



# Factor Nine News

## The Coalition for Hemophilia B

Fall 2017



### Topics in Hemophilia

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**MEETINGS ON THE ROAD**



# THE COALITION FOR HEMOPHILIA B





# MEETINGS ON THE ROAD 2017



This year we had the wonderful opportunity to host eight Meetings On The Road nationwide! Our first meeting was held in Omaha, Nebraska Saturday, September 15<sup>th</sup>, hosted by our Chairman, Dr. David Clark. Dr. Clark presented updates on hemophilia B, with welcoming remarks by the Coalition's President, Wayne Cook. We were delighted to have Tai Chi master Rick Starks hold a session with the families to introduce them to the many health benefits of Tai Chi. Chris Liddell presented a great session on the subject of infusion support and we had a interactive engaging talk by Felix Garcia about the connections between the history of hemophilia and that of the ancient Spartans. Children's activities were fun filled with plenty of video games, board games and activities for all ages. It was wonderful to be in Nebraska!

Next stop on the agenda and for the first time ever, we held back-to-back meetings on Saturday, September 23<sup>rd</sup> in Columbus, Ohio and Ann Arbor, Michigan! In order to do this, we created teams of members from within the hemophilia B community. Each volunteer was a very special asset and we are so grateful to have good teams in place. In Ohio families learned about the impact of being a strong advocate for your family with Gina Perez and reconnecting the mind and body through fitness with Cassandra Starks. Children enjoyed a day trip to the Magic Mountain Fun, Golf

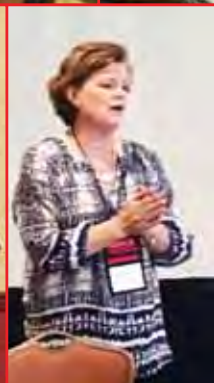
Cart, Arcade and Mini Golf Center. In Michigan, families were excited to learn about Pain Management with Nurse Practitioner Jim Munn and hearing Shonda Joshua speak on her son's struggles and triumphs over obstacles he faced due to his Hemophilia B. The Children went to Dave and Busters. We truly appreciated seeing our families in Columbus and Ann Arbor!

Our Houston, Texas meeting was held on October 7<sup>th</sup>. We were hesitant to hold the meeting after Hurricane Harvey, but decided to continue with our plan to bring education and lots of hugs to our hemophilia B families in Texas. Topics included the importance of continuing to be a strong advocate for your family - Donnie Akers' discussion empowered families to know their rights in the patient-doctor relationship and how to speak with their medical team. Speaker Joanne Garza put the spotlight of unaffected siblings in families where one child has hemophilia.

Children enjoyed a fun day trip to Dave and Buster's Arcade while the little ones were kept busy in the childcare room playing fun games and activities with onsite childcare. We also celebrated a special birthday cake for one of our caregivers!

We were very happy we made it to Texas and saw first hand the great spirit of Texans who truly know how to weather the storm!





On Saturday November 4<sup>th</sup>, we held our second set of back-to-back meetings in Louisville, Kentucky and Salt Lake City, Utah. In Kentucky, families came together to learn about overcoming challenges with Chris Liddell and the difficulties of raising multiple kids including one with a chronic disorder. Shelley Gerson led a workshop to help empower parents/caregivers with new tools to ensure each child's needs are met. The children went to Dave and Busters with childcare on premises. In Utah, families gathered to explore ideas with speaker Cassandra Starks that having a healthy mind can help you deal with obstacles caused by hemophilia and other daily stressors. They also learned about infusion support for caregivers of patients with Patty Eastin. Children were chaperoned to the Natural Museum of Utah, which we were told was truly amazing.

After many years, we were happy to return to Utah!

The final two meetings were held November 11<sup>th</sup> in Indianapolis, Indiana and Scottsdale, Arizona. In Indiana, the families gathered together to learn about gene therapy with specific emphasis on what exactly it is and how it works from keynote speaker, Dr. Amy Shapiro. She also including information on current trials in hemophilia B. Dr. Shapiro has a way of taking hard to understand scientific information

and making it so that people with hemophilia and their families can understand it better, which will help them make well informed decisions.

Families also enjoyed listening to Chris Liddell speak on infusion techniques and William Dewaine who presented on rebuilding the Body through Diet. Children had fun at Tilt Studio and Arcade, where childcare was provided on premises. In Arizona, Brenda Adamson held a workshop to help shed a spotlight on unaffected siblings and Megan King, a mother of six (two boys with hemophilia B), spoke on the importance of trusting a mother's instinct and not feeling "less than" when decisions have to be made in the best interest of the child as all children are unique and there is no "one size fits all." Megan had us laughing one minute and crying the next. That takes special talent! Megan is a one-woman powerhouse who will be on the road with us next year!

The children's daytrip to Dave & Busters with childcare on premises had the youngsters having a ball!

Every one of our eight Meetings On The Road had a family session with Rick or Cassandra Starks to teach the health benefits of the gentle art of Tai Chi. ended with a

Factor Nine Family Meeting also featured Dr. David Clark or Biology

major Tony Vetter introduced “What’s New in Hemophilia” educating everyone on current clotting factor products and what’s in the pipeline.

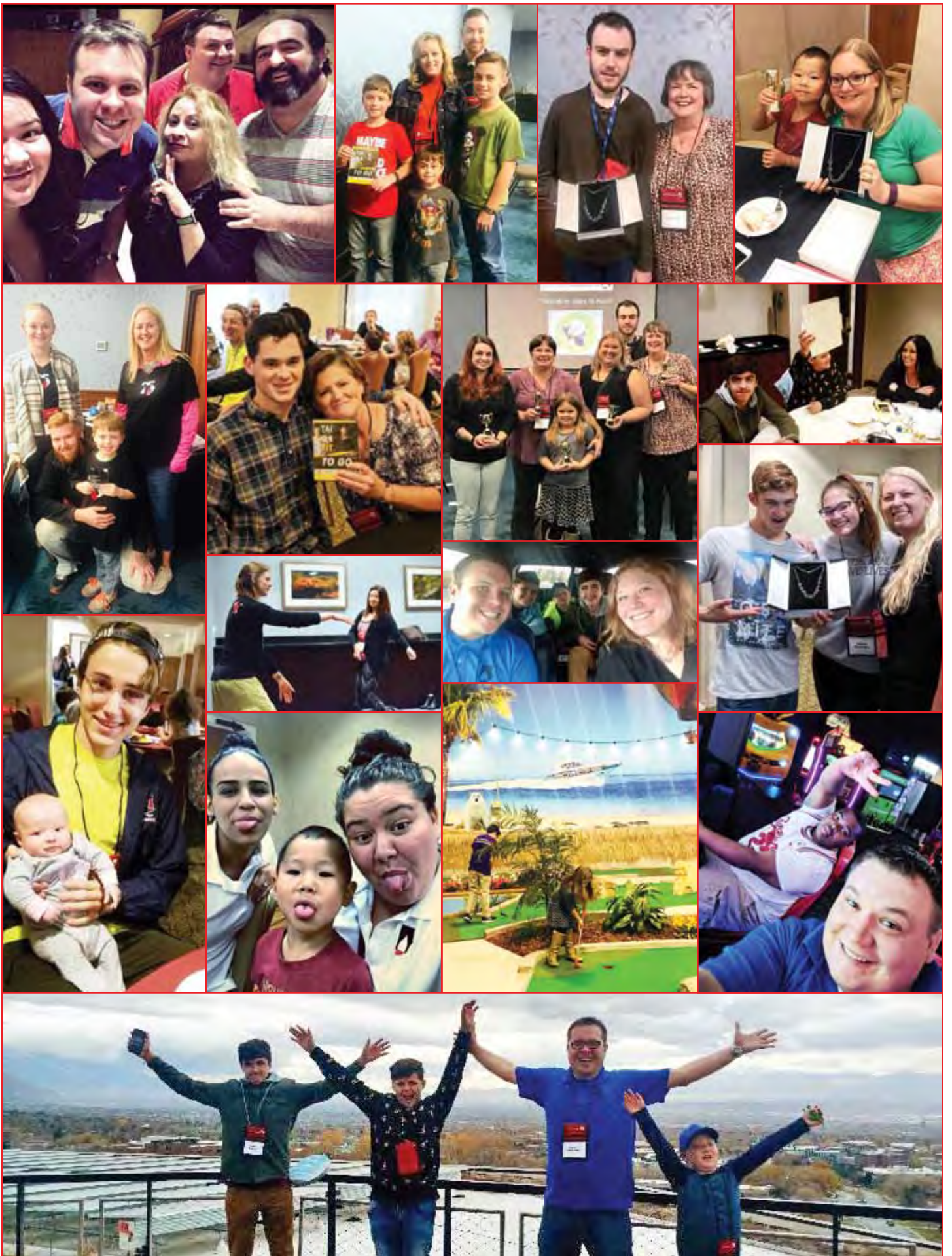
Within these smaller, more intimate meetings, families were able to partake in a game called Are You Smarter Than Your Hemophilia B?

created by the Coalition to help enhance knowledge of hemophilia B. Followed by roundtable group sessions of sharing and talks about more programs topics we can bring to our meeting in the future. The meeting ended with raffle prize drawings and a group photo. We love visiting all of our families and

we hope to come to a state near you in the future! We are truly thankful to Pfizer and CSL Behring for their generous sponsorship to help educate and reach as many families as possible across the USA! Together our goal is to educate, empower and support! Thank you!

