Winter/Spring 2018

KHF Hemosphere

4th Annual Hemophilia Walk

The 2017 Kentucky Hemophilia Walk rallied our bleeding disorders community to fundraise for a common cause in support of the programs and services the Kentucky Hemophilia Foundation provides year-round. We are so proud of their peer fundraising achievements and so very grateful. The extent of our service delivery is directly related to the outcome of this fundraiser.

Congratulations to all the teams and walkers who participated. Wetherby Park in Middletown was the perfect venue for this community event. The Halloween theme was well received, and many "ghoulish" costumes were strolling through the park. Everyone wore a smile. It was great fun to watch all the kiddos' excitement as they collected Trick or Treat candy along the way, jumped around in the Bouncy House, visited with the clowns for face painting and balloon animals and then had an opportunity to paint their own pumpkins to take home. Door prizes and prizes for best costumes added to everyone's merriment.

Twenty teams and a total of 180 walkers raised $39,086 or 78% of our $50,000 goal. We thank you so much, especially the Team Captains who encouraged, motivated, and inspired their teams! We also thank our local sponsors and donors: CSL Behring and Novo Nordisk, Gold Sponsors; CVS Caremark, Silver Sponsor; First Choice Home Infusion, Genentech, Kosair Charities, Bronze Sponsors; Matrix Health and Republic Bank, Kilometer Sponsors; Papa John's Pizza for their generous pizza donation, and our many volunteers who ensured the success of this marvelous community fundraiser.

The list of the top fundraisers can be found on page 4.

See more photos of the Walk and other KHF activities inside

UDC Data on Females with Bleeding Disorders

The Hemophilia Treatment Centers (HTCs) Network Investigators, a group of researchers from several U.S. HTCs, recently published a study on disparities associated with bleeding symptoms, age at diagnosis and provider interventions for females with bleeding disorders. Females living with an undiagnosed bleeding disorder are unwittingly foregoing the care and resources that can have real world implication for their quality of life, including appropriate treatment, education and psychosocial support.

Investigators focused, in part, on the differences in phenotype of adolescent vs adult women with heavy menstrual bleeding (HMB) and a bleeding disorder (BD). Study data was drawn from the U.S. Centers for Disease Control and Prevention's Universal Data Collection (UDC) surveillance project, which culled information annually on enrolled patients at 135 federally funded HTCs. The project included annual data from 1998-2011. In 2009 a female component was added to the UDC which was implemented in 23 HTCs. The module was designed to capture data related to diagnoses, menstrual bleeding, other bleeding symptoms, obstetrical-gynecological symptoms, treatment and gynecological/reproductive history.

The UDC data encompassed 269 females with HMB/BD, including von Willebrand disease (VWD), other factor deficiencies and platelet disorders. Of these, 79 were adolescents (median age 16) and 190 were adults (median age 27). The majority enrolled were diagnosed with VWD (223; 83%) with the remaining diagnosis encompassing other factor deficiencies, platelet function disorders and unspecified BDs.

The median age at diagnosis was 12 for adolescents vs. 16 for adults. Forty-five adolescents (57%) and 138 adults (73%) experienced a delay in diagnosis, defined by the number of years passed since a patient's first bleed. Srivaths and her co-authors observed that there was a reduction in long delays in diagnosis for adolescents vs. adults and that this could be an indicator of a positive trend. "We suspect the difference in delay in diagnosis in adults vs. adolescents is because of improved earlier diagnosis due to greater awareness in the past decade through national advocacy organizations such as the National Hemophilia Foundation and the Foundation for Women and Girls with Blood Disorders."

Differences were also seen in bleeding phenotypes of adolescent vs. adult females with HMB/BD. Adults experienced more frequent bleeding complications, anemia, gynecologic procedures and surgeries. While both groups were treated with hormonal therapy and antifibrinolytic agents equally, the synthetic hormone desmopressin acetate was utilized more often by adolescents due in part to its ease of administration via nasal spray. Adults more often opted for hormonal therapy and surgeries. The authors suggested that this was due to the additional contraceptive benefits of hormonal therapy and a preference for the type of "definitive outcome" associated with surgical interventions.

