did cardio at the gym and hit up the Wii Fit. I even saw a nutritionist to get me to

track with what I was eating. With Dylan hurt on and off, I did what I could to train for the run.

I thought I was doing okay, but then the day before the marathon, Dylan participated in a *Kid's Rock* event and had hurt his ankle. I ran the mile with him, pushing him in his stroller, but I got so winded I had to finish the short mile walking. I thought, if I can't do this today, how I am going to run tomorrow? I was motivated because I had gathered donations from various people. I had to do it!



The next morning I woke up and stretched, drank water, and woke up my husband and son and made our way downtown. I was so nervous. It was so crowded, and everyone looked physically fit and they all had someone to run with. I was doing a big marathon for the first time and I was doing it alone. I found my assigned group/corral and my husband, Jason, and son, Dylan, stood with me until it started. The people on the side yelling and cheering you on helps to keep you motivated, but what kept me going was my family - Jason and Dylan kept meeting me at unexpected places. I was so happy to see them; they would give me that extra push when I needed it. Even with the blisters and leg cramps, I finished and

passing that finish line was such an awesome feeling! I was sore the next couple of days, but in a heartbeat would do it again. Next year, my goal is to get more donations and finish under 4 hours.

My son is everything to me. Hemophilia gave me the life I am living and I'm happy to have it as part of our life. I believe it makes us all stronger people. Dylan is 7 years old now, and I tell him he makes me a better person every day! I still worry about my son getting hurt, but every mother worries about their children. Last year, I took my first vacation with my husband, without my son. He stayed with his grandparents. It was hard and worried if he would get hurt while we were gone or if he would get his factor.. I worried a lot about him and I missed him so much, but everything worked out. He was fine. We were fine.

Treatment and knowledge has sure changed over the years, for the better. I continue to learn more about bleeding disorders and have definitely learn something new at our annual functions. I believe with spreading the knowledge of hemophilia, it will no longer be an unknown word. It will be a well-known bleeding disorder that will continue to inspire more changes and we will benefit from that change. There is no cure for hemophilia, but I will do everything I can to help support the community. 🇺🇸

EXPLORING BARRIERS

“I’m not going to do this anymore!” I remember thinking to myself as I pulled the butterfly needle from my arm. It was my third attempted infusion for the morning. Even though I had been self-infusing for a number of years, I felt tired, frustrated and angry at having to constantly stick myself. Recently I had experienced a rash of blown or missed veins and that morning was the final straw for me. Vowing to “bury my head in the sand” when it came to my hemophilia, I slapped on yet another band-aid, hastily shoved items into the sharps container, placed the peel-off sticker from my factor vial onto my infusion log calendar so as not to alert my mother, grabbed my backpack and headed off to school.

That was the start to one of the more foolish decisions I’ve made in my life. I managed to hide not taking my infusions for a little over a week. It was then I found myself stuck on the couch with an elbow bleed like I’d never experienced before. The bleed required visits to my hemophilia treatment center and also some time in physical therapy. I was around fifteen years old at the time, and the lesson I learned then has stuck with me through adulthood.

Unfortunately, my transgression in choosing to ignore taking my prescribed medications as recommended is not unique. Non-adherence to prescribed medications places countless patients at risk and adds a significant cost burden to our healthcare system. “People who are prescribed self-administered medications typically take less than half the prescribed dose,” notes one health researcher, R. B. Haynes. An article from the National Center for Biotechnology states, “When preventative or treatment regimens are very complex and/or require lifestyle changes and the modification of existing habits, non-adherence can be as high as 70 percent.” The consequences of non-adherence are not only putting patient’s health at risk, but it also costs a lot of money. According to the same article, “Yearly expenditures for the consequences of non-adherence have been estimated to be in the hundreds of billions of dollars. Estimates of hospitalization costs due to medication non-adherence are as high as 13.5 billion dollars in the U.S. annually.”

