CHAPTER Factors

SPRING 2016

ADVOCACY

2016 Washington Days

Midwest Hemophilia Association

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*The Free Trial Program is available to newly diagnosed patients and patients who are currently using other therapy. Participation in the Free Trial Program is limited to 1 time only. This program is complimentary and is not an obligation to purchase or use a Bayer product in the future. Reselling or billing any third party for the free product is prohibited by law.

†The Free Trial Program includes up to 6 free doses to a maximum of 5,000 IU for new patients and 40,000 IU for previously treated patients.

‡The program does not guarantee that patients will be successful in obtaining reimbursement. Support medication provided through Bayer's assistance programs is complimentary and is not contingent on future product purchases. Reselling or billing any third party for free product provided by Bayer's patient assistance programs is prohibited by law. Bayer reserves the right to determine eligibility, monitor participation, determine equitable distribution of product, and modify or discontinue the program at any time.

§People with private, commercial health insurance may receive co-pay or co-insurance assistance based on eligibility requirements. The program is on a first-come, first-served basis. Financial support is available for up to 12 months. Eligible patients can re-enroll for additional 12-month courses. The program is not for patients receiving prescription reimbursement under any federal-, state-, or government-funded insurance programs, or where prohibited by law. All people who meet these criteria are encouraged to apply. Bayer reserves the right to discontinue the program at any time.

Printed in USA. 03/16 PP-775-US-0080

Chapter Factors is published by:

The Midwest Hemophilia Association

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Kansas City, MO 64141
(816) 479-5900
www.midwesthemophilia.org

News and Notes

Courtesy of National Hemophilia Foundation

The United States Department of Health and Human Services designated this March to be the first ever Bleeding Disorders Awareness Month. This news was unveiled at NHF Washington Days along with the Red Tie Challenge. The goal of Bleeding Disorders Awareness Month is to create greater awareness and understanding of hemophilia and all inheritable bleeding disorders. With that said, NHF in partnership with TogoRun, a professional communication company specializing in healthcare, announced with great fanfare…The Red Tie Challenge.

You may have seen the bits and pieces of the Red Tie Challenge on MHA's Facebook page. In a nutshell the Red Tie Challenge is a movement created to start a conversation about bleeding disorders and further support March as Bleeding Disorders Awareness Month. The Red Tie symbolizes the blood ties that bind over 3 million Americans to our community. Close to 400 bleeding disorder advocates from around the country descended on Washington D.C. and took the Red Tie Challenge to Capitol Hill. Members of Congress were asked to conduct a 1-minute floor speech recognizing March 2016 as the Bleeding Disorders Awareness Month, while wearing a red tie (that was provided for them by the advocates). They were also encouraged to post a selfie of themselves wearing the red tie to their social media outlets using the Twitter hashtag #RedTieChallenge, and on their Facebook page. The response was overwhelming. Over 275,000 Twitter impressions were recorded along with over 11 million impressions at 220 media outlets. NHF and the Bleeding Disorders Community reached an audience of 65 million with the Red Tie Challenge during the first-ever Bleeding Disorders Awareness Month, March 2016.

RED TIE CHALLENGE™
REDTIECHALLENGE.ORG

Wow, that is amazing and can demonstrate how important a public awareness campaign can be for our community. Advocates and Congress displayed their red ties very creatively and this was such a good way to increase awareness. The Red Tie Challenge was so well received we unveiled it at Missouri Advocacy Days two weeks later. So when you see all the red ties in the photos you will know what it's all about. I'm sure the Red Tie Challenge will be bigger and better next year. For more information go to *https://redtiechallenge.org/*.

Mark Cox
Executive Director

twitter @MidwestHemo

QUICK NOTES

Message from the President

Hello everyone! MHA has several very exciting things on the horizon. The Strategic Planning Committee is developing a new strategic plan that will in all likelihood cover the next several years. The committee is focused on several objectives one of which is to bolster up our youth education programming. MHA realizes that our youth will soon become our leaders. Our executive director will be attending a seminar that's sole purpose is to help chapters build successful youth programs. The goal is to announce the details sometime towards the end of April/ early May. This new strategic plan will allow the board and its committees to concentrate on its key elements and we are excited that new worthwhile ventures will arise.

As you have read in our executive director's report, MHA is also working with NHF in being part of the Red Tie Challenge. This movement created by the bleeding disorders community and their advocates at the National Hemophilia Foundation (NHF) is to start a conversation about inheritable bleeding disorders and support during the month of March from here on out. March 2016 was the first Bleeding Disorders Awareness Month.

Kyle Rivas Photography

Why the red tie? Because it symbolizes the blood ties that bind our community. How do you take the Challenge in 2017? Get a red tie and record your best tie look, while pledging to support March as Bleeding Disorders Awareness Month, and challenging a few friends, too! Then post your video to Twitter with #RedTieChallenge or post on your Facebook page, and think about making a donation at *redtiechallenge.org*. Be creative and have fun!