The authors cited several study limitations, including those associated with a small patient sample size and the use of retrospective, self-reported study data based on patient recall. Data were also limited to patients enrolled at HTCs, which may not be representative of the broader U.S. population. Despite the limitations, Srivaths and fellow investigators note that this study is important as it represents the first concerted effort to compare the bleeding phenotype, diagnosis and management of females with HMB/BD. They acknowledge that while public awareness related to women affected with bleeding disorders has been enhanced in recent years, that additional studies are necessary to establish definitively whether better patient/healthcare provider education translates to better outcomes. The results of such studies could inform future efforts to reduce hematologic/gynecologic complications for females with BD/HMB.

The article "Differences in Bleeding Phenotype and Provider Interventions in Postmenarchal Adolescents when Compared to Adult Women with Bleeding Disorders and Heavy Menstrual Bleeding," was published in the January issue of the journal *Haemophilia*. The lead author was Lakshmi V. Srivaths, department of pediatrics, section of hematology/oncology at Baylor College of Medicine in Houston, TX.

Prophylaxis Could Reduce Hospitalizations for VWD Patients

Researchers from Sweden and the U.S. recently published an article that focused on potential correlations between patients with von Willebrand disease (VWD), hospitalizations and prophylaxis. The authors conducted a retrospective study of inpatients and outpatients, both with and without VWD. Their objective was to investigate the frequency of hospital admittances and determine whether the implementation of a prophylactic treatment regimen is associated with a reduction in hospitalizations.

The lead author of the article was Elena Holm, MD, Department of Translational Medicine, Lund University, Skåne University Hospital in Lund, Sweden. Holm and her colleagues were joined by Thomas Abshire, BloodCenter of Wisconsin and Departments of Pediatrics and Medicine, Blood Research Institute, Medical College of Wisconsin in Milwaukee.

The authors reviewed patient data from two primary groups. The first group encompassed population-based registers from the National Board of Health and Welfare and Statistics Sweden. Data from these registries were incorporated into Sweden's Congenital Bleeding Disorders study. These registries included 2,790 individuals with a diagnosis of VWD between the year 1987 and 2009. They found that VWD patients were admitted to hospitals at a rate 2.3 times higher than the unaffected control groups, and spent on average, 2.0 times as many days as hospitalized inpatients. The most common impetus for these hospitalizations were gastrointestinal (GI) bleeding, menorrhagia (heavy menstrual bleeding) and epistaxis (nose bleeds). Outpatient visits were also twice as common amongst VWD patients.

For the second segment of their research, investigators tapped the von Willebrand Disease Prophylaxis Network (VWD PN), an international study group established to evaluate the prophylactic regimens of patients with VWD. In all, 105 patients from participating treatment centers in North America and Europe were counted in this study, including individuals with type 3 (52%), type 2A (22%), type 1 (12%), type 2B (9%) and other types (4%). As in the registries, GI bleeding was the most common cause of hospitalization. Of the 122 bleed-related hospitalizations reported, 75 occurred prior to the initiation of prophylaxis and 47 after start of prophylaxis, which translates to 712 and 448 events per 1000 patient years. These findings would indicate that significantly fewer hospitalizations occurred after the initiation of a prophylactic treatment regimen.

The authors cited limitations such as a dearth of data on additional variables that could inform study conclusions and a lack of information that could help remove sources of bias or to investigate outcomes related to VWD type or mode of treatment. On the other hand, a major strength of this type research is that general population data fed by national registries allow investigators access to decades worth of healthy control data to match with affected patients, allowing for long term comparisons.

Holm and her fellow investigators also note the potential positive impact of prophylaxis in VWD patients as demonstrated by the VWD PN.