The stakes and cost of non-adherence in the hemophilia community are high. In my example, the additional factor, hospital visits and subsequent trips to the physical therapist greatly increased my healthcare cost for the year, not to mention the added out-of-pocket copays I was responsible for. Even more worrisome, my health and overall well-being was placed at risk by neglecting the prescribed recommendations of my healthcare providers. Many in our community have similar stories. One mother recalls the depression that began to sink in for both her and her son when infusions were not going well. She recalls, “Mornings were just so hectic. With all the kids flying around and his moderate needle phobia, stress was high and infusions were often not getting done. I found myself battling with my son every treatment day. There were many bumps, bruises and bleeds...it became clear he was starting to resent me and soon we were both depressed, it was sucking the life out of both of us...”

Research has indicated non-adherence typically is the result of multiple influences. The World Health Organization sites a number of issues resulting in patient non-adherence including social and economic factors, condition-related factors, therapy-related factors and patient-related factors. According to their *Adherence to Long Term Therapies*, social and economic factors include level of education, employment status, unstable living conditions, lack of an effective social support networks, cost of medication, poverty and family dysfunction. Condition-related factors are compromised by the severity of the symptoms or disease, and availability of effective treatments. Therapy-related components such as the complexity and duration of the treatment program have shown to play a major role in adherence. Patient-related factors include the knowledge, attitude, beliefs and perception of the condition by individual. Depression and drug or alcohol addiction also play a major role in patient adherence.

As the causes for non-adherence are multi-faceted, so too are the approaches and interventions to promote maintaining a treatment plan. Because influences affecting adherence are unique from person to person, programs

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RS TO ADHERENCE

By Justin Lindhorst

need to be tailored to the individual. Among other things, research has shown increased adherence by patients with a strong social support group and frequent patient-to-patient interaction. The World Health Organization notes, “Social support, i.e. informal or formal support received by patients from other members of their community, has been consistently reported as an important factor affecting health outcomes and behaviors. There is substantial evidence that peer support among patients can improve adherence to therapy.” Additionally, an article in Strategic Medicine shares questions that can be used to empower and promote adherence:

- What part of living with (chronic illness) do you find most difficult or unsatisfying?
- How does (the situation described above) make you feel?
- How important is it to you for this situation to change?
- Are you willing to take action to improve the situation for yourself?
- What are some steps you could take to bring you closer to where you want to be?

Maintaining a prophylaxis regimen can be difficult. However, the importance of adhering to this therapy calls for us to find unique and innovative ways to overcome these difficulties. After much turmoil, the mother previously mentioned found infusions much easier to complete while her son lay in bed sleeping. “He’s always been a heavy sleeper, so one morning after discussing a plan with some other hemophilia moms, I finally decided to give it a try. I snuck in, put the numbing cream on and when it came time to infuse, I nailed it on the first try! I almost did a dance right there in the bedroom. Soon I became a pro at performing infusions while my son slept; within days we were both getting along better while the stress level, depression and the bruises began to fade away.”

After my horrible elbow bleed, I knew it was time to change the way I was approaching caring for myself. I took some time with my mother and my treatment team to evaluate and come up with an individualized plan to

make sure I received infusions with minimal frustration. After my elbow bleed cleared, we took my regimen down to three times per week, as opposed to every other day. I began taking extra steps in the morning to insure my veins were ready for infusion, including staying hydrated and taking a warm shower. If I ever missed or became frustrated, I would ask my mother to infuse me. Just a few relatively simple steps made the process so much easier and allowed me to more effectively adhere to my treatment plan.

Staying on top of scheduled infusions is not always an easy task. We all have those frustrating days where we just want to throw our hands up and walk away. Next time this happens to you, take a step back and try to better understand the factors contributing to your frustration. Pick up the phone, get online or reach out to your peers in the community for support and advice. Discuss your concerns with your treatment team and work together to identify solutions. Taking action will help put you on the path to better overall health, allowing you or your loved one to experience a better quality of life while also reducing cost to the medical system.