**Thank you to Pfizer and CSL Behring for sponsoring our Meetings on the Road and thank you to our many exhibitors!**













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

### Important Safety Information

IDELVION is used to control and prevent bleeding episodes in people with hemophilia B. Your doctor might also give you IDELVION before surgical procedures. Used regularly as prophylaxis, IDELVION can reduce number of bleeding episodes.

IDELVION is administered by intravenous injection into the bloodstream, and can be self-administered or administered by a caregiver. Do not inject IDELVION without training and approval from your healthcare provider or hemophilia treatment center.

Tell your healthcare provider of any medical condition you might have, including allergies and pregnancy, as well as all medications you are taking. Do not use IDELVION if you know you are allergic to any of its ingredients, including hamster proteins. Tell your doctor if you previously had an allergic reaction to any FIX product.

Stop treatment and immediately contact your healthcare provider if you see signs of an allergic reaction, including a rash or hives, itching, tightness of chest or throat, difficulty breathing,

lightheadedness, dizziness, nausea, or a decrease in blood pressure.

Your body can make antibodies, called inhibitors, against Factor IX, which could stop IDELVION from working properly. You might need to be tested for inhibitors from time to time. IDELVION might also increase the risk of abnormal blood clots in your body, especially if you have risk factors. Call your healthcare provider if you have chest pain, difficulty breathing, or leg tenderness or swelling.

In clinical trials for IDELVION, headache was the only side effect occurring in more than 1% of patients (1.8%), but is not the only side effect possible. Tell your healthcare provider about any side effect that bothers you or does not go away, or if bleeding is not controlled with IDELVION.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call **1-800-FDA-1088**.

**IDELVION®**, Coagulation Factor IX (Recombinant), Albumin Fusion Protein  
Initial U.S. Approval: 2016

**BRIEF SUMMARY OF PRESCRIBING INFORMATION**

These highlights do not include all the information needed to use IDELVION safely and effectively. Please see full prescribing information for IDELVION, which has a section with information directed specifically to patients.

**What is IDELVION?**

IDELVION is an injectable medicine used to replace clotting Factor IX that is absent or insufficient in people with hemophilia B. Hemophilia B, also called congenital Factor IX deficiency or Christmas disease, is an inherited bleeding disorder that prevents blood from clotting normally.

IDELVION is used to control and prevent bleeding episodes. Your healthcare provider may give you IDELVION when you have surgery. IDELVION can reduce the number of bleeding episodes when used regularly (prophylaxis).

**Who should not use IDELVION?**

You should not use IDELVION if you have had life-threatening hypersensitivity reactions to IDELVION or are allergic to:

- hamster proteins
- any ingredients in IDELVION

Tell your healthcare provider if you have had an allergic reaction to any Factor IX product prior to using IDELVION.

**What should I tell my healthcare provider before using IDELVION?**

Discuss the following with your healthcare provider:

- Your general health, including any medical condition you have or have had, including pregnancy, and any medical problems you may be having
- Any medicines you are taking, both prescription and non-prescription, and including any vitamins, supplements, or herbal remedies
- Allergies you might have, including allergies to hamster proteins

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

- Known inhibitors to Factor IX that you've experienced or been told you have (because IDELVION might not work for you)

**What must I know about administering IDELVION?**

- IDELVION is administered intravenously, directly into the bloodstream.
- IDELVION can be self-administered or administered by a caregiver with training and approval from your healthcare provider or hemophilia treatment center. **(For directions on reconstituting and administering IDELVION, see the Instructions for Use in the FDA-Approved Patient Labeling section of the full prescribing information.)**
- Your healthcare provider will tell you how much IDELVION to use based on your weight, the severity of your hemophilia B, your age, and other factors. Call your healthcare provider right away if your bleeding does not stop after taking IDELVION.
- Blood tests may be needed after you start IDELVION to ensure that your blood level of Factor IX is high enough to properly clot your blood.

**What are the possible side effects of IDELVION?**

Allergic reactions can occur with IDELVION. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the chest or throat, difficulty breathing, light-headedness, dizziness, nausea, or decrease in blood pressure.

Your body can make antibodies, called inhibitors, against Factor IX, which could stop IDELVION from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

IDELVION might increase the risk of abnormal blood clots forming in your body, especially if you have risk factors for such clots. Call your healthcare provider if you experience chest pain, difficulty breathing, or leg tenderness or swelling while being treated with IDELVION.

A common side effect of IDELVION is headache. This is not the only side effect possible. Tell your healthcare provider about any side effect that bothers you or does not go away.

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

*Based on November 2016 PI revision.*



The Generation IX Leadership program kicked off on Thursday, September 28<sup>th</sup> as attendee arrivals started funneling out of busses and onto the campgrounds of the YMCA Camp Heyo-Went-Ha in Traverse City, Michigan. They greeted each other, new introductions were made and others hugged happily to see one another again. Everyone gathered in the main room to unwind from travel, talk, play card games and catch up. Generation IX Leadership programs allow members of the hemophilia B community to come from all over the United States to participate in workshops designed to strengthen their advocacy and leadership skills so they are empowered to advocate for themselves, family members and their community.

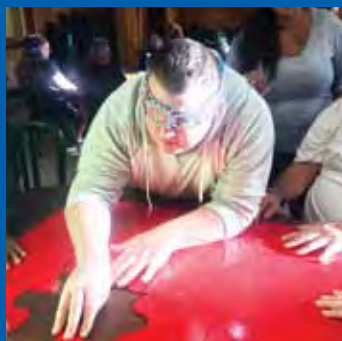
## GENERATION IX MICHIGAN LEADERSHIP PROGRAM

The weekend ensued when the Generation IX team leaders, Pat Torrey and Jacose Bell began with introductions and building an agreement amongst its participants to help understand to respect and adhere to boundaries created by the group. Afterwards the group participated in icebreakers to get to know each other and then weekend expeditions began! Group members went out in the nearby campgrounds to learn to magnify their surroundings by only focusing on a 4x4 square inch of nature. Equipped with string, magnifying glasses and

notepads with pens, the group separated into pairs and went micro-exploring in different locations over the campgrounds.

After lunch, the Generation IX crew had everyone pack up and go to an offsite high ropes course and zip line activity. The zip line activity allowed for easy accessibility by providing the group with a spiral staircase to climb up and let go of their fears and then zip line down! The evening program revolved around teaching participants to “Lean-In” and learn to put yourself in the others person’s shoes instead of being







guarded or defensive. This practice will allow you to take a step back and understand the other side of the situation, which helps to create better a better outcome.

On Saturday the group broke into teams of four and went to a nearby lake to canoe with the sole intention of partaking in a scavenger hunt to find bags of puzzle pieces, which would lead them to their next activity. Upon returning, the teams were separated, seated at four different tables and blindfolded. They then had to uncover their puzzle pieces, only to find out that the puzzle pieces were mixed in among all four tables to create four different puzzles.

Through advocacy and teamwork, leaders emerged from the group and worked with one another as a whole to help get the corresponding pieces to each table. After the pieces were at each table the puzzles had to be completed, as everyone still remained blindfolded! After completion, they took a break, but then gathered as a

whole to discuss their strengths and challenges while being blindfolded.

Closing the weekend events, the group completed a final activity where they paired up in twos and tried to come to an agreement on various topics on which they disagree. Through listening and talking, many pairs learned it was difficult to sit and listening without passing judgment. Others found it powerful to learn to “Lean-In” to see the perceptions of others. Saturday night and Sunday morning, the group said their goodbyes and departed to the airport to return home after an eventful and empowering weekend.

The Generation IX Leadership Program is extremely beneficial for advocates and emerging advocates to learn to help strengthen their skills in leadership. On behalf of GutMonkey and the Coalition for Hemophilia B, we would like to thank Aptevo for their generous sponsorship of this wonderful program.



