Summer 2017

KHF Hemosphere

What Does Gene Therapy Mean to YOU?

Ladonna Pettus remembers the cover of Hemalog, a hemophilia magazine from 1990, promising "A Cure by the Year 2000?" It seemed at once like a vision and a done deal. Ladonna's son with hemophilia was around two at the time. She recalls, "I had such hope. He is almost 30 now."

Many parents who remember that magazine cover had those hopes. Their children are adults now, and although gene therapy trials are underway, it seems that the passion and dreams for a cure have been tempered. Alvin Luk, head of clinical research and operations at Spark Therapeutics, is working on hemophilia gene therapy. He offers, "We all underestimated the complexity of gene transfer."

Maybe this is why, when I repeatedly asked 2,600-plus hemophilia "friends" on Facebook about their thoughts on gene therapy, only a handful of people replied. I'm sharing their comments here. Normally, the hemophilia community is vocal and active. Does this lack of response indicate that we are mostly unaware when it comes to gene therapy? Are we not sure what it is?

Defining gene therapy: A cure?

Parents and patients sometimes use the terms "gene therapy" and "cure" interchangeably. But the definitions aren't the same. When we think of a cure, we think of eradicating the disorder or disease. In other words, a person with hemophilia no longer has it. In fact, a permanent cure for hemophilia already does exist. Steven Riedle notes that his brother with hemophilia had a liver transplant in October 2016, and is indeed cured of hemophilia.

But a liver transplant is not a feasible option.[1] Many patients and caregivers are waiting—hoping—for a safe, widely available therapy that will cure hemophilia permanently. Yet we may need to adjust our definition of cure. Community members who responded to my questions seem to realize that most current gene therapy trials promise to make hemophilia less severe by increasing circulating levels of factor in the blood.

Very few patients or parents understand gene therapy as thoroughly as Ray Stanhope, former National Hemophilia Foundation president, and person with hemophilia. He defines gene therapy as "the use of a viral vector to modify cells in the body to produce an additional specific protein which is either missing or produced at a lower than normal level in a person with hemophilia."[2] What Ray describes is not necessarily a cure, but an improved therapy.

What level of success?

If current gene therapy trials promise to increase circulating factor in the bloodstream, what level would be considered successful—a "cure"? Remember that severe hemophilia means less than 1% circulating factor, moderate means greater than 1% to 5%, and mild means 6% to 50%. Anything over 50% is considered in the normal range. [3]

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For Ray, levels of circulating factor would have to be well over 40% and closer to 50% (normal) to be considered a cure.

But others think that even converting someone from severe to mild hemophilia could be considered a success. Nichole Foley writes, "I think taking a person from severe to mild hemophilia is enough of an advantage for some of these kids that have constant challenges, and hopefully it will alleviate inhibitor issues."

Bryce Loehrke says, "If gene therapy could permanently bring me to the levels of even mild hemophilia, I would consider myself cured for the most part. Having severe hemophilia A, I've often said that those with mild hemophilia don't even have hemophilia. I don't mean to diminish the fact that they still have issues from it periodically, but often with much less severity or frequency, sometimes to the point of not knowing they have it until later in life."

Tina Ruis takes this even further. "My 24-year-old son with severe hemophilia B—his left leg is unbearable. His calf is massive, and he can barely move without a walker. Levels of 11% to 15% would be worthwhile; over 25% would make me cry with joy."

Stephen Brewer would be happy if gene therapy worked, even if it wasn't permanent: "I would accept having mild hemophilia even if only for a few years."

Chris Templin and his daughter both have hemophilia B. Chris notes that aiming for "mild" hemophilia is fraught with inconsistencies. "I think it's interesting how people think all those with mild hemophilia bleed less then severe hemophilia patients. I know some milds who bleed more than some severe patients."

