Factor Nine News

The Coalition for Hemophilia B Fall 2013

Topics in Hemophilia

Life and Times of Janya (Davis) Roland

As a child of 1960's, my life was safe, calm and wonderful in terms of the world. I was born into a loving and awesome family writhed with hemophilia.

Story continued on page 2

Life and Times of Janya (Davis) Roland continued...

At this time in the country all that was known about hemophilia was that it was a man's disease. Factor IX, which my family had, was diagnosed in 1952 by a physician who was studying a boy by the name of Stephen Christmas.

As a young girl, 3 years of age, I was covered with bruises from head to toe and my parents were worried. Upon examining me, my pediatrician told my mother, "I will have to say she is bruised more than the usual child." Although he knew my father had hemophilia, he never tested me for the disease because it was a "man's disease". He ran leukemia tests and I tested negative; therefore, I just bruised easily and that was it. Thank God I didn't have any major accidents as it would possibly been the end of my life.

When I grew up and married, I knew I was a carrier of factor IX, but didn't know much more than that. I had a daughter, now 25, with no difficulties and proceeded on with life. I got pregnant a year and a half later and carried that child for five months when it was determined that he was not viable. I was told my son had suffered a heart attack. I accepted that and continued on with life.

A year later, I conceived again and carried my daughter for four and a half months - -and the same thing happened. I knew there must be more to that being the reason, but had no proof. At that time I was studying to be a nurse and traveling back and forth to Memphis, Tennessee. I decided to get involved with the Tennessee hemophilia foundation and was a member of the board at the age of 24. I learned a lot, but as life took over, I changed degrees and conceived again. This time another sweet daughter was born, but there were complications. I had to have a c-section. All went well with her during the delivery, but for me the bleeding was great! After a month and a half, it slowed down and eventually stopped. I thought it was bad, but never gave thought to the possibly that hemophilia was the cause of this lengthy bleeding episode.

As life continued I divorced and moved to Memphis, where I met the love of my life. We married and wanted another child since my husband did not have any children. Two years after we married, we finally conceived and were elated to say the least. We held off telling the family, but that didn't work, because it was a tubal pregnancy and I had to end the pregnancy. I knew in my heart that was not what I was supposed to do, but for my safety I took the shot to terminate the pregnancy on September 11, 2001 and went home The U.S was accosted and so were our lives. As the days progressed nothing seemed to be happening. On September 24th my fallopian tube ruptured and I was rushed into surgery. Three days after surgery, I went home and proceeded with life... so I thought! A friend was sitting with me while my husband and children were working and at school. I was in the bed, but got up to go to the bathroom when I noticed blood dripping on the floor from my incision. I screamed and my friend came running. I kept bleeding more and more. She took me to my doctor and they proceeded to sit on my abdomen and push the blood out of my incision. After filling up twenty blue bed pads, they sent me home to bath twice a day and push the blood out in the tube until it stopped. This continued for a week. To say the least, it was nasty and terrible!

As the years went by, so did our determination to have a child. However, I ruptured a disk in my back, my L5, and needed surgery. I had the surgery with no complications. At this time in life, we had found out that my father had pancreatic liver cancer and he passed away in February 2005. We still wanted a child and had even discussed adopting. Again, my L5 ruptured and another surgery was in the works. I scheduled my surgery, but truly felt that I was pregnant and needed to take a pregnancy test. IT WAS POSITIVE! I put off the surgery. God was good and protected this child.

On November 10, 2005, our son was born. I knew he would more than likely have hemophilia. Upon his arrival, we dove into the hemophilia world and have not stopped. We attend every meeting possible and absorb all the information out there. We got pregnant again a year after our son's birth, but lost our daughter to other complications with the pregnancy. After this, we, with another family, began a support group for people with hemophilia in the Memphis area.

When our son was three years old, our pediatric hematologist at St. Jude, Dr. Reiss, asked us if we would be a part of a CDC study. They desired to study the gene and how hemophilia IX was linked. They wanted to test my daughterd, to see if they carried this gene as well. After the study, much to our surprise, all three of our children fwere ound to have hemophilia. WOW, it wasn't just a male disease! It was a huge surprise that we not only had factor IX, but also factor VIII and vWD. Amazing! With these diagnoses, it made us eager to obtain more education about factor VIII and vWD in order to help our children. My husband attended all the insurance seminars, Parents with Children with Hemophilia, etc., as I absorbed all the factor seminars.

While sitting in these seminars, I realized that they were describing my symptoms. My life was a history book of bleeding! How could this be, I only carried factor IX, so I thought. After sitting in many meetings, my sister and I went to see our father's hematologist, Dr. Dugdale, and

she found we both had vWD. What a shock, but at the same time a relief! Maybe now I could get some help for my monthly cycles. My thoughts went to "so that's why I almost lost my life during the tubal pregnancy," so much was being answered! They also told me that their office was studying my family and had documented it since as early as the mid-19th century. They continued after our diagnosis and found that Factor VIII and vWD was had also been in my family.