Today as an adult, I rarely miss a dose as I’m well aware of the consequences. Through my past experiences and by learning from continued interaction with my peers in the bleeding disorder community, I have found being proactive, as opposed to reactive, allows me to take better control of my condition. This gives me the opportunity to live my life the way I want (within reason of course!). The sooner we can learn this lesson and impart it to others, the better off we will be as individuals, caregivers and as a community. 🧠



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My Son, Michael

By Karen Bishop

I am not really sure where to begin... I feel like I blinked my eyes and my son is 19. He is a freshman in college and has more courage than anyone I have ever met in my life. He has taught me so much in the past 19 years. He has taught me what life is all about.

My husband and I knew when Michael was born and diagnosed with severe Hemophilia B, and subsequently diagnosed with an inhibitor, there would be challenges. Not ever facing anything like this before, we gathered all the information we could and joined support groups. Nothing can really prepare you for the heartache and obstacles that would incur. Just when you think you have yourself together emotionally, the dreaded insurance nightmare happens and another chapter opens up. It doesn't seem fair that you not only have to take care of your child with hemophilia as he grows up and is in and out of the hospital, you have to fight with insurance companies, too. I had to change jobs and figure out a way to keep him insured while trying to manage his care.

Probably the most difficult times in Michael's life, for me, was watching him go through so much physical pain. I don't think there is anything worse than to hear your son cry with pain and not be able to make it go away. I am a nurse and I like to help people feel better. I often felt helpless with Michael. It never seemed to be fast enough. I don't like it that he can't always walk, but I hate it when he hurts.

When I look back over these years, I know that what has happened in Michael's life has made him the young man he is today. Although as a child

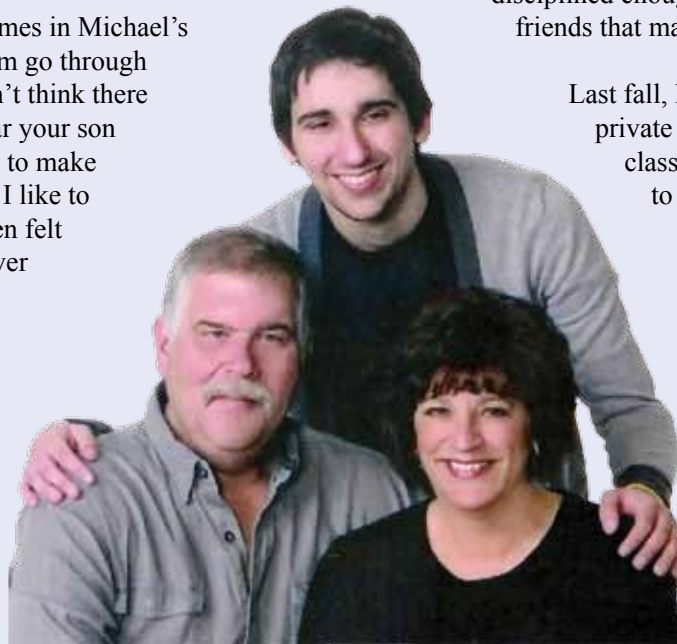
he could not play football and soccer or skateboard, he put all of his energy into art and music and writing. He has been drawing for as long as I can remember. He learned to play the guitar at a young age and he has always had a pen and paper in his hand. We always tried to provide him with the tools that would help him excel in the things that he could do and liked to do. He is currently majoring in Creative Writing and minored in Philosophy. He is also an excellent photographer. Michael has found things that he can do and has learned to do them well.



Finding the right school for Michael was very important. He attended a Catholic school through the 8th grade. The classes were small and the teachers always accommodating. High school was a bit more of a challenge. Locally, there is just one public high school. The school is very large and physically it was difficult for Michael, even with an IEP in place. He attended his freshman and sophomore year at the high school and then completed his junior and senior year at home with an online classes offered through the high school. Michael was disciplined enough and had a network of friends that made it successful.

Last fall, Michael started college. The private college he attends has small classes and a campus that is easy to maneuver. This has been a very good choice for Michael and he has adapted very well to college life.

Life is about accepting challenges and facing them. "Life isn't about waiting for the storm to pass; it's about learning to dance in the rain." I am so thankful my son came into my life and showed us all how to live. 🐶



The Story of Noah

By Angie Bennett

Our Miracle Baby...