"The VWD PN enrolled the largest cohort using prophylaxis for the management of VWD, concluded the authors. "Prophylaxis using well defined regimens, as in this study, reduced the need for in- and outpatient visits which should translate to increased quality of life for patients and their families."

The article "Bleeding-related Hospitalization in Patients with von Willebrand disease and the Impact of Prophylaxis: Results from National Registers in Sweden Compared with Normal Controls and Participants in the von Willebrand Disease Prophylaxis Network," was published in the February 2018 edition of the journal *Haemophilia*.

Event News

Fall/Winter Highlights

Gettin' in the Game

Gettin' in the Game participants, Carter Tierney from Owensboro and Brody Vanderpool from Owingsville, Kentucky, had a great time at the Gettin' in the Game Junior Championships in Phoenix, AZ. Participants may choose golf, baseball, or swimming as their sports discipline for participation in these championships. Prior experience is not required. The Gettin' in the Game Junior Championships are an annual program provided by CSL Behring

Walk Winners

The culmination of the Walk was the announcement and awarding of prizes for top fundraisers among individuals and Walk Teams. The top teams were: Mac's Pack from Frankfort in 1st place with $4,279, Tag's Turtles from Bowling Green in 2nd place with $3,475, Team LEVI from Cynthiana in 3rd place with $3,166, Team Jordan from Louisville in 4th place with $2,505, Andy's All-Stars from Almo in 5th place with $1,745, Jackson's Globeclotters from Louisville in 6th place with $1,737, Team Jack from Louisville in 7th place with $1,135, and Team Brody from Owingsville in 8th place with $1,123. The top five individual fundraisers among teams were: Tag Poynter with $2,730, Karen Lucky with $2,416, Alane Foley with $1,421, Dianne Hardman with $1,040, and Pat Tharp with $1,000.

Our next Walk is on Saturday, September 29th. Teams have already started fundraising. Start your team today! Register at **www.uniteforbleedingdisorders.org/event/KY18**

Fall/Winter Highlights

Year-End Holiday Event

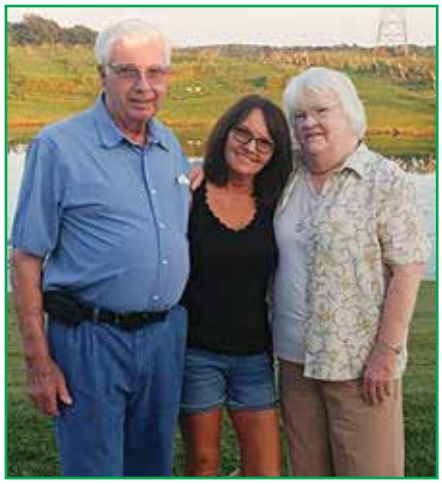
The annual Holiday celebration on December 3 was a festive and fabulous event. 138 adults and children from far and near gathered for this popular family event at the Clifton Center in Louisville. Chef John's hors d'oevres were once again a hit, and through Santa's magic each child in attendance received a gift lighting up many happy little faces. The adults enjoyed chatting with old and new friends, bidding on silent auction items, visiting with exhibitors, and sampling the various bake contest entries. Nicki Lennon won the bake contest with her scrumptious Chocolate Chip Cheesecake Bars. Runner up was Jessica Masticola with her Carmelitas, and in third place Myra Loeser with her Pumpkin Chocolate Cake.

Door prizes and gifts for our 2017 volunteers who helped with various events and fundraisers throughout the year concluded the event. New this year was DJ Axel who delighted our guests with a wonderful selection of all-around and seasonal favorites. We thank our exhibitors for their participation in this event: Bayer HealthCare, CSL Behring, CVS Caremark, First Choice Home Infusion, Genentech, Grifols, Novo Nordisk, Octapharma, Pfizer, and Shire. Many thanks to our guests for donating silent auction items, our bake contest participants for showcasing their talents, and our much appreciated volunteers.