During this time my husband and I still wanted more children and began to see an infertility specialist, Dr. Kutteh. He was made aware of my diagnosis and wanted to do further testing. So we proceeded and he found that I also had factor 2 prothrombin and factor 5 Leiden. He told my husband and me, "You are a strange cookie!" We continued trying to get pregnant and he said that a small dose of heparin would possibly keep me from miscarrying. I asked him if he thought that maybe I threw a clot to my second and third children's hearts and if that's why their heart stopped, and he said yes. I can't tell you what a relief to know the real reason I lost them.

With all of this knowledge now, I tried very hard to get help with my bleeding. To no avail, after seeing seven hematologists from the Mississippi River to the Tennessee River. I was exhausted and worn down from all the naysayers because of my factor 2 and factor 5 diagnosis. My husband too was stressed and frankly tired of the out-of-network costs while trying to find a physician that would help his wife. He knew the stress I was under and the heartbreak I felt with every denial to help me with my bleeding. I was kept hostage in my home for days during each month because of my bleeding. My life was held back from all that I could be doing. That was the story, until my dear husband found an oncologist/hematologist that was new in our home town of Collierville, Tennessee. He urged me to give her a chance and I am so glad I did. I proceeded to see Dr. Margaret Gore of Integrity Oncology.

On my first visit, she simply sat and listened to my story, while studying my file and history. Something that had never been done! She was stumped by an iron deficiency she saw in my file. She requested to draw blood to see if it was still this low. I was called back two days later for the reading of the results. When I returned, she came in my room with her mouth open and said, "You only have 2% serum iron in your body." I said, "Okay, what does that mean?" She explained that you have daily iron that most blood test show and it's usually fine, but the serum iron is in the blood cells and its normal range is 60-150 and "yours is only 2%!" She continued to say, I could have died if I had gotten severely hurt or had a wreck! She was not letting me

leave her office without someone else driving and I was to return the next day for a 5-hour infusion of pure iron. I followed that with persistency and had finally found my hematologist! It was truly amazing!

As I sat in her office the next day being infused I conversed with her chemo patients. They wanted to know my story and I sure had plenty of time to tell it. They too were amazed that I was still living after all of that. When the day was over, I truly felt 25 all over again! My energy was surprising and I now can ride in the car on a long trip and not fall asleep in the first 15 minutes; I can now watch a movie completely through. I didn't know what I was missing because my whole life was that way.

After that was taken care of she began to study my disorders to find the conclusion that my bleeding overpowered my clotting at certain events in my life therefore, she was not afraid to help me with my bleeding. She prescribed two doses of Stimate every month and that miraculously help my quality of life! After that, I had to have achilles tendon repair surgery and I was given DDAVP before my surgery - there were no complication ,only minor bleeding for a few days, which was normal.

I know you know the old saying, "Oh what a relief it is!" Let me say, it truly is so wonderful to finally have a productive life, content in all that I am and do. Let me be truthful, the hurt is still there and always will be for the four I have lost, but I know that they are with God and He must have needed them for His purposes. We did not get pregnant and that's okay because we now are going to foster and adopt. God does use our infirmities to grow our faith. Mine sure has!

The only FDA-approved recombinant factor IX indicated for routine prophylaxis to treat adults with hemophilia B[1]

For more information, contact your Baxter representative today:

Danielle Kiesel

Phone: **(862) 881-9889**

E-mail: **danielle_kiesel@baxter.com]**

To learn more, visit www.RIXUBIS.com.

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]

Indications for RIXUBIS [Coagulation Factor IX (Recombinant)]

RIXUBIS is an injectable medicine used to replace clotting factor IX that is missing in people with hemophilia B (also called congenital factor IX deficiency or Christmas disease).

RIXUBIS is used to prevent and control bleeding in adults with hemophilia B. Your healthcare provider may give you RIXUBIS when you have surgery. RIXUBIS can reduce the number of bleeding episodes in adults when used regularly (prophylaxis).

Detailed Important Risk Information for RIXUBIS [Coagulation Factor IX (Recombinant)]

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

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

You can have an allergic reaction to RIXUBIS. Call your healthcare provider or get emergency treatment right away if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.

Your body may form inhibitors to factor IX. An inhibitor is part of the body's defense system. If you form inhibitors, it may stop RIXUBIS from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor IX.

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

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

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

Please see Brief Summary of RIXUBIS Prescribing Information on following 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.

Reference: 1. RIXUBIS [Prescribing Information]. Westlake Village, CA: Baxter Healthcare Corporation; June 2013.

Baxter

Ed Wilson, a dedicated member of our community, passed away on Sunday, November 17, 2013 He was 71.

Ed was a wonderful person and always had a big smile on his face. He was very active in our community and had a great passion for advocacy. Our thoughts and prayers go out to his wife, Catherine and family on his passing. We will miss him greatly.

Godspeed Ed.

Ed Wilson
November 17, 2013

Dr. David Clark, Chairman of The Coalition for Hemophilia B welcomed everyone in attendance at our Factor Nine On The Road Family Meeting, which took place on September 28th at the Chicago Marriot Suites Hotel in Rosemont, Illinois. The meeting was sponsored through the generous support of Pfizer, Inc.