The price of success

If gene therapy is successful and becomes available, how much would it cost? Some families think that because gene therapy trials are being held at university hospitals and hemophilia treatment centers, its cost may be lower than that of current commercial therapies. But this is not correct, because the trials are underwritten by pharmaceutical companies and the manufacturing process would ultimately need to be upscaled by a commercial pharmaceutical company.

The issue of cost for a new therapy is complex, and includes these questions:

- What is an acceptable therapeutic factor level: moderate, mild, normal?
- How long will the treatment last: three years? permanently?
- Will other factor products need to be used during the treatment period?

Ray estimates the cost of a one-time treatment of gene therapy at "close to $1 million, given the low number of patients, the cost of research and development, and assuming that the therapy is successful for four years." He adds, "For the manufacturers, as much as they can charge; for the insurers, the least amount they have to pay."

Nichole Foley doesn't care: "Cost-wise, I am sure it will be astronomical, but if [gene therapy] enables kids to live a normal life, I'd think it would be worth it!"

Bryce believes, "If there's an effective lifetime cure, $250,000 will be a lowball figure. We need to convince insurance providers of the long-term savings of a permanent or semipermanent cure."

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What if gene therapy is good for only a few years?

The term "cure" isn't applicable at all if gene therapy—even if it brings your factor levels to normal—lasts for only a few years. This is a real concern.

Ray explains: "Given that the current vectors are viral and the immune system develops a response to that vector so that once used, it cannot be used again, this is problematic if the period of time that the treatment persists is short. There may not be time to develop an alternate type of vector. However, given the speed at which medical advances are occurring and accelerating, having the treatment persist for more than ten years might be enough to get you to the next vector, whatever that might be."[3]

Amber Brandt, mother of a child with hemophilia, worries, "Regular factor is so expensive, I don't see gene therapy being cheap by any means. And I'm sure it would be a huge struggle to get insurance to cover it. But if it only lasts a few years, I don't even think it would be worth [trying] it at all."

Wait-and-see approach

Patients who don't see any solutions to these concerns may adopt a wait-and-see approach. Some are inherently mistrustful of playing with genes, or of the whole commercial industry of factor manufacturing. Some feel that current therapies are good enough for now.

Brandi Worthington admits, "I don't know anything about gene therapy." Amber adds, "It's fascinating, but I would never choose that option for my son. He can choose that if he wants when he is an adult."

"I won't be a first adopter for gene therapy by any means," declares Bryce, "primarily due to distrust of the entire pharmaceutical industry for various legitimate and historical reasons. We need to know the consequences as well as benefits [of gene therapy]."

Ray concludes, "Depending on the factor levels achieved and the duration of the treatment and the usable number of vectors…I might wait and see."

Stepping-stone to a cure

Ray understands well the nuances and importance of educating the hemophilia community about gene therapy. Parents and patients will one day need to make an informed decision about whether to participate in it. "We as a community first need to define the parameters of what we would consider a cure," says Ray. "I have always had a strict interpretation of this word. A cure would be a single treatment that provides normal hemostasis over the lifetime of the person living with hemophilia. Anything less than this should be considered a stepping-stone toward a cure."

Stephen still has hope and carries the definition of cure even further. "Looking forward, a cure would include increasing circulating factor levels, [and] eliminate hemophilia from future generations [of a family].[4] This is the ideal I hope for."

Laurie Kelley, May 2017

1. Factors VIII and IX are produced primarily in the liver, although the cells lining the blood vessels also produce and hold reserves of factor VIII for release into the bloodstream when needed. Replacing the liver indeed cures hemophilia, but this is not deemed a viable option for treatment because it is too risky. Only patients with hemophilia who face liver failure are considered for this operation.
2. A vector is a modified virus used by molecular biologists to deliver genetic material into cells.
3. Among the general population, normal factor levels are between 50% and 150%, with most people being close to 100%.
4. Changing the genetics of future generations is not gene therapy, but human germline engineering. This practice is currently banned. It's highly unpredictable, dangerous, and considered unethical.