Another piece of news is Arrowhead Stadium (Home of the Kansas City Chiefs) was lit up in red in recognition of April 17th at World Hemophilia Day. MHA and Biogen partnered for this exciting and educational event at Arrowhead Stadium. We will post pictures on our social media accounts.

Finally, MHA is now on Twitter. Please follow us @MidwestHemo.

— Aimee Tempera, President

Fighting Our First Inhibitor

Our youngest son has an inhibitor, but we've been fighting it for over a year and are (hopefully) getting it out of his system. When we discovered the inhibitor, we were overwhelmed. More like – terrified. He was only 10 months old, showed no signs of an inhibitor, and we were at the hospital to have him receive a port when we received the news. It was a terrible, horrible, no good, very bad day!

But I'm here to say that inhibitors are not a death sentence. They are game changers, for sure, but with the support of a good medical team, a great dose of patience, and fabulous outreach programs like Inhibitor Conferences, they can be less scary. Manageable, even.

Having the support of a competent medical team, like Dr. Wicklund, Andrew

Wilson and crew in Kansas City, is the first line of defense against inhibitors. They want to eradicate your inhibitor just as much as you do, and they are informed and experienced to do just that. Now I recognize that not all inhibitors are the same – some are much more stubborn and cumbersome than the one our son developed, but a good medical team has insight and experience dealing with inhibitors and can start you on the right track towards tolerization.

Patience, however, and persistence, are equally important in the fight against inhibitors. We started our son's Immune Tolerance Treatment with daily doses of factor. Luckily, with a port and emla cream, he complied without too many complications. But we were tied to those daily infusions, hoping they would work, and that our efforts were not in vain. Monthly labs were drawn to test his inhibitor level as well, and we needed to patiently await results that I was not very patient to receive. But wait we did, and with time his titers dropped, and each time we rejoiced that we were closer to (hopefully) a more normal life.

In the summer, we attended the Inhibitor Summit in Denver, Colorado. And let me tell you, networking with all of the other inhibitor families there was AMAZING! Hearing stories about what has worked (and not worked) for other people, receiving the most recent information about inhibitor research, and just being with other people who were dealing with the same issues as us was a great boost to our morale.

Later in the fall we attended a Regional Inhibitor Conference in Minnesota as well. This one was a much smaller conference, which gave us an opportunity to get to know some people on a much more personal level, which was also fabulous.

While attending this conference, our little inhibitor boy also managed to cut part of his pinky finger off in an escalator at the hotel. Yep, that was another terrible, horrible, no good, very bad day. But the conference staff were wonderful about directing us to the correct hospital with a pediatric hematologist, arranging rides to the hospital for us, helping us order more factor, etc. They cared about us, treated us like family, and made sure we had everything we needed after the accident. We couldn't have asked for better people to help us address our needs in a time of crisis!!

Our little boy is now 2. He runs and dances and jumps off the couch like any other toddler would. He doesn't know we've been fighting an inhibitor for him, and that we are winning the battle one infusion at a time. And I'm ok with that. I hope the inhibitor leaves and never returns. But if it does, I know how I'll fight it again. ■

— By Mareena Snarey

MHA Scholarship

If you are planning on sending a child to college or you yourself are attending college, please make note of the offers detailed here for possible Scholarship money available.

The Midwest Hemophilia Association is pleased to offer a $1,000 scholarship for post-secondary education to a person with a bleeding disorder who lives in the organization's service area. The service area has been defined by the association as western Missouri and eastern Kansas. The scholarship is for continuing education via college or trade school. The scholarship will be applicable to the 2016-2017 school year. The funds may be used at the discretion of the recipient (i.e. books, tuition, room and board, etc.)

If you are interested in receiving an application, please contact Mark Dudley via e-mail at *mdudley@blueridgebank.net*. The application is also available on line at *www.midwesthemophilia.org*. Click on the Resources tab at the top and click on the drop down menu Applications and Registrations. Completed applications must be received no later than June 1, 2016.

Additional scholarship opportunities are available for persons with bleeding disorders such as hemophilia or von Willebrand disease. Contact NHF at 1-800-42- HANDI or their website *www.hemophilia.org* for more information on additional scholarship availability.

— Mark Dudley

2016 MHA CALENDAR

June 17, 2016
Columbia Education Event
Laser Lanes
Columbia, Missouri

July 9, 2016
Springfield Education Event
2K Sports Training
Nixa, Missouri

July 31, 2016 - Aug. 5, 2016
MHA Summer Camp
Lake Doniphan Conference & Retreat Center
Excelsior Springs, Missouri

August 13, 2016
Ozarks Pull for a Cure - A Sporting Clay Event
Ozark Shooters Sporting Complex
Walnut Shade, Missouri

September 9, 2016
MHA Annual Golf Tournament
Drumm Farm Golf Club
Independence, Missouri

September 10 - 11, 2016
MHA Family Fun Fair
Hilton Garden Inn
Independence Missouri