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## Indications for RIXUBIS [Coagulation Factor IX (Recombinant)]

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

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

## Detailed Important Risk Information

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.

Allergic reactions have been reported with 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 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.

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 following page for RIXUBIS Important Facts.**

**You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088.**



# **RIXUBIS**

## **[COAGULATION FACTOR IX (RECOMBINANT)]**

### **Important facts about**

### **RIXUBIS [Coagulation Factor IX (Recombinant)]**

This leaflet summarizes important information about RIXUBIS. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about RIXUBIS. If you have any questions after reading this, ask your healthcare provider.

#### **What is RIXUBIS?**

RIXUBIS is a medicine used to replace clotting factor (Factor IX) that is missing in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Hemophilia B is an inherited bleeding disorder that prevents blood from clotting normally. RIXUBIS is used to prevent and control bleeding in people with hemophilia B. Your healthcare provider may give you RIXUBIS when you have surgery. RIXUBIS can reduce the number of bleeding episodes when used regularly (prophylaxis).

#### **Who should not use RIXUBIS?**

You should not use RIXUBIS if you

- are allergic to hamsters
- are allergic to any ingredients in RIXUBIS.

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

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

#### **How should I infuse RIXUBIS?**

RIXUBIS is given directly into the bloodstream. RIXUBIS should be administered as ordered by your healthcare provider. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia B learn to infuse their RIXUBIS by themselves or with the help of a family member.

Your healthcare provider will tell you how much RIXUBIS to use based on your weight, the severity of your hemophilia B, and where you are bleeding. You may have to have blood tests done after getting RIXUBIS to be sure that your blood level of factor IX is high enough to clot your blood. Call your healthcare provider right away if your bleeding does not stop after taking RIXUBIS.

#### **What are the possible side effects of RIXUBIS?**

Allergic reactions may occur with 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. Some common side effects of RIXUBIS were unusual taste in the mouth and limb pain. Tell your healthcare provider about any side effects that bother you or do not go away. These are not all the side effects possible with RIXUBIS. You can ask your healthcare provider for information that is written for healthcare professionals.

#### **What else should I know about RIXUBIS?**

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.

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

**You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088.**

**The risk information provided here is not comprehensive. To learn more, talk about RIXUBIS with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at [http://www.shirecontent.com/PI/PDFs/RIXUBIS\\_USA\\_ENG.pdf](http://www.shirecontent.com/PI/PDFs/RIXUBIS_USA_ENG.pdf) or by calling 1-800-FDA-1088.**

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The Fall 2017 Men's Retreat held in October at the Scottsdale Marriott at McDowell Mountains Resort in Arizona was a huge success! Attendees traveled from all over the country to gather together for a weekend of education, inspiration, support and comradery.



# MEN'S RETREAT

BY WAYNE COOK

The retreat began Thursday evening with an outdoor dinner reception to welcome everybody. Dinner was followed by a rap session, which helped break the ice and gave everyone a chance to meet one another.

Friday morning started with an energizing Tai Chi class led by Rick Starks, a Tai Chi master who has been teaching at our meetings, retreats and symposiums for over four years. Our first speaker of the day was Donny Akers, an attorney from Louisiana who has been involved in helping the hemophilia community for over 20 years. Donny talked about elder law and insurance issues.

After lunch, two of our committee members, Felix Garcia and Matt Scalfani addressed the attendees. Felix discussed the trials and tribulations of living with hemophilia and Matt talked about the experience





of being a dad raising a child with hemophilia. Dr. Kim Maurer, the Medical Director of the Comprehensive Pain Center in Oregon spoke about the opioid epidemic and comprehensive pain management in the hemophilia community.

After a short break we all assembled on the east lawn of the resort to take our group photo before we started our "Bleeder Olympics." Bleeder Olympics is

an event where teams compete against each other in backyard BBQ games like Hillbilly Golf, Can Jam, Corn Hole and Bocce Ball. This provided plenty of opportunities to laugh, have fun and bond with one another. We ended our day with a nice dinner held in the courtyard of the hotel and enjoyed the rest of the evening chatting and getting to know one another.

Saturday morning started with Tai







Chi held on the west lawn followed by breakfast. Tony Vetter, a young man who has been part of the men's retreat for the last four years, led the first session of the day. Tony introduced an app he created called "B Connected." B Connected provides access to information on a wide variety of topics, for example, legislation and aging with hemophilia, along with a chat room where people can connect. After Tony's presentation, Chairman of the

Coalition Dr. David Clark, a research scientist, talked about what's new in the treatment of hemophilia, inhibitors and gene therapy. Dr. Clark's talk was very informative and insightful on new products and clinical trial updates. His presentation was followed by an aquatic therapy session at the pool led by Dr. Jeff Kalberg, a physical therapist from the Phoenix area who also has hemophilia. The aquatic exercises help strengthen joints with very low impact



**Thank you to Pfizer for generously sponsoring our Men's Retreat!**



and it is a program that everyone enjoyed.

On our final night, we boarded a bus and stopped at a local car show with 700 cars on display including old school hot rods, custom built cars, classics and new age cars. Next we headed to Octane Race Experience for go-cart racing on a 1/3-mile track reaching speeds up to 45 mph. Some opted for the virtual reality experience where they "hunted down space aliens." The guys driving the go-carts experienced the adrenaline rush of competing against one another

for bragging rights and trophies for first, second and third place finishers. The night ended with a get together back at the hotel for a last chance to enjoy everyone's company and say our goodbyes to those who had early morning flights on Sunday.

We will continue to hold the Men's Retreat and make sure to combine great speakers with a meaningful, bonding experience that we hope will last a lifetime. We are going to start planning for our April 2018 meeting, so please get your lotto applications in as soon as possible!



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“

Coming to IXINITY, I feel much more  
like I'm in control of my own life now.

—Heidi has hemophilia B and uses IXINITY

➤ See why Heidi switched to IXINITY at [JustBIXperiences.com](http://JustBIXperiences.com)

This information is based on Heidi's experience. Different patients may have different results. Talk to your doctor about whether IXINITY<sup>®</sup> may be right for you.

## INDICATIONS AND IMPORTANT SAFETY INFORMATION

### What is IXINITY<sup>®</sup>?

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

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

### IMPORTANT SAFETY INFORMATION for IXINITY<sup>®</sup>

- You should not use IXINITY if you are allergic to hamsters or any ingredients in IXINITY.
- You should tell your healthcare provider if you have or have had medical problems, take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal remedies, have any allergies, including allergies to hamsters, are nursing, are pregnant or planning to become pregnant, or have been told that you have inhibitors to factor IX.
- You can experience an allergic reaction to IXINITY. Contact your healthcare provider or get emergency treatment right away if you develop a rash or hives, itching, tightness of the throat, chest pain, or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.

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

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

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





## IXINITY® [coagulation factor IX (recombinant)]

### Brief Summary for the Patient

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit [www.IXINITY.com](http://www.IXINITY.com).

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

### What is IXINITY?

IXINITY is a medicine used to replace clotting factor (factor IX) that is missing in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Hemophilia B is an inherited bleeding disorder that prevents clotting. Your healthcare provider may give you IXINITY when you have surgery.

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

### Who should not use IXINITY?

You should not use IXINITY if you:

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

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

### What should I tell my healthcare provider before using IXINITY?

You should tell your healthcare provider if you:

- Have or have had any medical problems
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal remedies
- Have any allergies, including allergies to hamsters
- Are breastfeeding. It is not known if IXINITY passes into your milk and if it can harm your baby
- Are pregnant or planning to become pregnant. It is not known if IXINITY may harm your baby
- Have been told that you have inhibitors to factor IX (because IXINITY may not work for you)

### How should I infuse IXINITY?

IXINITY is given directly into the bloodstream. IXINITY should be administered as ordered by your healthcare provider. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia B learn to infuse their IXINITY by themselves or with the help of a family member.

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

Your healthcare provider will tell you how much IXINITY to use based on your weight, the severity of your hemophilia B, and where you are bleeding. You may have to have blood tests done after getting IXINITY to be sure that your blood level of factor IX is high enough to stop the bleeding. Call your healthcare provider right away if your bleeding does not stop after taking IXINITY.