Interesting and wonderful speakers spoke on several timely and important topics regarding *Insurance*, *Win-Win Negotiations*, *Exploring Mental Health*, *Genotyping* and *Hemophilia Updates*. From all of us at The Coalition of Hemophilia B, it was wonderful to each of you that took the opportunity to join us! We look forward to providing more programs for you in 2014!

We were delighted to see so many of you at our Family meeting held on Saturday, October 5, 2013 in conjunction with the National Hemophilia Foundation 65th Annual Meeting in Anaheim, California. Special thanks to Pfizer, Inc. for their generous support of our meeting!

The CSL Behring

The weekend my son and I spent attending CSL Behring's "Getting' in the Game Junior National Championship" will go down as one of my son's favorite weekends of his life. Yes, he is only 9 years old, but that does not diminish his experience. It built confidence, it improved his golf game, but the most important thing he learned is that hemophilia will not limit his opportunities in life.

We arrived in Phoenix midday on Friday, a few hours before the golf clinic later that afternoon. That gave me the opportunity to treat my son to a small taste of my childhood – lunch at In-N-Out Burger. After the quick meal, we headed back to the Sheraton Wild Horse Pass Resort (which we learned was located on an Indian Reservation) and rested at the pool. My son loved the two story waterslide, as did a number of the other kids attending the event.

Later that afternoon, we arrived at the Whirlwind Golf Course, part of the resort. It was a beautiful desert course (given our surroundings, we were warned many times to not look for lost golf balls in the shrubs because of the rattlesnakes!). I was a little jealous that I was not the one playing this weekend.

At the clinic, golf professional Perry Parker started with all of the kids around the driving range talking about the importance of stretching and other golf basics. Stretching and an overall healthy and active lifestyle is important to everyone, but he stressed it was especially important for those with bleeding disorders.

After a brief introduction, the field of golfers was split into three groups to work different stations during the clinic – chipping, putting, and the driving range for swing analysis. Every station had great teaching pros and plenty of help from the volunteers from CSL Behring.

After the clinic, we were all treated to a dinner at the hotel, where Perry and his baseball counterparts spoke about the weekend and what other participants have learned at this event in previous years. Since this has been a popular event for a number of years, there are now kids helping with the event who had participated in previous years. This was another great way to connect with the younger kids.

The golf competition started Saturday morning. There were three players per group, and CSL Behring volunteers served as personal caddies – they even had caddie bibs

Gettin' in the Game
Junior National Championship Experience

By: Blake Pera

with player's names on them. It was very cool, and John V was pumped to see his name on the bib. John V's caddie was incredibly nice and patient. He was also very helpful throughout the round, having played golf at Michigan State. His insight, along with Perry's pointers the day before, really paid off. Not every shot was perfect, but the kids had a great time, and John V later told me that he had one of his best experiences golfing at this event. After the round while we were alone in the clubhouse, he told me "I had so much fun. I cannot stop smiling, and I don't know why!" That statement really got me, and I was so pleased that he had such a great time.

After lunch, the kids broke off into a series of rap sessions, while the parents attended similar sessions. Initially, John V did not want to attend the rap session, as he wanted to go to the pool. After the session, he was again smiling ear-to-ear, and he finally admitted that he had a great time interacting with the other kids and engaging in the conversation.

The dinner Saturday night concluded the weekend's activities. My son continued to have a great time throughout the day, making new friends and playing a sport he enjoys. After dinner, every participant received medals and a certificate, and they also gave trophies to the top three performers for golf and baseball. To John V's surprise, he placed second in the tournament! He was so pumped; it was hard for him to go to sleep that night.

The improvement in John's golf game from Friday's clinic to the end of Saturday's round was great to watch, but the overall experience he had this weekend is what truly made it so special. He is already asking how he will be able to attend next year!

My son and I cannot thank everyone at The Coalition for Hemophilia B and CSL Behring for this tremendous opportunity!

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HEMOPHILIA

Here's to your defining moments

Living with hemophilia isn't just about hemophilia, it's about life … and the incredible moments that define it. This understanding is the driving force in everything we do, whether developing new therapies or creating meaningful programs.

So here's to you and to the defining moments that inspire all of us.

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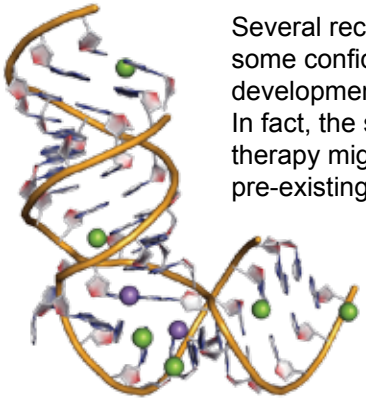
Hemophilia B Gene Therapy May Reverse Inhibitor Development

by Dr. David Clark

Gene therapy may represent an eventual "cure" for hemophilia, but one big question is whether the method will work in patients with inhibitors. Inhibitors are antibodies that the immune system produces because it thinks infused factor is a foreign protein that needs to be eliminated from the body. The inhibitor antibodies neutralize the infused factor and prevent it from working. Only about 3 - 5% of hemophilia B patients develop inhibitors, but the consequences for those who do can be significant.