October 8, 2016
Kansas City Hemophilia Walk
Shawnee Mission Park
Shawnee, Kansas

October 16, 2016
Wichita Hemophilia Walk
Exploration Place
Wichita, Kansas

December 3, 2016
MHA Award Banquet
Kauffman Stadium
Kansas City, Missouri

Alphanate®

Antihemophilic Factor/von Willebrand Factor Complex (Human)

Physician **Preferred**

ALPHANATE is the **preferred plasma-derived FVIII** product for the treatment of **hemophilia A** among hematologists practicing in HTCs.*

*Results are statistically significant with a 95% confidence interval with a 6.5% margin of error and are based on a blinded national survey of 75 HTC-based Hematologists from a list of federally and non-federally funded HTCs within the US, conducted and validated by a reputable, independent third party, Adivo Associates LLC, on behalf of Grifols USA from October 2014 - January 2015. In order to qualify to complete the survey, Hematologists were rigorously screened according to market research standards having the necessary experience in the relevant treatment segment. Respondents were asked to assume no difference in terms of availability, cost, and reimbursement when indicating their most preferred plasma-derived FVIII brand.

HTC=Hemophilia Treatment Center; pdFVIII=plasma-derived factor VIII

Learn more at **alphanate.com**

Indications

ALPHANATE® (antihemophilic factor/von Willebrand factor complex [human]) is indicated for:

- Control and prevention of bleeding in patients with hemophilia A
- Surgical and/or invasive procedures in adult and pediatric patients with von Willebrand disease (VWD) in whom desmopressin (DDAVP®) is either ineffective or contraindicated. It is not indicated for patients with severe VWD (Type 3) undergoing major surgery

Important Safety Information

ALPHANATE is contraindicated in patients who have manifested life-threatening immediate hypersensitivity reactions, including anaphylaxis, to the product or its components.

Anaphylaxis and severe hypersensitivity reactions are possible. Should symptoms occur, treatment with ALPHANATE should be discontinued, and emergency treatment should be sought.

Development of activity-neutralizing antibodies has been detected in patients receiving FVIII containing products. Development of alloantibodies to VWF in Type 3 von Willebrand disease (VWD) patients has been occasionally reported in the literature.

Thromboembolic events may be associated with AHF/VWF Complex (Human) in VWD patients, especially in the setting of known risk factors.

Intravascular hemolysis may be associated with infusion of massive doses of AHF/VWF Complex (Human).

Rapid administration of a FVIII concentrate may result in vasomotor reactions.

Plasma products carry a risk of transmitting infectious agents, such as viruses, and theoretically, the Creutzfeldt-Jakob disease (CJD) agent, despite steps designed to reduce this risk.

The most frequent adverse events reported with ALPHANATE in >5% of patients are respiratory distress, pruritus, rash, urticaria, face edema, paresthesia, pain, fever, chills, joint pain, and fatigue.

Please see brief summary of ALPHANATE full Prescribing Information on adjacent page.

You are encouraged to report negative side effects of prescription drugs to the FDA.
Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

For more information: **Grifols Biologicals Inc.**
Tel. 888-GRIFOLS (888-474-3657)

 Printed in USA. April 2015 US/A8/0315/0013

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www.grifols.com

GRIFOLS

ALPHANATE®

Antihemophilic Factor/von Willebrand Factor Complex (Human)

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use Alphanate safely and effectively. See full prescribing information for Alphanate.

ALPHANATE (ANTIHEMOPHILIC FACTOR/VON WILLEBRAND FACTOR COMPLEX [HUMAN])

Sterile, lyophilized powder for injection.

Initial U.S. Approval: 1978

----------INDICATIONS AND USAGE ----------

Alphanate is an Antihemophilic Factor/von Willebrand Factor Complex (Human) indicated for:

- Control and prevention of bleeding in patients with hemophilia A.
- Surgical and/or invasive procedures in adult and pediatric patients with von Willebrand Disease in whom desmopressin (DDAVP) is either ineffective or contraindicated. It is not indicated for patients with severe VWD (Type 3) undergoing major surgery.

----------DOSAGE AND ADMINISTRATION ----------

For Intravenous use only.

Alphanate contains the labeled amount of Factor VIII expressed in International Units (IU) FVIII/vial and von Willebrand Factor:Ristocetin Cofactor activity in IU VWF:RCo/vial.

Hemophilia A: Control and prevention of bleeding episodes

- Dose (units) = body weight (kg) x desired FVIII rise (IU/dL or % of normal) x 0.5 (IU/kg per IU/dL).
- Frequency of intravenous injection of the reconstituted product is determined by the type of bleeding episode and the recommendation of the treating physician.

von Willebrand Disease: Surgical and/or invasive procedure in adult and pediatric patients except Type 3 undergoing major surgery

- Adults: Pre-operative dose of 60 IU VWF:RCo/kg body weight; subsequent doses of 40-60 IU VWF:RCo/kg body weight at 8-12 hour intervals post-operative as clinically needed.
- Pediatric: Pre-operative dose of 75 IU VWF:RCo/kg body weight; subsequent doses of 50-75 IU VWF:RCo/kg body weight at 8-12 hour intervals post-operative as clinically needed.