### What are the possible side effects of IXINITY?

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

- Rash
- Hives
- Itching
- Tightness of the throat
- Chest pain or tightness
- Difficulty breathing

- Lightheadedness
- Dizziness
- Nausea
- Fainting

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

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

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

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

### How should I store IXINITY?

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

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

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

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

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

### What else should I know about IXINITY?

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

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

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



Manufactured by:  
Aptevo BioTherapeutics LLC  
Berwyn PA, 19312  
U.S. License No. 2054

Part No: 1000973\_1  
CM-FIX-0078

## Cost of Gene Therapy Proportional to Number of Patients

Although gene therapy promises a cure for hemophilia and other genetic diseases, its cost has been a subject of concern. uniQure's Glybera, which was licensed in Europe in 2012 as the first gene therapy product in the world, was priced at approximately \$1,000,000 (depending on patient weight). Glybera was indicated for treatment of lipoprotein lipase deficiency, an ultra-rare disease in which the body does not process fat correctly. One patient was treated successfully before the product was taken off the market for lack of demand. According to uniQure, Glybera was developed as a proof-of-concept that gene therapy could work, and they knew it would not be profitable.

Since then, four other gene therapy treatments have been approved or are near enough to approval that the manufacturers have announced prices (none so far for hemophilia). Trinity Partners, a life sciences consulting firm, studied the markets for these treatments and found that there is a correlation between the cost of the product and the number of potential patients. The current prices range from about \$373,000 for Yescarta, a cancer therapy with an estimated patient population of 7500, to the million dollars for Glybera with an estimated patient population of less than ten. There are about 4000 hemophilia B patients in the U.S.

Another interesting development in this area is that the Centers for Medicare and Medicaid Services (CMS) has agreed to pay Novartis for their new gene therapy cancer treatment Kymriah (approx. \$500,000; approx. 300 patients) only if the treatment works. Each patient will be evaluated one month after treatment to determine whether it was successful. If not, CMS doesn't pay, and Novartis absorbs the cost. This kind of risk-sharing outcome-based arrangement may become more widespread with the increase in the number of high-priced treatments.

## Gene Therapy for Inhibitor Patients

One of the big questions about gene therapy is whether it will work for inhibitor patients. So far, the gene therapy clinical studies have excluded patients with inhibitors, or those who are prone to develop inhibitors. They don't want their results complicated by other conditions. The concern is what would happen to an inhibitor patient if he received gene therapy and his liver started pumping out uninterrupted amounts of factor IX? Would the immune system go crazy trying to neutralize all of that factor IX, and would that lead to a serious medical condition?

Inhibitors are antibodies that the immune system makes against infused factor because it thinks the factor is a foreign protein that shouldn't be in the bloodstream. Only about 3 - 5% of hemophilia B patients develop inhibitors, but those that do have serious problems. Inhibitors can sometimes be eradicated by a method called Immune Tolerance Induction (ITI). ITI uses frequent (often daily) infusions of factor to try to get the immune system to tolerate it. ITI works in about 70% of hemophilia A patients, but only in about 30% of hemophilia B patients. In addition, hemophilia B patients can develop life-threatening allergic reactions (anaphylaxis) to factor IX as well as nephrotic syndrome, a kidney disorder.

A review article published by researchers at Children's Hospital of Philadelphia analyzes results from a number of previous animal studies that suggest that factor VIII or factor IX gene therapy may actually act as a form of ITI to tolerize patients against producing inhibitors and help to eradicate pre-existing inhibitors. Studies with hemophilia B dogs have shown that gene therapy may act as a method of performing continuous ITI. Gene therapy was able to eradicate the pre-existing inhibitor in one hemophilia B dog and prevent inhibitor development in other inhibitor-prone dogs. This is a promising result, but it needs to be confirmed in humans. It also doesn't address the issues of anaphylaxis and nephrotic syndrome.

## Guidelines for Emergency Department Treatment

Hemophilia patients routinely have difficulty in dealing with hospital emergency rooms. The biggest issues are a lack of knowledge of hemophilia by emergency department staff and the lack of availability of factor products. The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) recently published MASAC Document #252 *Guidelines for Emergency Department Management of Individuals with Hemophilia and Other Bleeding Disorders*.

According to Donald J. (Donnie) Akers, Jr., JD, the legal counsel of the Hemophilia Federation of America (HFA), speaking at the Coalition Men's Retreat in October, this document now represents the "Standard of Care" for hemophilia patients in emergency rooms. The term Standard of Care has specific legal meaning – hospitals, physicians and staff can be in legal jeopardy if they do not follow the Standard of Care. Donnie recommends that all patients carry a copy of MASAC #252 with them, present it to the emergency room staff and tell them that this represents the Standard of Care for their condition.

The MASAC guidelines include the following important points, among others:

- Individuals with bleeding disorders should be triaged urgently – delays can significantly affect morbidity and mortality.
- Treatment decisions should be made on the suspicion of bleeding. Administration of factor should be started immediately, not after confirmation of a diagnosis.
- Consultation with the patient's hematologist or a Hemophilia Treatment Center is strongly advised.
- If a patient with hemophilia or a parent brings clotting factor to the hospital they should be allowed to use it. (Note that this may conflict with hospital and/or state policy. This is one argument in which use of the term Standard of Care may help. Also, point out that treatment should be started immediately; most hospitals don't have ready access to factor.)

MASAC #252 can be accessed on the NHF web site, [www.hemophilia.org](http://www.hemophilia.org).

### **HUGS Shows Economic Burden of Prophylaxis is Surpassed by Long-Term Benefits**

The Hemophilia Utilization Group Studies (HUGS) looked at the annual bleed rate (ABR) and associated costs among the hemophilia B population. They found that the average cost to treat mild-to-moderate hemophilia B was \$85,852 and for severe hemophilia B, \$198,733. Clotting factor accounted for 85% of the costs. The cost of clotting factor for prophylactic treatment was found to be 2.5 times more than for on-demand treatment. However, prophylaxis was associated fewer bleeding episodes and lower hospitalization costs. They mentioned that they found a surprisingly high ABR for patients with hemophilia B in the U.S. This could be because of the slowness in adopting prophylaxis, compared with other developed countries. Overall, they concluded that prophylactic treatment is associated with lower hospitalization costs, more full-time employment and a lower ABR.

### **University of Hawaii Gene Therapy**

Researchers at the University of Hawaii recently published a novel method for hemophilia B gene therapy. Instead of delivering the new factor IX gene using a virus vector, they use microbubbles. The factor IX genetic material is placed in tiny bubbles with a lipid (fat) coatings that are then infused intravenously. The microbubbles spread around the bloodstream, including to the liver. An ultrasonic beam focused on the liver breaks down the microbubbles and causes them to fuse with liver cells, depositing their factor IX genes. So far, the researchers have gotten good results in hemophilic mice. The advantage of this approach is that by not using a virus to deliver the genes, they should not have problems with the immune system targeting the vector and preventing the therapy from working.

### **Novo's Subcutaneous Concizumab Shows Good Thrombin Generation Results**

Novo Nordisk is developing Concizumab, an inhibitor of Tissue Factor Pathway Inhibitor (TFPI) for treatment of hemophilia A and B patients with inhibitors. TFPI is an anti-coagulant, an inhibitor of clotting. The clotting system contains a large number of clotting factors and anti-coagulants that keep each other in balance, so the blood clots when it should and doesn't clot when it shouldn't. Having too little factor VIII or factor IX upsets the balance making it harder to form a clot. Several organizations are working on products to inhibit anti-coagulants to try to restore the balance and make it easier to form clots.

Concizumab is a monoclonal antibody (a synthetic antibody made in the lab) that attacks TFPI. Novo recently published results showing that Concizumab administered subcutaneously to four healthy males significantly increased thrombin generation in their blood. Thrombin (activated factor II) is the enzyme that converts fibrinogen to fibrin, which sticks to itself to form a clot. The researchers are using thrombin generation as a marker for clotability of the blood. Novo is currently recruiting A and B inhibitor patients for a Phase II (dose determination) study of Concizumab.

### **uniQure Acquires Patents for FIX-Padua for Gene Therapy**

uniQure, one of the leaders in gene therapy for hemophilia B, recently acquired the family of patents on the more-highly-active factor IX molecule known as FIX-Padua and its use in gene therapy. FIX-Padua, which is about eight to nine times more active than normal factor IX, was first identified by Professor Paolo Simioni of the University of Padua in Italy, who patented it. Prof. Simioni will also become an advisor to uniQure. According to uniQure, they have only obtained these patents to secure their own use of FIX-Padua and have not developed a position on the other companies that are developing products based on it.