One of the major concerns with gene therapy has been whether the factor IX produced by the newly-introduced gene would also cause inhibitor development or worsen inhibitors that are already present. This could be especially dangerous since many hemophilia B patients who develop inhibitors also develop anaphylaxis, severe allergic reactions to factor IX (FIX). Since current gene therapy methods cannot stop the production of FIX once it starts, such a severe reaction could be very serious.



Several recent publications provide some confidence that inhibitor development might not be an issue. In fact, the studies suggest that gene therapy might actually eliminate pre-existing inhibitors. So far, this result has only been demonstrated in animals, but it does provide hope that the same effect will eventually be seen in humans.

The research groups used mice that have been bred to produce inhibitors and develop anaphylactic reactions to human FIX. They then performed gene therapy on the mice using viral vectors that target the liver. The gene therapy methods insert copies of normal FIX genes into liver cells, which cause the cells to produce human FIX. They monitored the production of FIX and the concentration of the anti-FIX inhibitor in the mice over time. Over several weeks, the groups saw the elimination of circulating inhibitor antibodies and corresponding increases in FIX activity levels in the bloodstream. The findings suggest that the immune system's response to FIX secreted from liver cells is different from the response to infused FIX. The treated animals also no longer had inhibitor-like reactions to infused FIX.

The researchers also compared gene therapy to immune tolerance induction (ITI) in the mice. ITI is the most effective method currently known for elimination of inhibitors. It involves infusing large daily doses of factor until the inhibitor disappears. It is effective in about 60% of hemophilia A inhibitor patients but only in 15 - 30% of hemophilia B inhibitor patients. It is expensive, demanding and, especially for hemophilia B patients, entails the risk of developing kidney damage or severe allergic reactions to FIX. The gene therapy techniques actually worked better than ITI in the mice. They were safer and caused no anaphylactic reactions, which were seen in some of the mice on ITI.

These results suggest that rather than being a danger to inhibitor patients, gene therapy could actually be a benefit by eliminating their inhibitors. This adds to the hope that gene therapy might ultimately be a cure for hemophilia.

Treatment News

The news this quarter involves two new companies hoping to commercialize gene therapy techniques for treatment of hemophilia B.

DIMENSION THERAPEUTICS

Dimension Therapeutics, a new start-up company, is collaborating with Regenx Biosciences on gene therapy for hemophilia B using Regenx's NAV adeno-associated virus system for gene delivery. The NAV vectors include advanced regulatory gene sequences for improved control of factor IX expression.

Spark THERAPEUTICS

Spark Therapeutics was founded by Children's Hospital of Philadelphia (CHOP) to commercialize their gene therapy work. Spark/CHOP currently has an ongoing Phase I/II clinical study for hemophilia B.

AlphaNine® SD
Coagulation Factor IX (Human)
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INTRODUCING

The AlphaNine® SD Savings Card Program

Designed specifically for the needs of patients with hemophilia B

You could save up to **$500 per month** on the costs of your prescription for AlphaNine® SD (coagulation factor IX [human]).

Restrictions apply—see inside to determine if you qualify.

Please see Important Safety Information about AlphaNine® SD on back and refer to accompanying package insert for complete prescribing details.

GRIFOLS

GRIFOLS

A history driven by innovation

By Mallory O'Connor

For more than 70 years, Grifols has been pioneering many of the methods used to collect human plasma and transform it into plasma-derived medicines for individuals with rare diseases.

From its inauspicious beginnings in 1910 as a small laboratory and clinic in Barcelona, Grifols has been guided by the ethics, compassion, and innovative spirit handed down through three generations of the Grifols family. Grifols was founded by Dr. José Antonio Grifols in 1940, in Barcelona, Spain in the aftermath of the Spanish civil war. Laboratorios Grifols specialized in the creation of tools for clinical analyses and in the process of blood transfusions. Grifols opened Spain's first blood bank in 1945 and was the first company in continental Europe to produce single-donor lyophilized plasma.

Laboratoriou Grifols Blood Bank, 1945

In 1951, Dr. Grifols' son, José Antonio Grifols, invented plasmapheresis – a technique to obtain plasma now used worldwide that has made it possible to safely collect adequate quantities of plasma for large scale production of plasma therapies.


José Antonio Grifols Lucas (right) and Dr. Edwin J. Cohn (left) attending the International Blood Transfusion Congress, 1951

Over the years Grifols has continued to be the leader in the industry. For instance Grifols incorporated HIV antibody testing of plasma before it was compulsory. Grifols also established PediGri®, the only program in the industry to offer a comprehensive quality and safety history of each product.