Event News

Easter Lily and Spring Flowers Sale

This annual fundraiser brings the colors and fragrances of spring into many homes, businesses, and churches. We are very grateful to our faithful volunteers who help sell these plants to family, friends, co-workers, and fellow church members. We were able to sell a total of 1,552 plants. A substantial number were sold by a cadre of amazing ladies: Janet Goff and Sharon McMahan sold the astounding number of 421 plants, followed by Jenifer Schultz with 67 plants, Sadalia Sturgill with 59 plants, Nita Wayne with 50 plants, Diane Burnett with 34 plants, Tina Pelly with 24 plants and Deborah Hitt with 18 plants. Proceeds benefit KHF's programs and services for Kentucky's Bleeding Disorders Community.

Family Day at the Louisville Zoo

This popular family event attracted over two hundred members of Kentucky's bleeding disorders community for an informative and fun day at the Louisville Zoo. Carnival games with a plethora of prizes entertained the little ones, who delighted in their wins, while the adults had a chance to relax, visit, and chat over a picnic lunch. A nice array of door prizes awaited the adults who always enjoy the excitement of the drawing.

The Call to Action for the 2017 Kentucky Hemophilia Walk resulted in several early-bird sign-ups who plan to be ahead of the curve with their fund-raising. We also recognized the top ten Walk Teams and top fundraisers from last year: Team Tag's Turtles raised $5,186; Team Brody raised $2,553; Team Jack! Raised $1,645; Team Ike-A-Maniacs raised $1,582; Team LEVI raised $1,527; Team Mac's Pack raised $1,115; Team Jordan raised $1,100; Team Andy's Avengers raised $1,030; Team GG Babies raised $925, and Team Louisville Globe Clotters raised $903. The top ten fundraisers who contributed to the success of the 2016 Walk were Tag Poynter, Brody Vanderpool, Karen Lucky, Cory Meadows, Ursela Kamala, Dianne Hardman, Pat Tharp, Amy Daugherty, Laura Webb, and Venus Marcum.

We thank the exhibitors who supported this year's Family Day at the Louisville Zoo and provided valuable product and services information to our attendees. They were Accredo, Aptevo Therapeutics, Bayer HealthCare, Bioverativ, CSL Behring, CVS Caremark, First Choice Home Infusion, Grifols, Matrix Health, Novo Nordisk, Octapharma, Option Care, Paragon Healthcare, Pfizer, and Shire.

Event News

Men's Night Out

A group of adventurous young men met at Coals Pizza in Middletown for a Men's Night Out Activity. John Vieke from Indiana told his captivating story about becoming a successful police officer while living with severe hemophilia. After a brief Q & A period, the men reconvened at Breakout Louisville to test their breakout skills. Lo and behold, these brilliant minds managed to break out of their confinement successfully before their allotted time was up. Congratulations and many thanks to our sponsor, CSL Behring!

Mini-Washington Days

In the beginning of June, the National Hemophilia Foundation (NHF) organized a mini-Washington Days activity for strategically targeted Chapters before the anticipated vote on the healthcare bill. KHF was one of the states invited to participate because of its two sitting Republican Senators, Senator Mitch McConnell and Senator Rand Paul. In meetings with the two Senators' staff, KHF advocated for the needs and concerns of the bleeding disorders community in Kentucky in regard to proposed and looming healthcare changes. Advocacy is a powerful tool and can positively impact important legislation. We thank the National Hemophilia Foundation for spearheading this crucial effort.

28th Annual Play a Round for a Cure Golf Scramble

Friendly skies and sunny rays made for a wonderful day on the golf course at Oxmoor Country Club for KHF's 28th Play a Round for a Cure Golf Scramble. This annual event is one of our primary fundraisers. Better than ever weather conditions, a super golf course, great food, friendly volunteers, and many nice prizes created the perfect setting for a successful event and fundraiser.