Poinsettia Fundraiser

For many years after the Kentucky Hemophilia Foundation was formed in 1960, selling poinsettias was the only fundraiser our organization engaged in to raise money for programs and services, but times and needs have changed. Poinsettias are easily available in a variety of retail stores, and many other non-profit organizations, schools, even prisons are also selling them. As a result, our sales have declined, but we are working hard with the help of several dedicated volunteers to keep this fundraiser going.

We want to thank these volunteers for taking orders from friends, family members, and co-workers and encouraging their churches to order from us as well. They are Lisa Blair, Louisville; Dianne and Steve Burnett, Louisville; Marion Forcht, Corbin; Janet Goff and Sharon McMahan, Owensboro; Deborah Hitt, Shelbyville; Myra Loeser, Louisville; Eric Marcum, Louisville; Jimmy Maricle, Louisville; Jim and Doris Ray, Louisville; Sadalia Sturgill, Lebanon Junction; Nita Wayne-Zehnder, Louisville; and Gail Yates, Louisville. We would like to recognize and thank Janet Goff especially for her decades-long commitment to selling hundreds of poinsettias and spring flowers each year in the Owensboro area. This season her daughter, Sharon McMahan, has taken the baton and stepped in her mother's footsteps going forward. They are big shoes to fill, but Sharon has already made great strides. We hope that other volunteers may step forward in the future to help us with this fundraiser.



More News

Moving into Spring....

Kentucky Advocacy Day

By all accounts, KHF's 5th Annual Advocacy Day in Frankfort was a successful event for Kentucky's bleeding disorders community to have their voices heard and tell their stories, express their concerns and educate legislators regarding the ramifications of living with a very expensive, incurable condition and resulting needs and challenges. Main talking points revolved around providing access to care, preservation of Medicaid expansion, and affordable co-pays and insurance premiums.

After an early morning continental breakfast and orientation, our teams met with their respective legislators. At noon, our group joined NORD (National Organization for Rare Disorders) representatives and guests in the Capitol rotunda as several speakers addressed issues involving rare diseases and then witnessed the presentation of proclamations for Rare Disease Day to Patrick & Jennifer Dunegan of NORD and for Bleeding Disorders Awareness Month to Ursela Kamala of KHF. The presentations were made by Kentucky's Secretary of State, Alison Grimes Lundergan.

A wrap-up luncheon was held at Serafini Restaurant in Frankfort. We thank our Kentucky bleeding disorder advocates, our facilitators, our planning committee (Chris Poynter, Melissa Bowie, Sara Ceresa, Brendan Hayes, Cory Meadows, Roy Pura, Lisa Raterman, and James Romano), and our sponsors: CSL Behring, Genentech, Novo Nordisk, Pfizer, and Shire. We were honored to coordinate this important advocacy event with the Tri-State Bleeding Disorder Foundation (TSBDF) and NORD (National Organization for Rare Disorders). TSBDF serves several counties in northern Kentucky along with KHF. Hemophilia and similar bleeding disorders are counted among 7000 identified rare disorders.

Note: In the United States, the Rare Diseases Act of 2002 defines rare disease strictly according to prevalence, specifically "any disease or condition that affects fewer than 200,000 people in the United States," or about 1 in 1,500 people.

Washington Days

In March, KHF was represented by an enthusiastic and experienced group of Kentucky Advocates during the National Hemophilia Foundation's (NHF's) advocacy event in Washington, DC, appropriately called "Washington Days." Among several hundred advocates from all over the country were Eric, Venus, and Drew Marcum, Sara Ceresa, and Myra Loeser who met with their members of Congress to advocate for protections for their fellow Kentuckians affected by bleeding disorders. We thank them for their efforts on the national level.

More News

2018 Spring Semester Scholarship Awards

KHF was pleased to award two $500 post-secondary scholarships for the 2018 spring semester. John Rhea received the Herb Schlaughenhoupt, Jr. Memorial Scholarship and Andrew Harmon the Betty Meadors Mattingly Memorial Scholarship.