Grifols has maintained its strong family heritage with the grandson of Dr. José Antonio Grifols, Victor Grifols, serving as President of the organization. Today, Grifols is a leading producer of plasma therapies, with a presence in more than 100 countries and is the world leader in plasma collection, with 150 plasma donation centers across the US. Grifols has a portfolio of plasma medicines in multiple therapeutic areas, including: pulmonology, hematology, immunology, neurology, infectious disease, and shock and trauma.

Today, Grifols continues its tradition of developing and producing innovative products designed to improve human health. Grifols is committed to increasing patient access to its life-saving plasma medicines through significant manufacturing expansions and the development of new therapeutic applications of plasma proteins.

To learn more about Grifols visit www.grifols.com

Message From Kim...

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! 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!!!***

B IS FOR BELONGING

Kim Phelan is one of those rare individuals whose compassion is matched only by her passion. She is the Vice President of the Coalition for Hemophilia B, and has been there from the beginning over 20 years ago. Kim has worked in every aspect of the Coalition, from advocacy to fund-raising.

Dr. Dave Clark has served as Chairman of the Coalition for the past 10 years. Dave has been writing much of the technical content of our newsletters for the past 20+ years. Kim says, "He has a wonderful gift for making complex scientific concepts easy to understand." Dave is a researcher who has been involved with factor IX since 1982, when he joined the American Red Cross Plasma Derivatives. He values the opportunity to contribute to the work of the Coalition and to work with the wonderful people in the hemophilia community.

Wayne Cook has been President of the Coalition for the past 10 years, and has been advocating for the community during that time. "I was always helping someone in the community, whether it was my chapter or a blood brother who needed someone to talk to," recalls Wayne.

Joyce and John Taylor founded the Coalition in 1990 after their son John was diagnosed with a severe form of hemophilia B. Finding it difficult to obtain much information on treatment options and living with hemophilia B, they recognized the need for an organization to serve this community. John flew all over the world and met with the top scientists in the field to learn all he could about hemophilia B and how patients could have a better quality of life. The Coalition also began to fund research and product development.

"It's wonderful when we see them again and they are more at ease and confident. It's priceless!"

Kim Phelan, Vice President of the Coalition, and Wayne Cook, President of the Coalition.

Today, the Coalition's "Factor Nine Newsletter" provides timely information on current events in the world of hemophilia B. The Newsletter includes research and inspirational stories of those living with hemophilia B, where our next meetings will take place, and timely information so members and their families are well informed.

EDUCATION IS POWER

The Coalition just celebrated its 7th annual New York Symposium. The meeting was successful in both educating our community and bringing families together from all over the nation. "It's very empowering and everyone walks away the better for it," notes Wayne. "Many types of people attend, and it is very special to see them bond." To help families attend the symposium, the Coalition holds a lottery drawing and flies in the winners. The families that win the lottery also join the Coalition at the Fundraising Dinner held the night before. It is important for the industry to see the people they are helping.

In addition, the Coalition holds several Factor Nine Family Meetings over the year throughout the United States. The meetings are educational, and a game we created called "Are You Smarter Than Your Hemophilia?" makes it fun to learn. Families always learn something new (especially because of Dave's scientific questions)! During these meetings, we hold a support group that is very helpful—especially for families with no prior history of hemophilia B, who make up about 30 percent of those diagnosed. Having children with hemophilia can put a strain on marriages. In fact, couples are more likely to divorce when their child has a severe health problem than couples with healthy children. By including our support groups in the meetings, we've created an "extended family" for our members. We encourage new members to exchange phone numbers with established members so they are never alone. As Kim puts it, "It's wonderful when we see them again and they are more at ease and confident. It's priceless!"

Dr. David Clark

INFUSING NEW IDEAS

This year, the Coalition had something new to be excited about—our first annual men's retreat held with educational support from Pfizer. Wayne explains, "The concept grew out of a simple question: What happens to the baby boomers, who have always been cared for by someone else, when they become their own caregivers, and even the caregivers for others later in life?" We wanted to help older men like Wayne expand beyond their comfort zone. Wayne adds, "We want to take them beyond hemophilia and infusion. What about high blood pressure, diabetes, joint problems, depression and other co-morbidities? How do we manage co-morbidities? How do we manage going into retirement dealing with our health care and the prospect of caring for our loved ones?" These are pressing questions for all of us. We hope that the success of this retreat will continue to help this special community into the future.

While the community has a camp for kids, retreats are also important. Kim points out, "When the information on the retreat was sent out, we received many requests to hold them for the younger adults and women with bleeding disorders. We've heard you. Please be patient; retreats for these groups will be coming down the road!"

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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.

BeneFix 3000 IU

The first 3000-IU dose for hemophilia B patients.

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

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

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†BeneFix was approved February 11, 1997.

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.
- 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.

Wyeth®
Manufactured by Wyeth Pharmaceuticals Inc.

Marketed by Pfizer Inc.
December 2012
Pfizer

In addition, we are working on a new monthly video series called "The B Scene," which will be posted on the Coalition's website, Facebook page, and YouTube, featuring people with hemophilia B and their stories.

GET INVOLVED. STAY INVOLVED.