By the slimmest of margins, team winners were Pfizer, 1st place; Bayer HealthCare, 2nd place, and Bioverativ, 3rd place. The Ball Drop was won by Larry Holbrook, the Putting Contest was won by Chaz Jacobs, and Golf Poker was won by Sam Browning.

Sponsors were: Platinum Level: CSL Behring; Gold Level: Bayer HealthCare, Novo Nordisk, Pfizer, Shire; Silver Level: HEMA Biologics; Bronze Level: Bioverativ; Team Plus Level: Amerimed, Cottrill's Specialty Pharmacy, CVS Caremark, Kosair Charities, Octapharma; Team and Player Level: BioRx, Matt Hawthorne, Glen Hitt, Sr. and Friends, Paragon Healthcare, Republic Bank & Trust Company; Tee Sign Level: First Choice Home Infusion and Grifols; Event Level: CSL Behring, Bayer HealthCare, First Choice Home Infusion, HEMA Biologics, Novo Nordisk, and Pfizer.

We thank all our sponsors, donors and volunteers who ensured the success of this event. The event raised $17,500 to help fund KHF's programs and services for Kentucky's bleeding disorders community.

More News

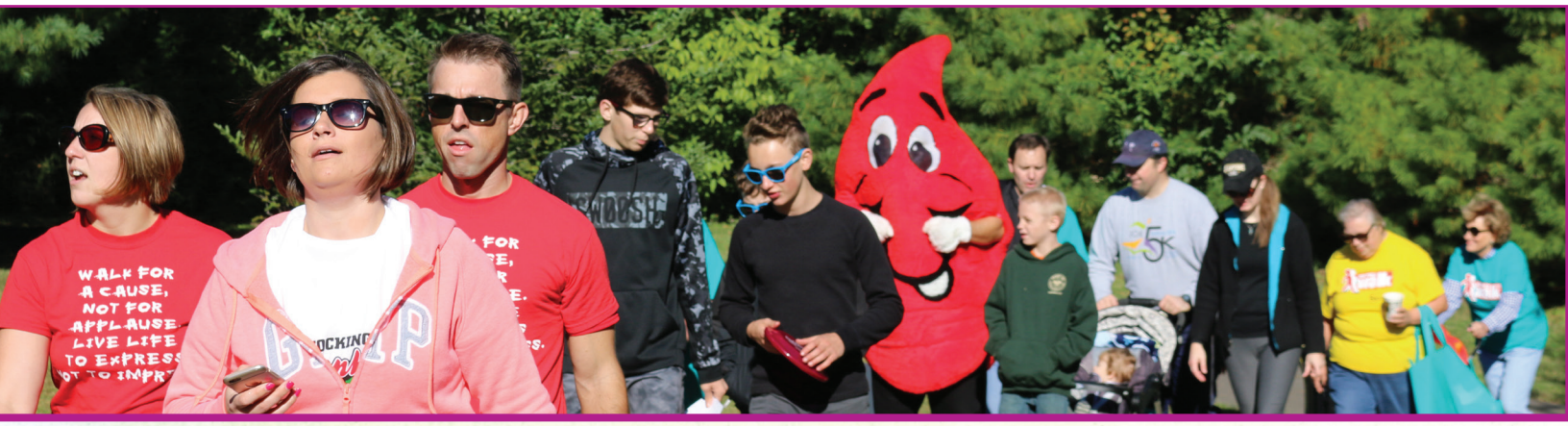
KHF *Walk* 2017

Wetherby Park in Middletown

There will be fun for everyone at this year's **KHF *Walk*2 Cure Hemophilia** on Saturday, October 14, 2017. This year, it's a Halloween themed event featuring Trick or Treat giveaways, children's activities, entertainment, door prizes, and more. Prizes will be awarded for best Halloween Costume (adults and children)! Prizes are also awarded for moneys raised by walkers and their teams! The more money you raise, the bigger the prize!