My pregnancy with Noah was not easy. I suffered from placental abruption and from sixteen weeks, was in and out of the hospital, and on continuous bed rest. Although my doctor's goal was to get me to the 27th week of gestation, he didn't seem to hold much hope that the pregnancy would be successful. Much to everyone's amazement, I made it to 36 weeks and a day. My husband and I were ecstatic when our beautiful baby boy, Noah, was born on April 23, 2010.

Going into the pregnancy, we knew that hemophilia ran in my family, but I did not know whether I was a carrier. Since I was adopted, I wasn't fully sure of the odds. Before we left the hospital the next day, Noah was tested and we were told that he did not have a bleeding disorder. My husband and I were relieved, to say the least.

We soon arrived home with our new baby! What joy we felt to be home with our son. However, our joy soon turned to concern. His heel stick, which oozed at the hospital, was still bleeding. I continued to check it, but it didn't let up. I called the doctor and was advised to bring Noah to the emergency room. The doctor met us there and informed us that there are two main kinds of hemophilia and that Noah had only been checked for one of them. At that moment my heart dropped! I knew in the back of my mind he had hemophilia, but kept trying to convince myself that he was fine. Noah was tested again, and we waited for what seemed to be an eternity.



Our Son Has Hemophilia...

The moment we learned our son has hemophilia is as vivid as if it were yesterday. It was April 25, 2010, 3:17 am. In the emergency room of the Texas hospital, I will never forget the words my husband said to me that night. With a soft voice and tears in his eyes, he leaned close to me and said, "Angie, he has hemophilia." I immediately picked up my baby and cried. My husband left to call to our parents.

When he returned, he held our precious little boy so that I could go outside for some air.

As I sat on the steps of the hospital that warm, breezy night, I started shaking and just could not stop crying. In my heart, I knew our lives would never be the same. A stranger came up to me and hugged me. She told me everything would be okay, that it was in the Lord's hands and He would take care of us. I wiped my tears, took a deep breath and walked back into the hospital, where my son was now being admitted. My husband and I were exhausted. In my son's hospital room, I cried myself to sleep.

A couple of hours later the doctor arrived to speak with us. He proceeded to explain about hemophilia B, but I was still too upset and in shock to really concentrate on his words. Just yesterday, we were told that Noah did not have hemophilia! A social worker came in to introduce herself and talk to us as well, but all I felt like doing was crying. I remember feeling guilty that I had given this to my son. Everything just seemed to be a blur.

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Finding Support...

I did my research by watching YouTube videos and reading every article I could find about hemophilia. Guess what, I cried some more! We attended a local conference and for a while, we had hemophilia treatment center appointments every two weeks. Through the local hemophilia chapter, another mom contacted me and told me her son also had hemophilia B. We talked about what it meant to have children with hemophilia and she offered me hope and comfort. She will always be very near and dear to my heart. She was a rock at this point in my life and I will forever be grateful to her.

The decision I made to start networking through Facebook changed my life forever. On Facebook, a mom of two sons with hemophilia B reached out to me and guided me to groups of other parents with similar experiences. She put us in touch with The Coalition for Hemophilia B, who invited us to their annual symposium in New York. There, we found even more support and education.

My husband and I went from trying to explain hemophilia to friends and family, to finding a whole community who knew first hand what we were experiencing. I was full of questions for my



newfound friends. These “strangers” became my support system. The moms told me things would get easier and, at the time, although I hoped they were right, I really didn’t believe them. It did get easier though and now, with a couple of years under my belt, I find myself reaching out to other new mothers. I may not have a lot of experience, but remembering what I felt like not so long ago; I just want to be there to provide support.

With Gratitude...

For communities such as ours, I am grateful. Since our son’s birth, hemophilia has become a part of our lifestyle, and we accept it. The men and women in this community have taught us how to advocate for Noah. People who were once strangers have become friends and confidants. They are the ones who have patiently listened to me cry and vent, and have witnessed the proud moments in my life as I learn to take a stand for my son. I am happy to be part of this little community. Thank you for all so much for your support. You will always have a place in my heart as my family continues on our hemophilia journey. 🧡



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.