The Coalition takes every opportunity to be involved in lives of our members and their families. Kim says, "We are extremely family-oriented because hemophilia is a disease that affects the whole family, even for adult patients. With our hands-on approach, many members and their families know they can call us day or night."

Several of our programs are designed to help patients through financial difficulties, such as the Patient Assistance Program, the Eshton Hewitt Quality of Life Memorial Fund, and the William N. Drohan Scholarship Fund. Our Factor Nine Holiday Program has been helping families in need by providing holiday gifts, food, clothes, boots, and more. We also conduct surveys to see the changing trends with our members so we are able to meet their needs and address concerns.

Plus, we exhibit during the year at several chapter locations and major national conferences, participate in NHF Washington Days, and always reach out to work with other organizations that are part of this close-knit community.

INSPIRING MINDS

The Coalition brings together some of the most dedicated professionals in the hemophilia community for our members. For example, this year's symposium featured Dr. Chris Walsh and Patrick Torrey. Dr. Walsh is Director of the Hemophilia Program at the Mount Sinai School of Medicine and a knowledgeable keynote speaker. Patrick Torrey is Director of the Leading Edge adventure education program, who talked to members of all ages about getting out of their comfort zone. Wayne adds, "There were sessions on everything from insurance to adherence to pain management."

For over 20 years, our mission has been to empower hemophilia patients and their families to act as advocates on their behalf, and to ensure that they have timely information about their health care so they can enjoy the highest quality of life possible. Kim adds, "We continually challenge ourselves to grow and change to meet the needs of the community. According to our member surveys, we're doing a pretty good job."

To learn more about what the Coalition for Hemophilia B can do for you and your family, visit www.CoalitionForHemophiliaB.org.

What Everyone Needs to Know About Health Insurance Marketplaces

By Michael Bradley, Vice President, Healthcare Economics and Reimbursement, Baxter Healthcare Corporation
Kim Isenberg, Senior Manager, Reimbursement and Advocacy, Baxter Healthcare Corporation

The historic health insurance reform legislation known as the Affordable Care Act (ACA, also called "Obamacare") was established in large part to provide health care coverage to as many Americans as possible. Many people living with Hemophilia B are already benefiting from some of the key provisions of the law, such as the elimination of pre-existing conditions provisions imposed by health plans, elimination of annual and lifetime caps on essential health benefits, and the extension of dependent coverage up to age 26[1].

Despite attempts to repeal the ACA, the federal government and many state governments continue to press forward to full implementation. In 2014, one of the final provisions of the ACA will take effect –consumers in every state will be able to buy health insurance from qualified health plans through online marketplaces (or exchanges). Open enrollment (selecting a plan) for these marketplaces began October 1, 2013 with coverage that starts January 1, 2014[1].

While federal and state government officials are still finalizing the details of the marketplaces, there are key provisions that every consumer should know. Healthcare reform is a confusing topic for many, and the goal of this article is to outline the most important changes that will occur in 2014.

Individual Mandate

Along with the ACA guarantee that no United States citizen or legal immigrant can be denied healthcare coverage because of a pre-existing condition comes the requirement that everyone must have minimum essential health insurance or pay a penalty starting in 2014. This provision of the ACA is often referred to as the "individual mandate." Potential penalties apply to each family member. For example, an uninsured adult over the age of 18 could be fined $95 or one percent of his or her annual family income, whichever is higher, when filing a 2014 tax return. The penalty for an uninsured child is half that of an adult. Certain individuals are exempt and will not have to pay a penalty[2] The Kaiser Family Foundation website (kff.org) offers extensive, "consumer-friendly" information on the ACA, including details on the individual mandate.

An individual that has health insurance through one of the following means satisfies the individual mandate requirement:

- Employer-based coverage
- Coverage purchased on the individual market
- Medicare
- Medicaid or other state children's health insurance plan
- Military coverage through TRICARE & Veterans Affairs
- Private health plan obtained through a state's marketplace/exchange [3]

Eligibility to Purchase Insurance through a Marketplace

In 2014, the marketplaces will be available for people who need an individual insurance plan, including people who work at businesses that will not provide employer-based coverage in 2014. If you are on Medicare or Medicaid, a state children's health insurance plan (CHIP), or have military coverage, there is a good chance that you will not need to access the marketplace to enroll in a private insurance plan[3].

The marketplace that is available in your state will provide a single application to determine eligibility for each household member. (Note: Undocumented immigrants are not allowed to apply for coverage through the marketplace.) The application will also determine if any individual qualifies for financial assistance in the form of premium tax credits and cost-sharing subsidies[4]. Each state marketplace will provide consumer assistance to help individuals compare plan choices and pick the best fit based upon price, provider network and quality score. Individuals can seek assistance online, by phone or through mail. The ACA requires each marketplace to also provide live, in-person help to consumers[5].

In order for a health plan to qualify for the marketplace it must:

1. Cover "essential health benefits" (EHBs), which are benefits that every plan must offer.
2. Provide four different levels of coverage that will allow you to choose a plan that is affordable and right for you.
3. Limit cost-sharing requirements, which means that you will not have to pay unlimited out of pocket expenses.