Pledges to support your participation in the Walk will give you the opportunity to help others! Proceeds help fund KHF's programs and services for Kentucky's bleeding disorders community!

For more information, or to register, please visit hemophiliawalk.donordrive.com/event/ky.



LOOKING AHEAD

4th Annual Kentucky Hemophilia Walk – October 14

Poinsettia Fundraiser – November/December

Year End Holiday Event – December 3

Advocacy Day – January/February

Vegasville Fundraiser – February 24

More News

KHF Membership
July 1, 2016 – June 30, 2017

Members, $20
James P. Huff
Melissa Noe
Ronald L. Swearingen

Supporting Members, $35
Susan Geralds
Judy Hayes
in memory of Jason Hayes
Mary E. Marasa
Donald L. Mattingly

Patron Members, $50
Sara Ceresa
Dr. David & Leslie Houvenagle
The Incorvia Family
Carol & John Nord
Stacey Powell & Family
Lonnie Surratt

Sustaining Members, $100
John & Leah Graham
Barbara W. Grayson
D. Spalding Grayson
Arthur Hackman
Fred & Darline Hartman
Thomas & Alice Hendrix
Vivian Marcum
Keith Peterson
Gail Yates
Cal & Nita Wayne Zehnder

Benefactor Members, $250
Deborah & Glen Hitt, Sr.
LTC John & Pat Tharp

Champion/Corporate Members, $500
Ted & Jennifer Forcht
Terry & Marion Forcht
National Cornerstone Healthcare Service, Inc. (NCHS)
Kristen & Keith Urbahn and Benjamin and William

In Memory

April 1, 2017 – July 31, 2017

Gone from our sight but never our memories; gone from our touch but never our hearts…

Mr. William L. Farmer, Sr.
Mrs. William L. Farmer, Sr.

Tom Graham
Gary L. Rice

Mildred Darline Hartman
Richard & Judy Hartman
Bradley & Melissa Haynie
Joan Majors
Vivian Marcum
Joe & Teri Ricklefs
Charlotte Shekell
Martha F. Simon

Do The Five

Follow these steps to prevent or reduce complications of bleeding disorders

1. Get an annual comprehensive checkup at a hemophilia treatment center.
2. Get vaccinated – Hepatitis A and B are preventable.
3. Treat bleeds early and adequately.
4. Exercise to protect your joints.
5. Get tested regularly for blood-borne infections.

To find out more about the National Prevention Program developed by the National Hemophilia Foundation in collaboration with the Centers for Disease Control and Prevention (CDC), click on www.hemophilia.org or call toll-free 800-42-HANDI.

KHF neither recommends nor endorses the products in this publication and does not make recommendations concerning treatment regimen for individuals. KHF suggests that you consult your physician or treatment center before pursuing any course of treatment. This publication is for general information only.

Save the Date

10/14/17

Walk2 cure hemophilia KHF

To sign up for the Walk, go to:
hemophiliawalk.donordrive.com/event/KY

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Pfizer Hemophilia

DEDICATION AND PERSONAL SUPPORT

The Patient Affairs Liaison role was created based on community feedback about the importance of helping to connect patients and caregivers with Pfizer Hemophilia tools and resources.

PATIENT AFFAIRS LIAISONS

Working for you—From the home of Motown to the Bluegrass State

Name: Chris Liddell

Home state: Michigan

Fun fact: If I'm watching TV, it's most likely sports-related. Go Tigers!

Ideal vacation spot: Anywhere quiet, unplugged from all electronics

What past experiences can you bring to this job? I've worked in hemophilia for over 10 years, so I've collaborated with and advocated for different members of this community.