Because of its higher activity, less FIX-Padua is needed to produce the same clotting activity. uniQure will use the FIX-Padua gene in their new gene therapy treatment for hemophilia B, called AMT-061. With their previous gene therapy treatment, AMT-060, they had treated ten patients who had reached an average factor IX level of about 7%. uniQure believes that replacing the normal FIX gene in AMT-060 with the FIX-Padua gene will help them reach much higher factor IX levels. They have already obtained good results in non-human primates and believe that they can achieve FIX levels of 30 - 50% in humans. They have also obtained FDA and EU approval to move forward with AMT-061 in their ongoing Phase I/II clinical studies and to have it included under their Breakthrough Therapy designation from FDA. uniQure will perform a six-week study with AMT-061 in late 2017 to confirm their results and dosing, and then start a pivotal trial in 2018.

# SONGS OF LOVE INSPIRING THE SOUL



John Belzer, Founder of Songs of Love Foundation with his dog Banjo in his music studio.

**BY MICHAEL PERLMAN**

**A harmonious chorus echoes from Forest Hills, and resonates in the hearts of children and their families countrywide. One man's heart pumps in the direction of our youth, to instill their hearts with hopeful visions of tomorrow. John Beltzer, a Forest Hills, NY resident, founded the Songs of Love Foundation in 1996, which has played an influential role in healing and boosting the emotions of children and teens facing chronic and terminal diseases and emotional challenges.**

As of August 2017, nearly 28,000 children to young adults between the ages of 1 to 21 have received a personalized "Song of Love," an original composition orchestrated by national songwriters and singers, which rejoices a child's life. Most often, brochures are mailed to hospitals nationally, and a child's parents complete a profile reflective of their child's favorite genre, hobbies, pets, friends, and family members.

"We have an active roster of 50 songwriters, and over the years we worked with close to 500 songwriters, everywhere from New York, New Jersey, and Florida to Minnesota, California, and Nashville," said Beltzer, who welcomes visitors to his Forest Hills office at 73-26 Yellowstone Boulevard that employs 3 full-time staff members.

Beltzer said, "We have composed songs for children facing various medical challenges including those with Hemophilia B. We would love to partner with the Coalition For Hemophilia B, and have the organization send referrals, and have parents and friends request 'Songs of Love.'"

A native of Sao Paulo, Brazil, Beltzer settled in Brooklyn at age 8 and moved to Forest Hills at 15. After convincing his father to buy him a drum set, he was self-taught on the drums, piano, and guitar. His fraternal twin brother Julio, who also played guitar, joined Beltzer by composing songs and forming the top 40 band, "Cameon." Julio composed a song titled "Songs of Love" in 1984, two months before passing away at age 24. Then Beltzer founded another band named "Cinema," which appeared on Star Search in 1987. "In January 1996, after a record deal did not materialize, I experienced an epiphany to create a national non-profit, 'Songs of Love,' which I named in tribute to my brother." As a humanitarian, composer, and performer, Beltzer is grateful for

many life lessons. He said, "I know how to sing and compose songs, and it's not just about trying to achieve commercial success. Anyone is given a particular talent for a much higher purpose, so no matter what you are good at, you can use that particular skill to create a better world."

Memorable experiences continue to surface while recording a Song of Love. He explained, "I have composed an estimated 3,000 songs, and every song is unique, so I always find myself pleasantly surprised when I create a melody that has never floated around in the universe in quite that way. It also gives me great pleasure to work with other singers, and especially those that have not recorded in a studio. It's great to observe the satisfaction on their faces, when they know that the song will bring a smile to the face of a sick child."

"We have a great symphony of love out there," said Beltzer. In summer 2016, Songs of Love rocked the Forest Hills Stadium stage. "We were Paul Simon's interactive opening act in a live recording of a pop/rock song for a child named Teddy Moore. We turned the stadium into a large outdoor recording studio, where two condenser mics faced the audience, and we added their voices over the pre-recorded chorus." Beltzer continued, "In the end, I asked Teddy if he had any words, and he said 'I love you Forest Hills.'"

Teddy's Song of Love among

various live recordings is available on YouTube, and such is the case for 2-year-old Maisie, who was diagnosed with cancer. In August 2017, hundreds of Forest Hills residents helped record Maisie's Song of Love at the annual Jazz Thursdays, with her mother Eliza by her side. Beltzer said, "We took that experience out of the stadium and into the streets, and what a unique and inspiring feeling, and a great bonding experience with the community." On an even happier note, he added, "Maisie went for her CAT scan the following day, and we found out that she's cancer-free, so you couldn't ask for better timing."

Beltzer welcomes more community partnerships in places such as MacDonal Park, Forest Hills Jewish Center, the Forest Park Bandshell, and Times Square, and a return to Forest Hills Stadium. "We would like to involve the 112th Precinct and our elected officials, and I would love to record a song on Arthur Ashe Kids' Day at next year's US Open," he said.

In May 2017, NY1 News recognized Beltzer in the popular "Queens People of the Week" segment. Songs of Love has been featured on EXTRA, CBS News, 60 Minutes, Today Show, Dateline, NBC Nightly News NY, ABC World News, BBC, CBS Early Show, The NY Times, People magazine, USA Today, and "Hallmark Heroes with Regis Philbin."

Celebrities including Billy Joel, Nancy Sinatra, Michael Bolton, David Lee Roth, Jamie-Lynn Sigler, and even Elmo have partnered. Songs of Love engaged thousands of Black Eyed Peas fans at their concert to mark the 10,000th recording. Sesame Street's Bob McGrath and 15,000 Mets fans recorded the 12,000th song at Shea Stadium. Jason Mraz recorded the 20,000th song, "Love Is All Around" for Christian Burns, who has since passed away. Beltzer stated, "The indestructible beauty and spirit of a child that passes away stays very much alive." To mark the foundation's



John Beltzer with Maisie and her mom, Eliza.

20<sup>th</sup> anniversary in 2016, a concert was held at Webster Hall celebrating the music of The Allman Brothers.

Beltzer has received thousands of letters of gratitude from parents. "There are so many amazing stories, telling us how their child's song has helped. The environment around the child is pierced with soothing melodies, and it takes away the trauma, fear, and pain. On car trips to the hospital, everyone sings and it transforms the moment in a powerful way." Beltzer recalled delivering a song to a hospitalized child with a brain injury. "As I walked out, she struggled to pick up her arm and wave goodbye. Her song triggered that response."

This past May, Songs of Love was presented with a \$1 million, 4-year challenge grant from the Leon and Toby Cooperman Family Foundation. Beltzer explained, "We have proudly named them our 'Songs of Love orchestra leaders.' Every dollar we receive is immediately doubled up to \$250,000 per year. This grant will enable us to reach out to an additional 4,000 children in the next 4 years, so we hope that parents and friends will contact us to record a Song of Love. We are always seeking singers and songwriters to give back with their talents, and we

also encourage companies to have their employees record with us."

To reach a broader audience, the foundation is creating a Songs of Love iPhone app, due for mid-September. "You can request a Song of Love, learn how to become a songwriter, watch clips, view upcoming events, and make a regular donation or donate your car."

Upcoming fundraisers include their 6th annual LI Swing & Sing Golf Outing at Glen Head Country Club in Long Island on September 19, and the 19th annual NJ Swing & Sing Golf Outing on September 25 at the Preakness Hills Country Club in Wayne, NJ. "Come out and do some swinging and singing," said Beltzer.

To participate, visit [www.songsloved.org](http://www.songsloved.org), "Like" [www.facebook.com/SongsOfLove](https://www.facebook.com/SongsOfLove), or call 1-800-960-SONG.



# IN MEMORY

## HAROLD R. ROBERTS, M.D.

1930 – 2017



The bleeding disorders community lost one of its true giants with the death of Harold R. Roberts, M.D. on September 9, 2017.

In a career spanning over 50 years, Dr. Roberts was a highly respected physician, researcher and mentor in hematology at the University of North Carolina (UNC). He was a true patient champion.

A native of North Carolina, Dr. Roberts earned both his undergraduate and medical degrees at UNC and joined the faculty in 1961. In 1967, he became Chief of UNC's Division of Hematology and founded the UNC Center for

Thrombosis and Hemostasis in 1978 where he served as director for 20 years.

He worked with other UNC colleagues including Dr. Kenneth Brinkhous to develop the first purified factor VIII product in the U.S. He was a charter member and first Executive Director of the International Society on Thrombosis and Haemostasis (ISTH) and a member of the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) where he was chair from 1988 to 1994.

God speed, Dr. Roberts.



# IN MEMORY

## PAUL BERKEMANN

March 31, 1942 ~ Sept. 19, 2017

Paul Berkemann, 75, of Norwalk, died peacefully Tuesday, September 19, 2017 at Regency Care Center in Norwalk.