----------DOSAGE FORMS AND STRENGTHS ----------

- Alphanate is a sterile, lyophilized powder for intravenous injection after reconstitution, available as 250, 500, 1000, 1500 and 2000 IU FVIII in single dose vials.

----------CONTRAINDICATIONS ----------

- Patients who have manifested life-threatening immediate hypersensitivity reactions, including anaphylaxis, to the product or its components.

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

- Anaphylaxis and severe hypersensitivity reactions are possible. Should symptoms occur, treatment with Alphanate should be discontinued, and emergency treatment should be sought.
- Development of activity-neutralizing antibodies has been detected in patients receiving FVIII containing products. Development of alloantibodies to VWF in Type 3 VWD patients has been occasionally reported in the literature.
- Thromboembolic events may be associated with AHF/VWF Complex (Human) in VWD patients, especially in the setting of known risk factors.
- Intravascular hemolysis may be associated with infusion of massive doses of AHF/VWF Complex (Human).
- Rapid administration of a FVIII concentrate may result in vasomotor reactions.
- Plasma products carry a risk of transmitting infectious agents, such as viruses, and theoretically, the Creutzfeldt-Jakob disease (CJD) agent, despite steps designed to reduce this risk.

----------ADVERSE REACTIONS----------

The most frequent adverse events reported with Alphanate in > 5% of patients are respiratory distress, pruritus, rash, urticaria, face edema, paresthesia, pain, fever, chills, joint pain and fatigue.

To report SUSPECTED ADVERSE REACTIONS, contact Grifols Biologicals Inc. at 1-888-GRIFOLS (1-888-474-3657) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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

- Pregnancy: No human or animal data. Use only if clearly needed.
- Pediatric Use: Hemophilia A - Clinical trials for safety and effectiveness have not been conducted. VWD - Age had no effect on PK.

GRIFOLS

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HELPFUL NUMBERS

KANSAS CITY REGIONAL HEMOPHILIA CENTER
1-816-302-6869 • 1-800-236-1713
Dr. Shannon Carpenter • Dr. William Jennings
Dr. Brian Wicklund • Dr. Jill Moormeier
Katie Foote, LMSW • Andrew Wilson, RN, CFNP
Melissa Armanees, RN, CPNP

UNIVERSITY OF MISSOURI HOSPITAL AND CLINICS HEMOPHILIA TREATMENT CENTER
1-573-882-9355
Dr. Barbara Gruner • Dr. Carl Freter
Dr. Tamara Hopkin • Lauren Grana, MSW

NATIONAL HEMOPHILIA FOUNDATION (NHF)
1-800-42-HANDI • www.hemophilia.org

NO INHIBITOR, NO PROBLEM?

By Laurie Kelley

Inhibitor Insights is typically written for families and patients with hemophilia and inhibitors. An inhibitor creates a new level of medical challenges, emotional overload, and lifestyle interference that patients with hemophilia alone don't face. That's why we have a special column just for those with inhibitors.

But not this time. Got hemophilia? Get tested!

That's the message in new guidelines from National Hemophilia Foundation's (NHF) Medical and Scientific Advisory Council (MASAC). If you have hemophilia, you should be tested for inhibitors at least annually. That goes for your child with hemophilia, too.

Inhibitors are a frightening complication of hemophilia. An inhibitor is an antibody, created by the immune system in response to what the body believes is a foreign and potentially harmful substance—factor. The inhibitor inactivates or neutralizes the injected factor so that it can't clot blood.

If factor is a normal part of the body, then why does the body reject it? When there is little to no factor in the body, and some is injected, the body does not recognize it and initiates an immune response against the factor. That's why mainly people with severe hemophilia develop inhibitors. Moderates and milds usually make enough factor for the body to recognize it when injected.

The US Centers for Disease Control (CDC) estimates that within a lifetime, up to one in five people with hemophilia will develop an antibody (inhibitor) to the infused factor that is used to treat bleeding episodes. But some patients are more at risk than others. What are your chances? And do you really need to be tested?

RISK FACTORS

The incidence of inhibitors in severe hemophilia A patients is about 20% to 30% and about 2% to 3% for people with hemophilia B. Yet certain people with hemophilia have a higher risk of developing inhibitors: people with severe hemophilia A or B; people with certain genetic defects in the gene coding for factor VIII or IX; people with a family history of inhibitors; African Americans; and people receiving infusions while fighting an illness or infection.

The greatest risk of developing an inhibitor is within the first 50 infusions of factor, yet an inhibitor can develop at any age and even after hundreds of infusions.

Although we often worry most about babies developing inhibitors during those first 50 or so infusions, older children, teens, and adults can also be at risk. I know a 20-year-old with mild hemophilia who got an inhibitor after a sports injury. He hadn't had factor in years, and then bombarded his system with 100% levels for five days to treat the injury. Boom—inhibitor.