To get in touch with Chris, call
Pfizer Hemophilia Connect 1.844.989.HEMO(4366)

What we do:

- Provide helpful information about Pfizer Hemophilia programs and services
- Serve as a resource to hemophilia treatment centers to help patients obtain access to Pfizer medicines
- Serve as a primary point-of-contact for local advocacy groups
- Participate in local and national events and programs
- Upon request, meet with patients and caregivers to answer questions related to Pfizer Hemophilia resources

"IT'S IMPORTANT TO CONNECT ON ALL LEVELS: HTCs, PATIENTS, FAMILIES, THE WHOLE COMMUNITY."

—Chris Liddell

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, or call 1-800-FDA-1088.

RIXUBIS
[COAGULATION FACTOR IX (RECOMBINANT)]
MOVING FORWARD

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, 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 or by calling 1-800-FDA-1088.

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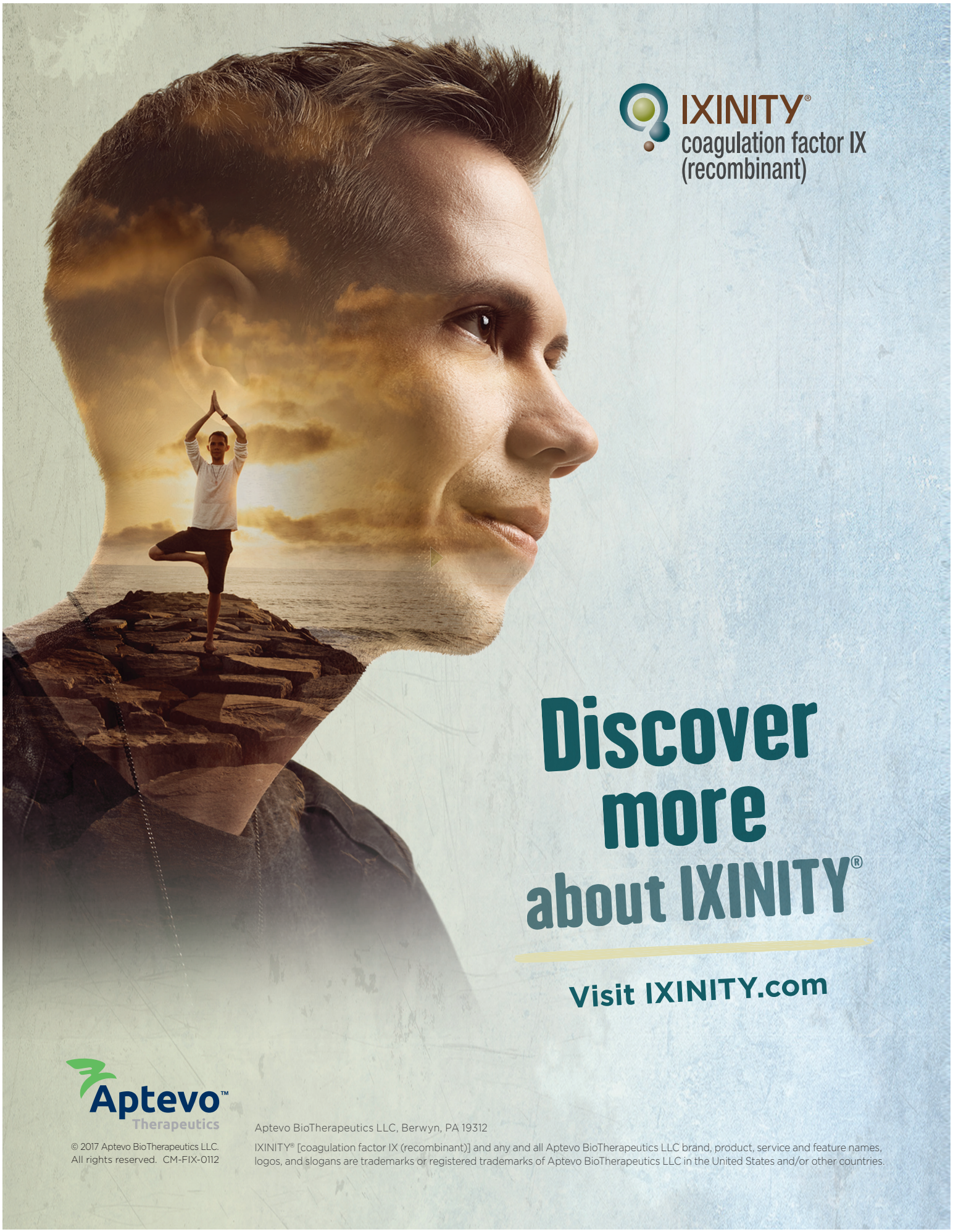