15+ YEARS[†]
EXPERIENCE
MATTERS

 **BeneFix**[®]
Coagulation Factor IX (Recombinant)
Room Temperature Storage

You asked for 3000 IU in a single vial with the same 5-mL diluent. You got it.

3000 IU IS HERE

The first 3000-IU dose for hemophilia B patients.



The individual depicted is not a hemophilia patient.
For illustrative purposes only.

What Is BeneFix?

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

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

Important Safety Information 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.

Should your doctor decide that BeneFix is right for you, you may be eligible to get a one-time 1-month supply

Up to 20,000 IU at no cost to you

Download the Trial Program Application today, then talk to your doctor to see if you are eligible.*



FreeTrialBeneFix.com

*One-time offer. Terms and conditions apply. Visit www.FreeTrialBeneFix.com for complete terms and conditions. You must be currently covered by a private [commercial] insurance plan. If you are not eligible for the trial prescription program, you may find help accessing Pfizer medicines by contacting Pfizer's RSVP program. For questions about the BeneFix Trial Prescription Program, please call 1.800.710.1379 or write us at BeneFix Trial Prescription Program Administrator, MedVantx, PO Box 5736, Sioux Falls, SD 57117-5736.

[†]BeneFix was approved February 11, 1997.

- 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.

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 for BeneFix on next page.

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December 2012





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For more information about our factor products for hemophilia, von Willebrand disease, and other rare bleeding disorders, or to learn about our innovative patient programs, please visit www.cslbehring.com or call consumer affairs at 1-888-508-6978.

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Industry News



Inspiration Biopharmaceuticals Files for Bankruptcy

Inspiration Biopharmaceuticals has filed for Chapter 11 bankruptcy and is seeking a buyer for their assets and technology. Inspiration has marketing applications pending in both the U.S. and Europe for a new recombinant factor IX product. However, their Phase III U.S. clinical study was recently placed on hold by the FDA after some patients developed antibodies against proteins produced by the Chinese hamster ovary (CHO) cells used to make the recombinant protein. Inspiration also has an active program for development a product for treatment of hemophilia A patients with inhibitors, as well as research and pre-clinical projects for several other recombinant coagulation factors.

Inspiration was founded by the fathers of two boys with hemophilia B. Their goal has been to develop lower-cost

clotting factor products to provide improved treatment options for bleeding disorder patients worldwide, especially in poorer countries. Until now, theirs has been an inspirational story (pun intended) seeming to show that even a small group of committed people can succeed in the world of pharmaceutical development. Although the CHO protein contamination should be relatively easy to solve by tweaking the purification process, the company apparently just ran out of money. This shows how difficult it can be to develop a new product, especially with the limited resources of a small company.

Sale of the company is now in the hands of the bankruptcy court. Hopefully, another company will continue their development efforts.



Pfizer - The Way You Log Is About To Change

Pfizer Hemophilia is excited to announce the launch of its first mobile logging tool, HemMobile™, a free mobile app designed to help hemophilia patients and caregivers using any factor replacement product log infusions and bleeds and stay aware of general health and wellness.

Developed with input from members of the bleeding disorders community, HemMobile™ was officially introduced at the recent National Hemophilia Foundation Annual Meeting in Orlando. HemMobile™ is currently available on an iPhone®, iPod Touch®, or iPad®. To download, visit the iTunes store from your iPhone®, iPod Touch®, or iPad®.

HemMobile™ can be password protected, and Pfizer will not collect any personal information unless users decide to enroll in Hemophilia Village, which means users have control over what information they share and with whom they share it. HemMobile™ gives users the option of backing up their information via iCloud® for easy retrieval.

HemMobile™ users can record the date, time, location, and reason for every infusion regardless of what factor is used. Users can also add notes related to each log, as

well as photos to each bleed log entry. HemMobile™ allows users to view infusion and bleed log history and send reports of this history to their care team. Pfizer factor users can capture the lot number, product expiration date and IU amount with their device camera for added convenience.