Essential Health Benefits (EHBs)

You can expect to read and hear a lot about EHBs in the news media in the coming months. The ACA provides for 10 broad benefit categories that must be included, at a minimum, in EHBs. Some states may require additional benefits beyond what the ACA requires, but the 10 "essential" benefit categories include:

- Ambulatory (outpatient) services
- Emergency services
- Hospitalization
- Laboratory services
- Maternity & newborn care
- Mental health and substance use disorder services, including behavioral health treatment
- Pediatric services, including oral and vision care
- Prescription drugs
- Preventive and wellness services and chronic disease management
- Rehabilitative and habilitative services and devices4

Choice of Plans

Each marketplace is required to offer four levels of plans based on the amount of coverage and payment that they provide, as well as different approaches to access to care, such as allowing people to choose from a larger number of healthcare providers. Each level of plan is designated with a precious metal from lowest (bronze) to highest (platinum).

PLAN	PLAN PAYS (for medical costs)	MEMBER PAYS (cost-sharing)
BRONZE	~ 60%	~ 40%
SILVER	~ 70%	~ 30%
GOLD	~ 80%	~ 20%
PLATINUM	~ 90%	~ 10%

As you might expect, as the benefits and coverage offered through the plan increases, so does the monthly/annual premium. For example, the Platinum plan will typically carry a higher monthly premium than the other plans.

Individuals under 30 years old and some people with limited incomes can choose a fifth option, known as a "catastrophic" health plan. A catastrophic plan is designed to protect people from worst-case situations. They typically require the insured to pay all medical costs up to a certain amount, usually several thousand dollars. These policies generally have lower premiums than a comprehensive plan, but coverage only begins once you have exceeded the annual deductible set by the plan[4].

Premium and Cost-Sharing Assistance

Similar to many employer-based health plans, the ACA provides for cost sharing, such as deductibles, co-payments and co-insurance. The ACA limits the amounts that health plans can charge you for health insurance coverage. Annual cost sharing (the amount you must pay before full insurance payments kick in) will be capped, and the amount of the cap is based upon the plan you select. In addition, financial assistance will be available for low- to moderate-income individuals in the form of premium tax credits and cost-sharing subsidies to help people pay for coverage provided by a marketplace-qualified health plan. Now is the time to do your research.

Remember, open enrollment began October 1, 2013 for coverage that starts January 1, 2014. There are many components of the ACA that are beneficial to the bleeding disorders community. Learning about your state's insurance marketplace will help you understand if this is an option for you and your family. If you qualify for marketplace enrollment, then knowing more about your choices will make you better prepared to enroll in a plan later this year.

For more information, including eligibility requirements, visit healthcare.gov or call your state's Department of Insurance. You can also learn more about health insurance and Baxter's CARE (Coverage, Assistance, Resources, Education) program by visiting nava.baxter.com or calling 1-888-BAXTER-9.

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Living with Hemophilia

by Dr. David Clark

Living with hemophilia has a significant impact on people's lives, yet there has been only a limited amount of research to look at those effects. The HERO (Hemophilia Experiences, Results and Opportunities) study is a large ongoing international analysis of the experience of living with hemophilia. It is examining hemophilia's effects on interpersonal relationships, careers, access to care and quality of life. It is the largest-ever multi-national study of psychosocial issues in hemophilia. The study is supported by Novo Nordisk.

The study includes a review of the existing literature and interviews and surveys with people with hemophilia (PWH) and parents of children with hemophilia (CWH). The interviews and surveys were conducted in ten countries: Algeria, Argentina, China, Canada, France, Germany, Italy, Spain, the United Kingdom and the United States in 2011 and 2012. Some highlights of the study's findings so far include:

Relationships of PWH

- 65% of PWH were married or in a long-term relationship, but 31% reported that hemophilia affected their ability to develop close relationships with partners.
- 78% of PWH reported being satisfied with their overall sexual relationship.
- 51% of those in long-term relationships reported that hemophilia negatively affected their sex lives. The most common reasons for the negative effects were infection with HIV/HCV (79% of respondents) and limitations in movement (50%).
- Many reported receiving helpful advice from their Hemophilia Treatment Center (HTC).
- Most PWH were satisfied with the support of their partners (93%) and family (88%).
- 66% of PWH disclosed their hemophilia to most or all of their friends.
- 25% reported having at least one negative experience telling a friend about their hemophilia.

Families of CWH

- 84% of parents of CWH were married or in a long-term relationship.
- Most parents of CWH were satisfied with the support of their partners (93%) and family (83%).
- Parents were generally satisfied with the support they received outside of their partner and family.
- The most common reason for dissatisfaction was the outsiders' lack of knowledge about hemophilia.