Aptevo BioTherapeutics LLC, Berwyn, PA 19312

BAYER

LIVING! with HEMOPHILIA

Looking for a new, fresh perspective on living with hemophilia?

Introducing your all NEW guide to **Living With Hemophilia**

Discover the new online destination for learning about hemophilia, living a healthy life and even leading in the hemophilia community. It's all at the new **LivingWithHemophilia.com**. Our site has been totally redesigned to give you more of the information you want and less of the stuff you don't want.

See What's New at

www.LivingWithHemophilia.com

Biotherapies for Life® **CSL Behring**

For adults and children with hemophilia A

REACH HIGHER

With the Long-lasting Protection of AFSTYLA

2x WEEKLY AVAILABLE

FDA-approved for dosing 2 or 3 times a week

ZERO BLEEDS (median AsBR*)

In clinical trials, whether dosed 2 or 3 times a week

COMPARABLE TO **NATURAL FACTOR VIII**

Identical to natural Factor VIII once activated

Zero inhibitors observed—Low incidence of side effects in clinical trials

In clinical trials, dizziness and allergic reactions were the most common side effects.

Visit AFSTYLA.com to sign up for the latest news

*Annualized spontaneous bleeding rate in clinical trials (interquartile range [IQR]=0–2.4 for patients ≥12 years; 0–2.2 for patients <12 years).

Important Safety Information

AFSTYLA is used to treat and control bleeding episodes in people with hemophilia A. Used regularly (prophylaxis), AFSTYLA can reduce the number of bleeding episodes and the risk of joint damage due to bleeding. Your doctor might also give you AFSTYLA before surgical procedures.

AFSTYLA is administered by intravenous injection into the bloodstream, and can be self-administered or administered by a caregiver. Your healthcare provider or hemophilia treatment center will instruct you on how to do an infusion. Carefully follow prescriber instructions regarding dose and infusion schedule, which are based on your weight and the severity of your condition.

Do not use AFSTYLA if you know you are allergic to any of its ingredients, or to hamster proteins. Tell your healthcare provider if you previously had an allergic reaction to any product containing Factor VIII (FVIII), or have been told you have inhibitors to FVIII, as AFSTYLA might not work for you. Inform your healthcare provider of all medical conditions and problems you have, as well as all medications you are taking.

Immediately stop treatment and 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 FVIII, which could stop AFSTYLA from working properly. You might need to be tested for inhibitors from time to time. Contact your healthcare provider if bleeding does not stop after taking AFSTYLA.

In clinical trials, dizziness and allergic reactions were the most common side effects. However, these are not the only side effects possible. Tell your healthcare provider about any side effect that bothers you or does not go away.

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 the following brief summary of full prescribing information on the adjacent page, and the full prescribing information, including patient product information, at AFSTYLA.com.

AFSTYLA® Antihemophilic Factor (Recombinant), Single Chain

AFSTYLA®, Antihemophilic Factor (Recombinant), Single Chain
For Intravenous Injection, Powder and Solvent for Injection
Initial U.S. Approval: 2016

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use AFSTYLA safely and effectively. See full prescribing information for AFSTYLA.