Paul was born March 31, 1942 to Fred and Alvoretta (Hunt) Berkemann. He grew up on a farm in Perry, IA and graduated from Perry High School in 1960. He then attended Oklahoma State Tech in Okmulgee, OK where he earned his degree in drafting and architecture. This is where he met and married D'Anne Williams. They moved to Norwalk, IA in 1962 after accepting a position with Pittsburg Des Moines Steel, which later was named CBI. He enjoyed his work drafting water towers and was a very loyal and hard worker. He held this position for 47 ½ years before retiring.

Paul enjoyed spending time with family, going out to eat, reading, watching NASCAR, classic and antique cars and driving around in his Buick. In 2010 he moved into Regency Assisted Living and enjoyed participating in activities, especially trivia and bingo and was President

of the Resident Tenant Council. Paul was also involved with the Iowa Chapter of National Hemophilia for many years.

Left to cherish his memory are his daughters, Paula Cordes of Des Moines, IA and Susan Rainbolt, of Okmulgee, OK; Grandchildren include Mavluda Mavlonazarova, Rachel Rainbolt and Beth Rainbolt; Great grandchildren, Steven, Star and Kenny Roy; Brother David Berkemann, his wife Betty and their two daughters Kenna and Kelly. He will also be missed by extended family, many friends and special caregiver Brandi.

He was preceded in death by his parents, an infant sister, his former wife, and son-in-law Rick Cordes. The family would like to thank all the kind and loving caregivers at Regency Assisted Living and Care Center, UnityPoint Hospice and Ethel with Visiting Nurses.

Rest in peace, Mr. Berkemann.



# BUILDING A FAMILY ONE SHOE AT A TIME

By Michael Perlman

Days turned into weeks and into months, but it was worth every moment. Meet Marykay Thrower, a longtime hemophilia nurse and native of St. Louis, Missouri, who helped coordinate a shoe drive to give a young boy with hemophilia named Maxen from Guangzhou, China a permanent home in America with a loving family.

Now another shoe drive is underway to offer a second boy with hemophilia, a 10-year-old named Mason, from Maoming, China, a new chapter in life with that same family. Both boys are all too familiar with life in an orphanage, sheltered from true love. Thrower's generosity of spirit caused a chain reaction of family members, friends, and strangers to contribute to the initiative. "I am overwhelmed by the ripple effect," she said.

Thrower said, "We are all caught up in our lives, but this was a chance to become part of a wonderful gift for a boy halfway across the world. It's a story of boy who found the love of a family, parents, siblings, grandparents, and an extended family, as well as a community.

As a nurse coordinator, Thrower worked at a pediatric hemophilia treatment center for 15 years, prior to becoming a senior clinical consultant for over 13 years. She served as nurse educator in a major company that manufactures and sells hemophilia Factor, and retired in August.

Around 9 years ago, as a result of her position, she crossed





paths with Jason and Danelle Humphreys, residents of Elkhart, Illinois. Their son Jaxon is now 10 and daughter Ella is now 7. Then in 2015, their family grew when they adopted Maxen, who is now 9. To finalize the adoption, they spent Christmas in China, and Maxen became their gift.

Thrower said, "Over the years, I admired their commitment to their two biological children, and a deep friendship developed. Maxen was once a little boy with no family and no medical care, and now he runs and laughs and belongs to this amazing couple."

The Humphreys family teamed up

with Angel Bins, a non-profit shoe drive fundraising company based in California. "When they decided to adopt a child with hemophilia, I watched their progress through multiple fundraisers, and supported them to an extent, due to regulations imposed on my involvement by my company and pharmaceutical standards," said Thrower.

When her husband, Herbert Lee Thrower, Jr. passed away in August 2015, she felt lost. She explained, "I saw this as a way to cope with my grief and channel my energy and promote the initiative in Missouri, Arkansas, and Oklahoma." She was already well acquainted with various neighborhoods, since this is where she would frequently travel for work. "It became easy to ask and transport used shoes," she continued.

On December 5, 2015, the Humphreys' home became a destination for numerous friends, family members, and neighbors to gather. Thrower had social media and emails at her fingertips, and asked people she already knew if they wanted to join forces. "I made it a challenge and a competition," she said.

Teamwork and perseverance were key ingredients towards a successful shoe drive, which resulted in a collection of two tons of shoes, better known as over 19,000 pairs. This generated \$8,500 towards their adoption fees. She said, "From a double garage, we formed a line and passed down the bagged shoes to the people loading the truck, and we were barely able to close the door."

Today, Maxen cherishes simple pleasures such as going fishing with his father, coloring with his siblings and cousins, playing Minecraft with Jaxon, and playing basketball. Thrower keeps in touch with Maxen and his new family once a week over the phone, texting, and Facebook. While pursuing her work for the Southern Illinois Bleeding Disorder Community, she would visit them at least once a month. The family was reciprocal, as they paid a visit to St. Louis to spend quality time together, in addition to collecting shoes.

As a token of their appreciation, Thrower received a gift from the family. "In 2016, when I first met Maxen, I was presented with a shadow box containing small shoes from China, as a remembrance of my shoe drive work," she fondly recalled.





One shoe drive was a success story, and the motivation continued for the Humphreys and Thrower for another partnership. As of late June 2017, a second shoe drive was underway, and nearly a week later, another large bag was filled with shoes. She said, "There is still room in this family's hearts for one more son. Mason is in an orphanage in China and needs a forever family, so we're doing it again." The shoe drive is in collaboration with an organization called Funds2orgs. Minimally worn pairs of shoes are in need, excluding slippers and metal cleats.



Thrower explained, "We need at least another 7,500 pairs by the end of January 2018. We have been collecting shoes from churches, schools, businesses, and individuals who have asked friends and created drop-off locations." A community of at least 35 of Thrower's friends is opening up the channels of communication in various parts of the country, including Arkansas, Nebraska, Kansas City and Wichita, Kansas, Waterloo and Greenville, Illinois, and Sunset Hills, Lake of the Ozarks, Columbia, Eureka, Springfield, and South County Kirkwood, Missouri. The number of

friends participating is bound to grow.

Her son David has networked with his friends at softball and volleyball, in addition to work, and another son named Philip was enthusiastic about lending a helping hand with pickups and rubber-banding pairs of shoes. Her daughter Liz has also sparked the interest of a wide range of friends.

Despite their commitments, her children ultimately made time for other children who are less fortunate. As a case in point, she explained, "David lives in a condo in Lemay, Missouri and works full-time, and has an amazing heart.

He is always available for his family and friends; he also realized how it could help us get through the grieving after his father passed away.

Thrower has a compassionate heart, but she does not quite see herself as a humanitarian and remains modest. "I have always believed that we only live once, so whatever acts of kindness we can fulfill is my motto," she said.

Anyone interested in participating in the shoe drive to assist Mason in finding a loving home, can contact Marykay Thrower at [mkthr0wer@aol.com](mailto:mkthr0wer@aol.com).



It's the beginning of 2009 and a mom in the process of adopting her daughter from China is having a conversation with her adoption agent in New York City. In the course of their conversation, the agent notices that this mom has identified knowledge about hemophilia in her paperwork. At the time Melissa Penn had 3 children, her oldest son being affected by hemophilia. Pam Thomas, her adoption agent, immediately recalled an adorable little boy she met during her previous trip to Nanjing, China. Her liaison at the orphanage explained to Pam that this 8 year-old boy had hemophilia and was therefore, not eligible for adoption. When Pam told Melissa about this precious little boy in China, a 10-minute conversation began a small action that multiplied like the ripples in a pond when a pebble is tossed into it.

# THE HEMOPHILIA ADOPTION RIPPLE EFFECT

BY SHARI LUCKEY

Several months later, in Ann Arbor, Michigan my husband Dave and I received an email that would change our lives forever. The email merely indicated that an 8-year-old boy lived in China with hemophilia, spending many weeks in the hospital each year, and he ultimately needed a family. During Pam and Melissa's short conversation earlier in the year, Melissa explained to Pam that hemophilia is a very manageable condition in the U.S., with most children receiving medication 2-4 times per week to prevent internal bleeding into joints and muscles. Melissa urged Pam to persuade the orphanage to make the little boy "paper ready" for adoption. Melissa then reached out to Laurie Kelley, an advocate and author in the hemophilia community, who sent the email we received to many in the hemophilia community.