HemMobile™ allows users to set private reminders for infusions, doctors' appointments and factor re-ordering. Users can also create unique patient profiles for themselves and/or patients with hemophilia in their care.

We hope that you are as excited as we are about the launch of HemMobile™. We are proud to have worked with you in support of the changing needs of those living with hemophilia. If you have an advertising opportunity for Pfizer to purchase for our latest chapter ad or 300 word article announcing the availability of HemMobile™, please contact your local Pfizer Hemophilia sales representative.

For more information on HemMobile™ and to learn more about the Pfizer's commitment to the hemophilia community, visit us at www.hemophiliavillage.com. For the latest news from Pfizer Hemophilia, please join Our Hemophilia Community on Facebook.

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Industry News



Abbott

Abbott Laboratories

Promising Results in Abbott HCV Trial

Abbott Laboratories has announced promising results in their “Aviator” Phase IIb clinical study of a three-drug combination without interferon for treatment of hepatitis C virus (HCV) infection. Ninety-seven percent of patients who had never been treated for HCV infection and 93% of patients who had responded poorly to the standard ribavirin/interferon treatment showed a sustained elimination of HCV activity for at least six months after the end of treatment. The treatment lasted 12 weeks and was well tolerated with minimal side effects.

Hepatitis C is a serious liver disease that affects as many as 170 million people worldwide, including many hemophilia patients who received plasma-derived clotting factors manufactured before the availability of effective viral reduction methods. Today’s plasma-derived products are much safer, with no known transmission of HCV since the early 1990s. HCV transmission today is mainly through sexual contact, intravenous drug use, and accidental contact with contaminated body fluids. Hemophilia patients are now at no greater risk than the general population.

Until 2011, the only treatment for HCV infection was a combination of oral ribavirin and intravenous interferon taken for a year. It was only effective in about half of patients and came with side effects that were difficult for many patients to tolerate. Many of the side effects are due to the interferon, which is a natural immune-system

protein that the body produces to fight infections like the flu. The aches, chills and fever of the flu are actually caused by interferon, not the flu virus. In 2011, two new drugs were introduced, Incivek (telaprevir) and Victrelis (boceprevir), which are both protease inhibitors that block reproduction of HCV. These new treatments are shorter, but both still include interferon and ribavirin and still have significant side effects.

The Abbott treatment under investigation uses three drugs, a protease inhibitor, a polymerase inhibitor and an inhibitor of a viral protein called NS5A, all of which block production of new viruses. Similar combination therapies have been very successful in treatment of AIDS. Hitting a virus hard from several different directions appears to knock it down quickly before it can develop resistance to the drugs.

The Aviator study also included ribavirin with the three other drugs. Abbott is now planning a Phase III study of the three-drug combo that will also look at the need for ribavirin. Success of the Phase III study would give Abbott the data necessary to file a product license application with FDA for the drugs.

This is great news for patients infected with HCV. Several other companies are also working on new HCV treatments. Although it will still take a few years for these products to reach the market, there is hope in sight.



ATTENTION: Inhibitor Family Camp Some dates have changed!



The National Hemophilia Foundation has recently announced the dates of its 2013 annual conference. These dates conflict with one of our two Inhibitor Family Camp programs, which is set to take place at Victory Junction in North Carolina from October 3rd-6th. In order to allow our participants the opportunity to attend both programs, we have rescheduled our camp. The new dates for Victory Junction in NC have been modified to October 17th-20th.

Inhibitor Family Camp at Painted Turtle in California will maintain its regularly scheduled dates of April 19th-22nd.

We apologize for any inconvenience this may have caused. If you have any questions about this message or our programs, please feel free to contact us. Or, visit our Inhibitor Family Camp site: www.comphealthed.com

Thank you for your attention.

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To Blog or Not to Blog... What Exactly IS *Blogging*?

By Pam Williams

My assignment was to check out blogs related to hemophilia. I couldn't be more unsuited for this task, but I gave it the "ole college try" and went searching.