John, whose family lives in Louisville, is pursuing a master's degree in Health Administration at the University of Kentucky with an anticipated graduation date of May 2019. In his spare time, John enjoys music, sports, and spending time with family and friends.

Andrew, whose family resides in Bedford, attends Midway University. His major is elementary education, and he anticipates receiving his bachelor's degree also in May 2019. Andrew enjoys sports, photography, and reading. We extend congratulations and best wishes to both young men for their accomplishments.

In Memory

October 1, 2017 – March 31, 2018

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

Jim Banta
Gail Yates

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

Alma Fryman
Danny & Karen Combs
Good Done Great for
Toyota Employees
Cathy M. Jackson
April Mason
M. & B. P. Stockton
Emily Lander, Mary Montague,
Cheryl Hughes, Craig Grucza
(Toyota Employees)

Alan Taylor Hall
Norma Hall
Terry & Elizabeth Watts

William Walter Hall
Norma Hall
Emma Schafer & Nancy Schafer
Terry & Elizabeth Watts

Fred Hartman
Bradley & Melissa Haynie
Vivian Marcum
Elizabeth A. Rhodes

Brenda Fryman Poe
David & Kathy Grear
Kathi & Mark Patrick
Anita Tackett

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.

Traveling this summer? You can always reach out to **KHF** at **www.kyhemo.org**

KOSAIR CHARITIES

WHAS CRUSADE FOR CHILDREN

COMMUNITY HEALTH CHARITIES KENTUCKY

COLONEL HONORABLE ORDER OF KENTUCKY COMMONWEALTH OF KENTUCKY

KENTUCKY HEMOPHILIA FOUNDATION
1850 Taylor Avenue #2
Louisville, KY 40213-1594

Non Profit Org.
U.S. Postage
PAID
Louisville, KY
Permit No. 883

Aptevo BioTherapeutics LLC, Berwyn, PA 19312

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated]

ADYNOVATE® is FDA approved for patients of all ages with Hemophilia A

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

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

The pediatric study of children <12 years of age (N=66) evaluated the immunogenicity, efficacy, PK (as compared to ADVATE® [Antihemophilic Factor (Recombinant)]), and safety of ADYNOVATE twice-weekly prophylaxis (40-60 IU/kg) and determined hemostatic efficacy in the treatment of bleeding episodes for 6 months.[1,2]

The pivotal trial of children and adults ≥12 years (N=137) evaluated the efficacy, PK, and safety of ADYNOVATE twice-weekly prophylaxis (40-50 IU/kg) vs on-demand (10-60 IU/kg) treatment, and determined hemostatic efficacy in the treatment of bleeding episodes for 6 months.[1]

+Children (<12 years) experienced a median overall ABR of 2.0 (IQR: 3.9) and a median ABR of zero for both joint (IQR: 1.9) and spontaneous (IQR: 1.9) bleeds[1,3]

+38% (n=25) of children (<12 years) experienced zero total bleeds; 73% (n=48) experienced zero joint bleeds; and 67% (n=44) experienced zero spontaneous bleeds[1]

Talk to your doctor and visit ADYNOVATE.com

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

Indications

ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. 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 the following page for ADYNOVATE Important Facts.

For full Prescribing Information, visit www.ADYNOVATE.com.

References: 1. ADYNOVATE Prescribing Information. **2.** Mullins ES, Stasyshyn O, Alvarez-Román MT, et al. Extended half-life pegylated, full-length recombinant factor VIII for prophylaxis in children with severe haemophilia A. *Haemophilia*. 2016 Nov 27. doi: 10.1111/hae.13119 [Epub ahead of print]. **3.** Data on file.

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ADYNOVATE
[Antihemophilic Factor (Recombinant), PEGylated]

Patient 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 that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. 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® [Antihemophilic Factor (Recombinant)]

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.

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.