Though there are certain risk factors, anyone can get an inhibitor. And so far, no one knows why they develop.

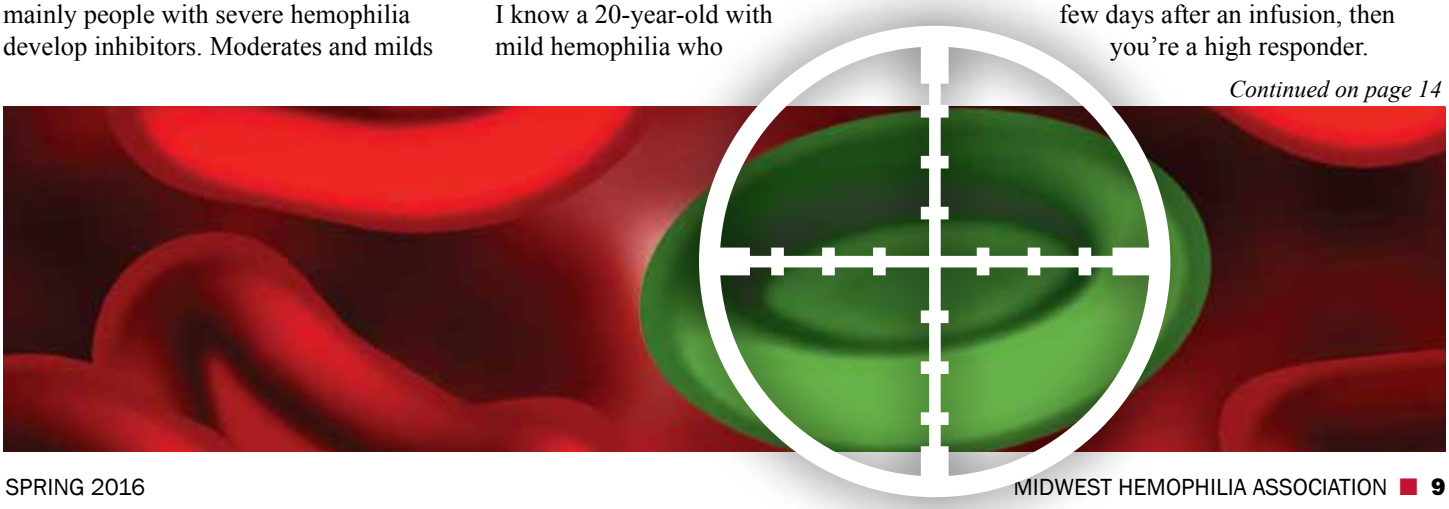
WHAT TESTING SHOWS

A blood test will determine whether you have (or your child has) antibodies to factor. The Bethesda inhibitor assay is a test that measures the titer (strength) of the inhibitor, described as Bethesda units (BU). An inhibitor titer can be 1 BU, 10 BU, or even as strong as 10,000 BU.

Inhibitors are classified in two ways: by titer and by the immune system's response to infused factor. An inhibitor less than or equal to 5 BU is a low-titer inhibitor; an inhibitor greater than 5 BU is a high-titer inhibitor. Low-titer inhibitors are preferable because you may be able to effectively treat bleeds by infusing larger-than-normal doses of standard factor concentrate.

If the inhibitor titer is less than or equal to 5 BU and remains at that level even after a factor infusion, then you are (or your child is) a low responder. If the inhibitor titer rises above 5 BU within a few days after an infusion, then you're a high responder.

Continued on page 14



First Time Advocate

2016 Washington Days

By Jaci Colter

What does it mean to be an advocate? A couple of months ago, I'm not sure that I could have answered this question. When asked to participate in Washington Days, I was somewhat hesitant as I am certainly not a professional lobbyist nor am I an expert in the area of bleeding disorders. I wasn't really sure what I could bring to the table, so to speak.

My son, Owen, was diagnosed at 9 months of age with severe Hemophilia B. We have no family history, so prior to his diagnosis we had only a vague knowledge of what hemophilia was. My husband, Brad, and I felt that it was our job as parents to learn as much as we could about anything relating to hemophilia. Participating in Washington Days presented an opportunity to learn even more about the bleeding disorder community.

The first day in D.C. was spent training on what to expect from the hill congressional visits. I left the training sessions feeling much more prepared for the day ahead. It was reassuring to know that we weren't out on our own and would be going to each appointment with other constituents from our state. We were also armed with fact sheets providing information about the requests we were going to be making the next day.

I was pretty terrified to go into my first appointment, but as the day went on it became much easier and I found that the staff members were really listening to what we had to say. I had printed a book as a leave behind for each appointment telling Owen's story with various pictures of our journey so far. I figured that even if they threw the book away after I left; it had still made an impact. One thing that I found very positive was that some of the congressional staff members recognized advocates that they had met on previous visits and were able to ask some very specific questions about the requests we were making. By the end of the day, I was exhausted but felt a huge sense of accomplishment for what I had done.