My first stop on the Internet train was to www.kelley.com. Anyone who has been involved in the hemophilia community is familiar with Laurie Kelley. She has written several books on bleeding disorders and she is active in promoting hemophilia worldwide. I logged on to Laurie's site, which led me to the HemaBlog tab. From here, you can find a section entitled *Hemophilia Blogroll*, where you will find a variety of blogs and stories ranging from vWD, moms, two brothers and even one entitled *Hemophilia is for Girls*, which is a blog about a woman with Factor 5. The home page lists other valuable topics to explore, including information on the Insurance Exchange and the Bayer Hemophilia Leadership Program.

I really wanted to find a hemophilia B blog, so I searched the topic and found www.wn.com/hemophilia-b, which lists the clinical trial and pending advances in the treatment of hemophilia B. I was unable to find a blog by a hemophilia B person, but I hope there's something out there and someone better at blogging than I am can locate it. The advantages of this site include videos, including one on the royal disease, and an images section where you can obtain a quick genetic lesson on hemophilia.

The next stop was to www.sweetaffliction.com. Here, you will find a blog about David and Andy, two men with hemophilia A. Categories on this site include: Health insurance, Hemophilia advocacy, Hemophilia fitness and personal stories. David and Andy each list their bios and their passion for hemophilia education through their blog.

www.Factorfacts.com can connect you with others with hemophilia. I didn't click on the "join us now" as I'm content with my Hemophilia B Facebook group; however, I did click on the "news" tab, which brought a drop-down for hemophilia blogs. Here you will

find Hemophilia Blogs from Around the World and Personal Hemophilia Blogs. This site is for people with hemophilia by people with hemophilia.

My final stop on the blog train was to www.thinksciencenow.com. I was intentionally looking to explore Bartholomew's BioNotes. Dr Bartolomew Tortello, Senior Director of Medical Affairs/Hemophilia at Pfizer updates his science listing every two weeks where his topics can range from *My IV Medicine Came From a Plant?* to *A Promising Way to Target Chemotherapy*. There is also information available regarding *Conducting Research and Clinical Trials*.



I don't know if I am comfortable with the idea of blogging - I'm still not sure of its use and why it's important to blog. I am not one who is going to log onto a site and just follow one person's trek through his/her life with hemophilia.

Is the information found on these blogs helpful to the hemophilia population as a whole? I see usefulness in the Laurie Kelley site as well as the *Think Science* site, but as far as the others, I probably wouldn't be interested or see any use in spending hours reviewing them unless I personally knew the person.

My only "blog" and I'm not even sure we can call it that, is our Hemophilia B/Factor 9 Support Group on FaceBook. Here, through a closed invitation-only group, those of us dealing with hemophilia B are able to ask questions, gain new insights and offer direction to each other. No question is too minor, and we've been known to discuss anything from the price of factor per unit to the benefits of toddlers wearing helmets when learning to walk. We talk about ports, emergency rooms and self-infusing. We share our stories as parents and patients with others who are just starting out in the hemophilia world. However our standing advice to all: talk to your medical care providers about your concerns and questions. Your medical team should be your first line of defense. 🦋

Never doubt that a small group of thoughtful, committed citizens can change the world; Indeed, it is the only thing that ever has.

~ Margaret Mead

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We are delighted to present
**The Coalition for Hemophilia B
First Annual Men's Retreat**
Created **BY Men with Hemophilia B
FOR Men with Hemophilia B**



Dates and details to follow soon!

Save the Date!

The Coalition for Hemophilia B



6th Annual Fundraising Dinner

Friday, March 22, 2013

Waters Edge Restaurant

401 44th Drive; Long Island City, New York

7th Annual New York Symposium

Saturday, March 23, 2013

Grand Hyatt New York Hotel

109 East 42nd Street; Park Ave.; New York, NY

The 2013 William N. Drohan Scholarship application is now available on our website. Please visit www.thecoalitionforhemophiliab.org to apply. The deadline is March 5, 2013.

We are now on Facebook!
Visit us under The Coalition for Hemophilia B



The *Factor Nine Group* moderated by Jill Lathrop is located on Facebook - search Hemophilia B Group

For back issues of **Factor Nine Newsletter** or for more information on research, please call or write to:
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