Since I have known hemophilia my whole life, it wasn't the scary part for us. My older brother by 3 years, Jimmy, had severe hemophilia B, he unfortunately passed away from AIDS related complications in 1993. With safe treatments and no more risk of HIV or AIDS, my husband and I moved forward with our family. Our oldest son, Jay, has severe hemophilia B with inhibitors and anaphylactic reaction to factor IX; I was diagnosed with mild hemophilia and a platelet dysfunction after post-child-birth complications; our twin daughters have platelet dysfunctions and one has mild hemophilia. We know and understand the challenges of living with hemophilia. For us, adopting was the unknown territory. However, within days of receiving that email we felt led to contact Pam and we were sprinting down the "adoption paper chase" trail.

The adoption process varies from country to country. For our adoption, we needed pre-approval from China, a home study from a local adoption agency, online training for older and special needs children, various legal papers and forms completed, and everything sent to China for authorization and approval. Our process took about 9 months from start to finish. The cost associated with a China adoption is currently around \$35,000 including travel. These fees are due at different intervals



throughout the process. Many people are frightened by the high cost of international adoption. However, numerous individuals have been able to acquire grants and fundraise enough to cover a large percentage, if not the full amount of their adoption costs.

When we were united with Luke in January 2010, he was quite frail and had a slight limp. He had a target right knee that had repeatedly bled over the years and was rather swollen. He spent the majority of his time with us in China in a borrowed wheelchair. Within a week of coming home we took him to the Hemophilia Treatment Center (HTC) to see the hematologist for a full evaluation. We had arranged this appointment ahead of time and requested a mandarin speaking translator, which was very helpful. Lab work was completed that day, as he had a toe bleed and severe hemophilia A was the confirmed diagnosis. He received a dose of factor VIII and upon waking the next morning with his toe feeling better, he gave the thumbs up for his new medicine. He soon began physical therapy for his knee and prophylaxis twice a week. Over the course of the next several months, he made amazing progress, growing physically stronger and regaining full use of his knee. The first summer Luke was home, he was able to attend Camp Bold Eagle where he learned to self-infuse and is now independent with his infusions. With occasional breakthrough bleeds that first summer,



the hematologist increased his prophylaxis to three times per week. Remarkably, he has not had a bleed in six years.

The ripple effect for Luke and our family has been substantial. He is physically strong and healthy and he has family and friends who love him. He is an excellent student, beginning his sophomore year in high school this year; he plays violin in his school orchestra and participates in team sports. Last year, he rowed on the crew team with his sisters and he enjoys running and riding his bike. This past year, I began working part-time in conjunction with the Hemophilia Foundation of Michigan (HFM) as the Advocacy and Outreach Coordinator. HFM has partnered with me to help further the reach to find forever families for children with hemophilia and to create educational materials and resources for hemophilia adoption.

The initial ripples of that short conversation between Pam, current China Director for Hand In Hand International Adoptions and Melissa continue to stretch much farther

than just our immediate family, growing bigger and flowing into waves, bringing the tides across the water to China and other countries. Since helping our family become complete, Pam was inspired to begin a crusade and search for boys with hemophilia in China. She locates the boys, educates the orphanage staff and China adoption officials that there are families in the U.S. open to adopting children with hemophilia, and she advocates for families on behalf of them. In the past two years, she has matched more than a dozen boys with hemophilia to their forever families. When Pam has a boy with hemophilia who is ready, she contacts me, Melissa, and several other moms in the hemophilia and adoption communities. We then go to work advocating for their forever families to find them. Several other adoption agencies are now beginning to advocate for boys with hemophilia. I have recently talked with agencies that have partnerships in Eastern Europe, Columbia, and the Dominican Republic that are ready and willing to advocate for children with hemophilia in need of families.

One mom, Kelly, who adopted her son a few years ago, started the Hemophilia Adoption Facebook page. This private group consists of those that have adopted a child with a bleeding disorder, are starting the process, or considering adopting a child with hemophilia. We are creating a network of advocates, mentors, and experts in hemophilia adoption. This community is growing as more children with hemophilia are adopted into forever homes. The sense of comfort realized when families understand there is a support system specifically for hemophilia adoption and they are not alone on this wonderful journey is remarkable.

In April of this year, I had a 5 minute conversation about waiting boys with hemophilia with a gentleman at a hemophilia event I was attending in Michigan. He, in turn spoke to his son and daughter-in-law who live in another state and relayed the information. As a result, his son and daughter-in-law are now in the

process of adopting a sweet little boy with hemophilia. It continues to amaze me how far reaching the ripples of a conversation or an action can spread.

I recently did a count of all the families I am aware of with adopted children with hemophilia or in the process of their adoption and came up with an astounding 23 families. I certainly cannot say all of these children were adopted because of a single conversation that happened seven years ago, but I truly believe many of these children would not have their forever families if that pebble had not been tossed into the pond by Melissa and Pam.

Countries, particularly China, are now becoming educated that children with hemophilia are indeed deserving of a chance to find their forever families. As a result, more and more children with hemophilia are being adopted. Seven years ago when Luke was living in an orphanage, it never occurred to his caretakers to attempt to find a family for him. We are so thankful Pam and Melissa had a conversation that would send ripples through our lives and continue to flow through the hemophilia community and beyond. I am so blessed to be a part of this ever growing community, and to witness families finding their forever sons.

There are still many children with hemophilia waiting for their forever families. For more information about hemophilia adoption please contact Shari Luckey at [sluckey@hfmich.org](mailto:sluckey@hfmich.org).





## RAISING THE MEADOW BOYS

LIVING LIFE TO THE FULLEST DESPITE LIVING WITH CHRONIC DISORDERS

"I Got You."- God

It is just another day. I get up, get the kids around for school, head to an exercise class, come home and start cleaning, picking up, doing laundry, the same old everyday stuff. We all know what that is like. Except today. My morning was the usual same old routine, but then I get a call from the school nurse. Evan's stomach hurts and his blood sugar is in the 300's. Not what you want to hear when you got things to do. I pick him up and we go home. He is asleep now. Resting because his body is exhausted from the restlessness from last night. His insulin pod expired in the middle of the night and it beeps constantly until you tell it to deactivate. It woke Evan up, of course, he deactivated it and just by chance I got up to check his number anyway. So we changed his pod at 2 am. Kinda hard to go back to sleep after that rude awakening. But anyway, he is finally getting the rest he needs.

I start to clean when I see my bible study books sitting on the couch. I usually try to get bible study time in of an early morning before anyone is awake, but I felt like I needed something extra today. I am studying Priscilla Shirer's Gideon. I come to a part where she asks what fears or insecurities do I have...HAHA, too many to list but my biggest is that I fear failing my kids. It is so hard to try

to parent 3 different kids. I know we all have that fear, but how do I parent one kid with a bleeding disorder, one kid who is normal and one who has a bleeding disorder and type 1? How do I do it without comparing or calling out who does what better or how do I not favor one because he has so many problems and not give the others slack? How do I be firm with the child that has so many health problems without making him feel like he is failing completely? How do I make time for the normal kid so he doesn't feel like he gets shoved to the side? How do I parent them fairly? How do we keep going? Trying to live with all this "stuff" that keeps piling on? .....midnight blood sugar checks, insulin before you eat, lows, highs, infusions, you get it. That is my worst fear, that they will some day grow up and feel like I didn't do a good job or they turn out to be horrible people because I did a lousy job of being their mom.

After sitting there in the quiet just thinking on that, all of the sudden I hear a small voice, one that I have been trying to hear for the past 382 days. A voice that says "I Got You". I have been trying my hardest to hear His voice for over a year and praise God I finally heard it! I finally received the message I have been trying so hard to hear.

Believe me, this past year I have prayed, screamed, cried, pleaded, yelled, begged, cursed, flat out threw temper tantrums, and laid in bed depressed, questioning if God really does exist. A peace come over me in that moment knowing that HE does exist and no matter what, He has my back. I can do this, I can raise and parent these kids and not fear what the future holds. I still don't know what the right way is to parent these yahoos but I know I can keep going, keep swimming, keep climbing, and keep pushing, knowing that I have finally heard Him whisper to me "I Got You."

Now, after crying and praising Him for the response I was so desperately seeking I want you to know that He has your back too. We all have issues we each deal with and I am sure most of us come to a point in our lives where we really question and doubt God. We are only human, and believe me I have had A LOT of human moments lately, but no matter what your challenges, fears, or insecurities are...He's got you! So you can also keep going, keep swimming, keep climbing, and keep pushing.

"Life is tough my darling, but so are you!"  
Blessings,

# WFH TWINNING PROGRAM

## MAKING A DIFFERENCE SINCE 1994



The World Federation of Hemophilia (WFH) Twinning Program has a long history of making a positive impact within the global bleeding disorders community. Since its ambitious beginning more than 20 years ago, the Twinning Program has consistently helped to develop city - and country - level support for people with hemophilia and other inherited bleeding disorders.