Beyond my experiences on Capitol Hill, one of the most beneficial parts of Washington Days was meeting with other members of the bleeding disorder community. There were people from all walks of life; children and moms of children with bleeding disorders, and adults that have lived with bleeding disorders for decades through the many ups and downs. I was able to sit and visit with so many different people that I might not have otherwise met. One mom in particular had some very similar experiences to my own and was able to offer some suggestions that I will definitely use at our next Hematologist visit.

As I scroll through my social media sites, I have seen countless people posting pictures in their red ties in support of NHF's Red Tie Challenge. Many of these are Congressmen wearing their red ties and speaking publicly to raise awareness for bleeding disorders. This is a direct impact of our efforts during Washington Days. Regular people, uniting for a cause, voicing their concerns and telling their personal stories have made it to our lawmakers and onto the general public. This is what it means to be an advocate.

This was my first time participating in Washington Days, but it won't be my last and I would strongly encourage anyone that has an opportunity to visit their government officials, both in Washington and locally, to do so. ■

Top Photo: Jaci Colter, left, and Congressman Sam Graves (R-MO).
Middle Photo: Jaci Colter, left; Val Bias, National Hemophilia Foundation President and CEO; and Bridget Tyrey, GHA Executive Director.
Bottom Photo: Mark Cox, MHA Executive Director, left; Congressman Kevin Yoder, (R-KS); and Brooke Connell, MHA Vice President.

ELOCTATE®
[Antihemophilic Factor (Recombinant), Fc Fusion Protein]

THE FIRST FACTOR VIII WITH A PROLONGED HALF-LIFE

Learn how a prolonged half-life may affect your infusion schedule

Meet your CoRe Manager Vicki Oberkrom
E: vicki.oberkrom@biogen.com T: 660-281-2447

Indications

ELOCTATE, [Antihemophilic Factor (Recombinant), Fc Fusion Protein], is a recombinant DNA derived, antihemophilic factor indicated in adults and children with Hemophilia A (congenital Factor VIII deficiency) for: on-demand treatment and control of bleeding episodes, perioperative management of bleeding, and routine prophylaxis to reduce the frequency of bleeding episodes. ELOCTATE is not indicated for the treatment of von Willebrand disease.

Important Safety Information

Do not use ELOCTATE if you have had an allergic reaction to it in the past.

Tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines, supplements, or herbal medicines, have any allergies, are breastfeeding, are pregnant or planning to become pregnant, or have been told you have inhibitors (antibodies) to Factor VIII.

Allergic reactions may occur with ELOCTATE. Call your healthcare provider or get emergency treatment right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash, or hives.

Your body can also make antibodies called, "inhibitors," against ELOCTATE, which may stop ELOCTATE from working properly.

The most frequently occurring side effects of ELOCTATE are headache, rash, joint pain, muscle pain and general discomfort. These are not all the possible side effects of ELOCTATE. Talk to your healthcare provider right away about any side effect that bothers you or that does not go away, and if bleeding is not controlled after using ELOCTATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see Brief Summary of full Prescribing Information on the next page.

This information is not intended to replace discussions with your healthcare provider.

Biogen

FDA-Approved Patient Labeling
Patient Information
ELOCTATE® /el' ok' tate /
[Antihemophilic Factor (Recombinant), Fc Fusion Protein]

Please read this Patient Information carefully before using ELOCTATE and each time you get a refill, as there may be new information. This Patient Information does not take the place of talking with your healthcare provider about your medical condition or your treatment.

What is ELOCTATE?
ELOCTATE is an injectable medicine that is used to help control and prevent bleeding in people with Hemophilia A (congenital Factor VIII deficiency).

Your healthcare provider may give you ELOCTATE when you have surgery.

Who should not use ELOCTATE?
You should not use ELOCTATE if you had an allergic reaction to it in the past.

What should I tell my healthcare provider before using ELOCTATE?
Talk to your healthcare provider about:

- Any medical problems that you have or had.
- All prescription and non-prescription medicines that you take, including over-the-counter medicines, supplements or herbal medicines.
- Pregnancy or if you are planning to become pregnant. It is not known if ELOCTATE may harm your unborn baby.
- Breastfeeding. It is not known if ELOCTATE passes into the milk and if it can harm your baby.

How should I use ELOCTATE?
You get ELOCTATE as an infusion into your vein. Your healthcare provider will instruct you on how to do infusions on your own, and may watch you give yourself the first dose of ELOCTATE.

Contact your healthcare provider right away if bleeding is not controlled after using ELOCTATE.

What are the possible side effects of ELOCTATE?
You can have an allergic reaction to ELOCTATE. Call your healthcare provider or emergency department right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash or hives.

Your body can also make antibodies called, "inhibitors," against ELOCTATE. This can stop ELOCTATE from working properly. Your healthcare provider may give you blood tests to check for inhibitors.

Common side effects of ELOCTATE are headache, rash, joint pain, muscle pain and general discomfort.

These are not the only possible side effects of ELOCTATE. Tell your healthcare provider about any side effect that bothers you or does not go away.