----------INDICATIONS AND USAGE----------
AFSTYLA®, Antihemophilic Factor (Recombinant), Single Chain, is a recombinant, antihemophilic factor indicated in adults and children with hemophilia A (congenital Factor VIII deficiency) for:

- On-demand treatment and control of bleeding episodes,
- Routine prophylaxis to reduce the frequency of bleeding episodes,
- Perioperative management of bleeding.

Limitation of Use
AFSTYLA is not indicated for the treatment of von Willebrand disease.

----------DOSAGE AND ADMINISTRATION----------
For intravenous use after reconstitution only.

- Each vial of AFSTYLA is labeled with the amount of recombinant Factor VIII in international units (IU or unit). One unit per kilogram body weight will raise the Factor VIII level by 2 IU/dL.
- Plasma Factor VIII levels can be monitored using either a chromogenic assay or a one-stage clotting assay – routinely used in US clinical laboratories. **If the one-stage clotting assay is used, multiply the result by a conversion factor of 2 to determine the patient's Factor VIII activity level.**

Calculating Required Dose:

Dose (IU) = Body Weight (kg) x Desired Factor VIII Rise (IU/dL or % of normal) x 0.5 (IU/kg per IU/dL)

Routine Prophylaxis:

- Adults and adolescents (≥12 years): The recommended starting regimen is 20 to 50 IU per kg of AFSTYLA administered 2 to 3 times weekly.
- Children (<12 years): The recommended starting regimen is 30 to 50 IU per kg of AFSTYLA administered 2 to 3 times weekly. More frequent or higher doses may be required in children <12 years of age to account for the higher clearance in this age group.
- The regimen may be adjusted based on patient response.

Perioperative Management:

- Ensure the appropriate Factor VIII activity level is achieved and maintained.

----------DOSAGE FORMS AND STRENGTHS----------
AFSTYLA is available as a white or slightly yellow lyophilized powder supplied in single-use vials containing nominally 250, 500, 1000, 2000, or 3000 International Units (IU).

----------CONTRAINDICATIONS----------
Do not use in patients who have had life-threatening hypersensitivity reactions, including anaphylaxis to AFSTYLA or its excipients, or hamster proteins.

----------WARNINGS AND PRECAUTIONS----------

- Hypersensitivity reactions, including anaphylaxis, are possible. Should symptoms occur, immediately discontinue AFSTYLA and administer appropriate treatment. (5.1)
- Development of Factor VIII neutralizing antibodies (inhibitors) can occur. If expected plasma Factor VIII activity levels are not attained, or if bleeding is not controlled with an appropriate dose, perform an assay that measures Factor VIII inhibitor concentration.
- If the one-stage clotting assay is used, multiply the result by a conversion factor of 2 to determine the patient's Factor VIII activity level.

----------ADVERSE REACTIONS----------
The most common adverse reactions reported in clinical trials (>0.5% of subjects) were dizziness and hypersensitivity.

To report SUSPECTED ADVERSE REACTIONS, contact the CSL Behring Pharmacovigilance Department at 1-866-915-6958 or FDA at 1-800-FDA-1088 or *www.fda.gov/medwatch***.**

----------USE IN SPECIFIC POPULATIONS----------

- Pediatric: Clearance (based on per kg body weight) is higher in pediatric patients 0 to <12 years of age. Higher and/or more frequent dosing may be needed.

Based on May 2016 version

Why We Walk

We walk to raise critical FUNDS for national research, local programs & services and to raise AWARENESS for the bleeding disorders community. Proceeds help fund KHF's programs and services for anyone in Kentucky's bleeding disorders community that may need a helping hand!

Your participation will help provide:

- $6,000 in emergency assistance and support
- $3,000 in educational scholarships and sponsorships
- A $40,000 Summer Camp Program for 35 children, teens, and young adults
- Medic Alert emblems and bicycle helmets
- Advocacy Day and participation in national advocacy efforts
- And so much more!

Kentucky Hemophilia Foundation

For more information, please visit
hemophiliawalk.donordrive.com/event/ky.

Walk2 cure hemophilia