The Twinning Program improves hemophilia care in emerging countries through a formal, two-way partnership between two hemophilia organizations. The program's philosophy is based on collaboration—designed to facilitate the transfer of expertise, experience, skills, resources, and information between established and emerging hemophilia organizations.

Since the Twinning Program's launch, there have been 215 twinning partnerships established in 113 countries. One of the program's recent successes is the Tanzania–Ontario Twinning partnership, which was selected as WFH's Twins of the Year in 2016. Before becoming part of the Twinning Program, patients of the Haemophilia Society of Tanzania (HST) relied on the use of fresh frozen plasma for treatment regardless of their bleeding disorder. This situation meant that the Tanzania–Ontario Twinning partnership had an opportunity to make a significant difference by facilitating discussions leading to the provision of factor concentrates within Tanzania.

One of the first steps taken by the Tanzania–

Ontario Twinning partnership was facilitating HST board training, which focused on building HST's capacity for support, education, and advocacy. This was done through a review of the HST's organizational structure – reviewing the roles of the office bearers, discussing the importance of succession planning, and analyzing ways to build their membership. The education and support needs of patients and their families were also part of this discussion. Once the relevant issues had been identified, an action plan was developed for 2017.

Twinning can make a huge difference when advocating for improving patient care and treatment, and the experience of the HST was no exception. “The WFH Twinning Program has been able to make a real difference in the bleeding disorders community,” said, Alain Baumann, WFH's Chief Executive Officer. “Pfizer's support has played a big part in that. We're grateful for their collaboration and support, and we are especially grateful for the knowledge and experience they have been able to share with us in order to make the continuous improvement of the program possible.”

For more information on the WFH Twinning Program for Hemophilia Treatment Centers, hemophilia organizations, and the Youth Twinning Program please visit <https://www.wfh.org/en/twins>.

The Twinning Program is supported by exclusive funding from Pfizer. PP-HEM-USA-0922-01





# WFH TWINNING PROGRAM

Pairing emerging and established countries to help improve care for those living with bleeding disorders worldwide.

In 1994, the World Federation of Hemophilia (WFH) recognized the need for an established support program to:



Unite developed and developing hemophilia centers and patient organizations.



Share information and best practices.



Provide resources and tactics to help improve care and provide treatment for those living with or managing a bleeding disorder.

The WFH Twinning Program is a collaborative partnership between countries centered on two types of stakeholder groups:



**Hemophilia Treatment Center (HTC)** twinning pairs emerging HTCs with established ones to help improve diagnosis and provide treatment and care for people with hemophilia.



**Hemophilia Organization Twinning (HOT)** pairs emerging and established hemophilia patient groups to share knowledge in areas such as patient education, outreach, fundraising, and all other aspects of operating a successful hemophilia patient society.

Since 1994, the WFH has worked with HTC and HOT twins to **establish a global movement** to ensure *every single person with a bleeding disorder receives quality healthcare no matter where they are.*



"Our twinning (with Quebec) has taught us that **nothing comes easy, and that everything is possible.** Everyone is committed to maintain what has been acquired, and at the same time, to **keep going forward and make things better.**"

—ISLEM NAFTI, TUNISIA, TUNISIA-QUEBEC TWINNING, 2005-2010

## TWINNING IS A TWO-WAY STREET

The WFH supports twins through:

- Matching partners
- Annual grants
- Educational materials
- Provides meeting space for twins at WFH global conference
- Ongoing coaching

Twins commit four years to their partnership. In return, they gain:

- Exposure to different cultures and customs
- Broadened personal and professional relationships
- New challenges and teamwork skills

In order to maximize the program's potential, twins are expected to practice patience and understanding with each other, to set realistic expectations regarding results and to devote the necessary time to foster the partnership.

## WHAT DO TWINS ACCOMPLISH?

The WFH Twinning Program has four core goals when it comes to improving hemophilia care. Twinning initiatives must be:

- Sustainable
- Realistic
- Specific
- Demonstrate mutual benefits for twins



# WFH TWINNING PROGRAM

Possible projects to help twins foster growth and spread knowledge include:

### HTC Twinning Activities

- Training programs for healthcare professionals
- Patient registry development and outreach activities
- Development of treatment protocols
- Exchange of information, such as publications or materials for health professionals and/or patients

### HOT Twinning Activities

- Advocacy initiatives
- Patient and family education
- Strategic planning
- Lobbying governments
- Constitutional work

"Even though there's a small population of people with hemophilia in the world, these **people need to have dignified and adequate treatment in order to contribute to society.** And the **World Federation of Hemophilia feels a better level of care is possible** in all the countries we serve. The Twinning Program has helped us achieve our goal of sustainable care and treatment for all."

-LUISA DURANTE, WFH REGIONAL PROGRAM MANAGER

### TWINNING IN ACTION: PROGRAM SUCCESSES



#### AREQUIPA (PERU) – LOS ANGELES (U.S.A.)

- Twinned from 2011-2014
- Developed a multi-disciplinary team fully trained in the proper management of patients with hemophilia and other bleeding disorders
- Education and training of primary care and emergency room physicians in three additional cities
- Initiated a home treatment program and prophylaxis program for patients
- Initiated new treatment protocols
- Provision of physiotherapy equipment and educational materials on hemophilia and von Willebrand disease



#### RUSSIA – UNITED KINGDOM

- Twinned from 1996-2003
- Increased number and strength of regional chapters
- Training for strategic planning, lobbying, volunteer development, fundraising, and database management
- Provision of educational materials and support for home treatment program
- Russian Hemophilia Society continued the legacy of the Twinning Program and served as the established twin for partnerships with Kazakhstan (2008-2011) and Uzbekistan (2012-2015)



### WFH TWINNING PROGRAM AND EXCLUSIVE PFIZER SUPPORT

#### Pfizer is proud to celebrate 15 years with the WFH...

...and many more years beyond that demonstrating ongoing commitment to improving the lives of those living with bleeding disorders through grants, donation of treatment products, and many other initiatives.

Pfizer recognizes that proper hemophilia care spans beyond a hematologist and factor replacement, and works in partnership with the WFH to support culturally relevant and sustainable well-rounded programs that help address aspects of a hemophilia patient's healthcare needs.

To learn more about the WFH Twinning Program visit <http://www.wfh.org/en/twins>.



THE WFH TWINNING PROGRAM IS SUPPORTED BY EXCLUSIVE FUNDING FROM PFIZER

15 YEARS



PP-HEM-USA-0513-01

YOU'RE INVITED!  
THURSDAY, MARCH 1, 2018  
TERRACE ON THE PARK, NEW YORK



THE COALITION FOR HEMOPHLIA B  
11TH ANNUAL  
ETERNAL SPIRIT AWARD DINNER



# UPCOMING EVENTS 2018!

**The Coalition For Hemophilia B  
2<sup>nd</sup> Annual Forelife Golf Outing and Fundraiser  
Thursday – March 22, 2018  
TPC Sawgrass; Ponte Vedra Beach, FL 32082**



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**The Coalition For Hemophilia B  
12<sup>th</sup> Annual Symposium  
Friday-Sunday – March 23-25, 2018**

**Sawgrass Marriott Golf Resort & Spa; Ponte Vedra Beach, FL 32082**





**YOU ASKED  
WE LISTENED**



**COMING JANUARY 2018**

**PEERS | FAMILIES | EXPERTS**

# KIDZ KORNER!

## Fall Harvest

K X H B T C R C G L R T F J S  
U O C G S E V A E L U P T N Y  
H I M Y Y A H A P W U J I A D  
O R T M B J L B P R K K W S S  
Q C R V U V P C Y P P D M F D  
I I O M C A W S F M L A V A Q  
Z I I R C L E H U M F E C L L  
N X E O N L X P U Y D D S L S  
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S B M T N B A I J Q I I M T R  
S C A R E C R O W W R C E X E  
C Z F Z D D F M Z R K F W N E  
R I T T J H Q Y E K R U T S S  
E A K E X K K L M T A X Y O R

LEAVES  
FALL  
ACORNS  
WIND  
TREES  
APPLES  
PUMPKINS  
MAPLESYRUP  
SCARECROW  
CORN  
RAKE  
SQUIRREL  
HAY  
TURKEY

(c) Web9treats





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**Facebook:** [www.facebook.com/HemophiliaB/](http://www.facebook.com/HemophiliaB/)

**Twitter:** <https://twitter.com/coalitionhemob>



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