How should I store ELOCTATE?

- Keep ELOCTATE in its original package.
- Protect it from light.
- Do not freeze.
- Store refrigerated (2°C to 8°C or 36°F to 46°F) or at room temperature [not to exceed 30°C (86°F)], for up to six months.
- When storing at room temperature:
- Note on the carton the date on which the product is removed from refrigeration.
- Use the product before the end of this 6 month period or discard it.
- Do not return the product to the refrigerator.

Do not use ELOCTATE after the expiration date printed on the vial or, if you removed it from the refrigerator, after the date that was noted on the carton, whichever is earlier.

After reconstitution (mixing with the diluent):

- Do not use ELOCTATE if the reconstituted solution is not clear to slightly opalescent and colorless.
- Use reconstituted product as soon as possible.
- You may store reconstituted solution at room temperature, not to exceed 30°C (86°F), for up to three hours. Protect the reconstituted product from direct sunlight. Discard any product not used within three hours.

What else should I know about ELOCTATE?
Medicines are sometimes prescribed for purposes other than those listed here. Do not use ELOCTATE for a condition for which it was not prescribed. Do not share ELOCTATE with other people, even if they have the same symptoms that you have.

44279-02

Manufactured by:
Biogen Inc.
Cambridge, MA 02142 USA
U.S. License # 1697

ADVOCACY in Missouri

Advocates from across Missouri visited the capitol building in Jefferson City on March 9, 2016 for Missouri Advocacy Days.

No Inhibitor, No Problem?

Continued from page 9

Treating a bleed in the presence of high-responding inhibitors requires special factor concentrates because standard factor concentrates are not effective at stopping a bleed.

Not all inhibitors are troublesome. Low-responding inhibitors that disappear spontaneously after weeks or months are called transient inhibitors. Of course the ones that stick around are the most worrisome, but all inhibitors should be considered serious, and need expert treatment by your HTC. In people with hemophilia and inhibitors, bleeds can be treated with special factor concentrates known generically as bypassing agents. Inhibitors can often be eliminated through a process called immune tolerance therapy (ITT) or immune tolerance induction therapy.

BE PREPARED

Don't wait until you notice that factor isn't working as it should. Don't wait for a bleed to worsen, while you wonder what's happening. Don't assume you need to give just one more infusion, or a higher dose, to stop that bleed. And even if you have mild hemophilia, don't believe that inhibitors could never happen to you—or to your child.

Get tested annually, even when you don't show any signs of an inhibitor. And always get tested before any surgery.

NHF recommends you take these steps:

- Ask your doctor about your risk for an inhibitor, how often you should be tested for inhibitors, and what you can do to help avoid developing one.
- Participate in the Community Counts program and take advantage of the free inhibitor testing provided as part of this CDC project.
- Participate in NHF's My Life, Our Future genotyping program; your genotype (the specific genetic change that causes you to have hemophilia) is a key indicator of your inhibitor risk.
- Participate in research studies because it takes data from lots of patients to identify the major risk factors for inhibitors.

MASAC's guidelines are based on results from the CDC's Hemophilia Inhibitor Research Study (HIRS). HIRS investigators and CDC researchers found that (1) people with hemophilia of all ages are at risk for developing an inhibitor and (2) unless people are regularly tested for an inhibitor, they may not know they have one until it causes a severe bleeding problem.

Don't wait until inhibitors are active and doing their work. Ask yourself: When was the last time—if ever—that I was tested, or my child was tested, for an inhibitor?

Make an appointment today! ■

INTRODUCING

ADYNOVATE
[Antihemophilic Factor (Recombinant), PEGylated]

PROVEN PROPHYLAXIS +
SIMPLE,* TWICE-WEEKLY DOSING SCHEDULE =

moments YOUR WAY

*ADYNOVATE allows you to infuse on the same 2 days every week.

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] Important Information

Indication

ADYNOVATE is used on-demand to control bleeding in patients 12 years of age and older with hemophilia A. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

You should not use ADYNOVATE if you:
- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE [Antihemophilic Factor (Recombinant)]

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

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 mice or hamsters.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE 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 VIII.

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

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do 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 following page for ADYNOVATE Important Facts.

For full Prescribing Information visit www.ADYNOVATE.com.

Reference: 1. ADYNOVATE Prescribing Information. Westlake Village, CA: Baxalta US Inc.

Baxalta

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated]

Important facts about

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated]

This leaflet summarizes important information about ADYNOVATE. 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 ADYNOVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADYNOVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADYNOVATE so that your treatment will work best for you.

What is ADYNOVATE?

ADYNOVATE is an injectable medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia). Hemophilia A is an inherited bleeding disorder that prevents blood from clotting normally.

ADYNOVATE is used on-demand to control bleeding in patients 12 years of age and older with hemophilia A. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

Who should not use ADYNOVATE?

You should not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE

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

How should I use ADYNOVATE?

ADYNOVATE is given directly into the bloodstream.