Continued on page 21

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- 65% of parents reported that hemophilia has not affected their relationship with their CWH.
- 66% reported that their child's hemophilia does not prevent them from having the types of family holidays that they want.
- 54% of parents felt disappointed that their child has hemophilia.
- 73% of mothers reported sometimes feeling guilty for passing on hemophilia to their child.
- Only 54% of fathers reported that the mother sometimes felt guilty for passing on hemophilia.
- 41% of mothers reported knowing their carrier status before the birth of their first CWH.
- 91% of parents thought their daughters should be screened for their carrier status.
- Parents reported that most or all of their CWH's friends (75%) and teachers (85%) were told about their child's hemophilia.
- 54% of parents reported that their child's hemophilia does not affect his relationships with his school friends.
- 39% of parents reported that they or their child had had a negative experience telling a friend about their child's hemophilia.
- 25% of parents reported that their child's hemophilia has made them feel isolated at school.
- 66% of parents also had unaffected children. The effect of hemophilia on the unaffected siblings was reported as no effect (19%), a negative effect (26%), a sometimes positive/sometimes negative effect (50%) or a positive effect (3%).
- The reported positive effects included more responsibility (56%) and greater closeness among siblings (41%).
- The reported negative effects included the other children not receiving as much of the parents' time (46%) and resentment at the attention given to the CWH (39%).
- 76% of parents reported that hemophilia had a negative effect on their unaffected children's lives.

Employment

- 76% of PWH reported that hemophilia had a negative impact on their employment.
- 41% of those reported the impact as small, 34% as moderate and 25% as large.
- 59% of parents of CWH reported that their child's hemophilia had a negative impact on their employment.
- 55% of those reported the impact as small, 32% as moderate and 14% as large.
- Many reported receiving helpful employment advice from their HTC.

Pain

- 89% of PWH reported that pain had interfered with their daily life.
- 50% reported dealing with constant pain.

Psychological Support

- 22% of PWH reported receiving psychological or counseling support in dealing with their hemophilia.
- 24% of parents of CWH reported receiving psychological or counseling support in dealing with their child's hemophilia.
- Most reported that they found the psychological or counseling support helpful.
- 33% of PWH reported that they would like to have received psychological or counseling support.

This is just a snapshot of the study findings. The complete analysis is very complex and is still being developed. One significant point is that many people have been able to get help in dealing with the impacts through their HTC or other healthcare provider. It may feel overwhelming at times, but remember you are not alone in this.

Elf-on-the-Shelf

Dear Kim,

This year we started doing *Elf-on-the-Shelf* for Tristan and Fantine. One night, our infusion night, the girl elf infused the boy elf. So the next morning, we woke up to find them like this:

I thought you might find this cute ;)

Tristan loved it. He walked up to them slowly and said, “Wow, he has hemophilia too... like me”.

Have a Merry Christmas!

Delphine Dubois Martin

Haemophilia Foundation of Nigeria

(Left to right) William Patsakos, Megan Buckie Adediran, Kim Phelan and Dr. David Clark

While at the National Hemophilia Foundation Conference in Anaheim CA we were graciously invited by Val Bias to meet a lovely woman named Megan Buckie Adediran, the Executive Director from the Haemophilia Foundation of Nigeria. Nigeria is part of the USA Twinning Program sponsored by Pfizer Inc.

Megan is a wonderful vivacious woman who is passionate and very determined to make a difference in the quality of life for people with hemophilia in her country. We encourage you to befriend her on Facebook to see the good work she is doing.

Nigeria has a population of 160 million people with approximately 16,000 patients with hemophilia. There are 328 hemophilia patients registered with the organization. Just 200,00 iu of factor products are donated each year for the care of all these patients.

They have formed six regional chapters and three are functioning with one volunteer Executive Director

The goals are to raise $129,000 USD, increase availability of factor products to one million iu in one year and establish awareness in the general population through four radio spots and 600 educational pamphlets.

To support the US Twinning with Nigeria and provide greatly needed resources please contact The National Hemophilia Foundation.

We think this is such a worthy cause and will do our part as well to see that Megan has support as well. If anyone would like to reach out to Megan. Her email is megan@haemo.org.ng.

We wish her a lot of luck and she should be very proud of her amazing accomplishments thus far with the help of Val Bias and the Twinning Program.

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

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Factor Nine Holiday Fund 2013!

The Coalition for Hemophilia B understands that 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, New York 10022

Please respond by *December 1, 2013* so that the Factor Nine Santa can load his sleigh with holiday gifts for all good boys and girls! 100% of your donation will be used to put a smile on a child's face.

We wish everyone a wonderful holiday season filled with love, happiness and good health!

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 22, 2013.***
(Your name and information will be kept *strictly* confidential.)

Send this form to: The Coalition for Hemophilia B Holiday Cheer
Attention: Special Elf Kim
825 Third Avenue, Suite 226
New York, New York 10022

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:	Wish List:	Wish List:
_____	_____	_____
_____	_____	_____
_____	_____	_____
_____	_____	_____
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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

We wish everyone a wonderful holiday and happy New Year!

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If you do not have access to a computer, please contact Kim Phelan at 212-520-8272 or hemob@ix.netcom.com to have the product insert mailed to you.

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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