You may infuse ADYNOVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADYNOVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADYNOVATE to use based on your individual weight, level of physical activity, the severity of your hemophilia A, and where you are bleeding.

Reconstituted product (after mixing dry product with wet diluent) must be used within 3 hours and cannot be stored or refrigerated. Discard any ADYNOVATE left in the vial at the end of your infusion as directed by your healthcare professional.

How should I use ADYNOVATE? (cont'd)

You may have to have blood tests done after getting ADYNOVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

Call your healthcare provider right away if your bleeding does not stop after taking ADYNOVATE.

What should I tell my healthcare provider before I use ADYNOVATE?

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 mice or hamsters.
- Are breastfeeding. It is not known if ADYNOVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What are the possible side effects of ADYNOVATE?

You can have an allergic reaction to ADYNOVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do not go away.

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

What else should I know about ADYNOVATE and Hemophilia A?

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE 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 VIII.

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

The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADYNOVATE. The FDA approved product labeling can be found at www.ADYNOVATE.com or 855-4-ADYNOVATE.

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.

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It's Time to Help Support Your Hemophilia Treatment Center

Just make the choice to get your factor from the Children's Mercy Hospital 340B program and you are helping to support your HTC team... it's that simple! All proceeds go to support the specialized clinicians, physical therapy, training, and other essential services you and your family have come to depend on at Children's Mercy Hospital.

Did you know that our factor program provides:

- Peace of mind... Hematologists are on call 24 hours a day, 7 days a week
- All brands of factor in a wide assortment of vial sizes
- A broad list of infusion supplies
- Customer Care Coordinators who know Hemophilia and are ready to be of assistance
- Interpreters who are fluent in over 180 different languages
- Coordination of in-home nursing services
- Shipments to your home or choice of location

Contact us at 816-221-4544
Ask for Cyndi Donate

Children's Mercy

Study of 50 Years of Hemophilia Healthcare Outcomes Yields Surprises

Results from a new study indicate that despite 50 years' worth of advances in the area of comprehensive care for patients with bleeding disorders, males with hemophilia still grapple with significant health-related issues affecting their quality of life. The study, "Men with Severe Hemophilia in the United States: Birth Cohort Analysis of a Large National Database," was published online on March 16, 2016, in the journal Blood. The lead author of the article was Paul E. Monahan, MD, of the Gene Therapy Center at the University of North Carolina at Chapel Hill. Co-investigators included professionals from the hemophilia treatment center (HTC) network and the Centers for Disease Control and Prevention (CDC).

To better understand the connections between the many changes in hemophilia healthcare and the dynamics affecting overall health, Monahan and his team reviewed data collected from 4,899 men with severe hemophilia and 2,587 men with mild hemophilia. All of these men had received care at HTCs from 1998-2011. Data were organized into four time periods, or "eras," representing major healthcare developments and therapeutic breakthroughs relevant to people with hemophilia: Era A included the oldest group, men born prior to 1958; Era B grouped men born between 1958 and1975; Era C included men born from 1976-1982; and Era D represented the youngest group, men born between 1982 and1993.

The main findings of the study included:

- In Era D more than one in three men with severe hemophilia reported frequent bleeds (more than five bleeds in six months), despite being treated with the most modern therapies. One in four of these men also reported a recurrent bleed in a "target joint."
- Across all eras, compared to men with mild disease, those with severe hemophilia were about three times more likely to report activity limitations. Further, they were twice as likely to report some use of assistive devices to help them move around, such as a cane or wheelchair.
- In every era, the proportion of men with severe hemophilia that missed at least 10 days of work or school in the last year due to upper or lower joint problems was two or three times that of men with mild hemophilia.
- Nearly half of the men in Era A were disabled and unable to work. Moreover, men with severe hemophilia were about three times more likely to be disabled as their mild hemophilia counterparts in every era.
- Infection-related health problems due to hepatitis B, hepatitis C and HIV are common among men with severe hemophilia, particularly in the older eras.
- Of the 551 deaths reported during the study period, liver failure was the most commonly reported cause of death, regardless of hemophilia severity or era. Bleed-related deaths accounted for 14.6% of deaths in men with severe hemophilia and 10.7% of deaths in men with mild hemophilia across all eras.

The study also yielded some unexpected findings. The overall rates of joint bleeding remain relatively high, even with the availability of more effective treatments. Also, despite the proven effectiveness of prophylactic factor therapy in preventing joint damage, men in all eras continue to underuse this option.

"Clear disparities remain in terms of frequent bleeding and disability between men with severe hemophilia and mild hemophilia across every decade of adult life. We thought the difference in functional outcomes would have narrowed over the years; that is, men with severe hemophilia should look more like those with mild disorder given improved therapeutics and access to care, but this wasn't the case," said Monahan. "What needs examination is why—despite widespread availability of preventive and on-demand therapies for home use—we still see disparities. It speaks to the need for continued disease surveillance to monitor and inform hemophilia interventions and outcomes." ■

– Source: PRNewswire, March 16, 2016

MOVING? New Address

Name

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