How should I use ADYNOVATE? (cont'd)

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.shirecontent.com/PI/PDFs/ADYNOVATE_USA_ENG.pdf 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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Shire

Now Approved

A ONCE-WEEKLY SUBCUTANEOUS (GIVEN UNDER THE SKIN) INJECTION FOR PEOPLE WITH HEMOPHILIA A WITH FACTOR VIII INHIBITORS

We extend our appreciation to the individuals, families, and healthcare providers who participated in the clinical trials that led to the approval of HEMLIBRA®. We thank you and celebrate with the community who made it a reality.

Discover **HEMLIBRA.com**

WHAT IS HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children with hemophilia A with factor VIII inhibitors.

WHAT IS THE MOST IMPORTANT INFORMATION I SHOULD KNOW ABOUT HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Discontinue prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with aPCC (FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the signs and symptoms of TMA during or after treatment with HEMLIBRA.
- **Blood clots (thrombotic events).** Blood clots may form in blood vessels in your arm, leg, lung or head. Get medical help right away if you have any of the signs or symptoms of blood clots during or after treatment with HEMLIBRA.

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

HEMLIBRA™
emicizumab-kxwh
injection for subcutaneous use
150 mg/mL

HOW SHOULD I USE HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

WHAT ARE THE OTHER POSSIBLE SIDE EFFECTS OF HEMLIBRA?

The most common side effects of HEMLIBRA include: redness, tenderness, warmth, or itching at the site of injection; headache; and joint pain. These are not all of the possible side effects of HEMLIBRA.

You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at (888) 835-2555.

Please see Brief Summary of Medication Guide on the following page for more important safety information, including **Serious Side Effects**.

Medication Guide Brief Summary
HEMLIBRA® (hem-lee-bruh)
(emicizumab-kxwh)
injection, for subcutaneous use

WHAT IS THE MOST IMPORTANT INFORMATION I SHOULD KNOW ABOUT HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Discontinue prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with aPCC (FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - confusion
  - weakness
  - swelling of arms and legs
  - yellowing of skin and eyes
  - stomach (abdomen) or back pain
  - nausea or vomiting
  - feeling sick
  - decreased urination
- **Blood clots (thrombotic events).** Blood clots may form in blood vessels in your arm, leg, lung or head. Get medical help right away if you have any of these signs or symptoms of blood clots during or after treatment with HEMLIBRA:
  - swelling in arms or legs
  - pain or redness in your arms or legs
  - shortness of breath
  - chest pain or tightness
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

See "**What are the possible side effects of HEMLIBRA?**" for more information about side effects.

WHAT IS HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children with hemophilia A with factor VIII inhibitors.

- Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.
- HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

BEFORE USING HEMLIBRA, TELL YOUR HEALTHCARE PROVIDER ABOUT ALL OF YOUR MEDICAL CONDITIONS, INCLUDING IF YOU:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA.
- are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

HOW SHOULD I USE HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.
- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.
- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- If you miss a dose of HEMLIBRA on your scheduled day, you should give the dose as soon as you remember. You must give the missed dose before the next scheduled dosing day and then continue with your normal weekly dosing schedule. Do not double your dose to make up for a missed dose.
- HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

WHAT ARE THE POSSIBLE SIDE EFFECTS OF HEMLIBRA?

- See "**What is the most important information I should know about HEMLIBRA?**"

The most common side effects of HEMLIBRA include:
- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

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

HOW SHOULD I STORE HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
- Store HEMLIBRA in the original carton to protect the vials from light.
- Do not shake HEMLIBRA.
- If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator for more than 7 days at 86°F (30°C) or below.
- After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.
- Throw away (dispose of) any unused HEMLIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

GENERAL INFORMATION ABOUT THE SAFE AND EFFECTIVE USE OF HEMLIBRA.

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMLIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMLIBRA that is written for health professionals.

WHAT ARE THE INGREDIENTS IN HEMLIBRA?

Active ingredient: emicizumab

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

Manufactured by: Genentech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94080-4990
U.S. License No. 1048

For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration
Issued: 11/2017

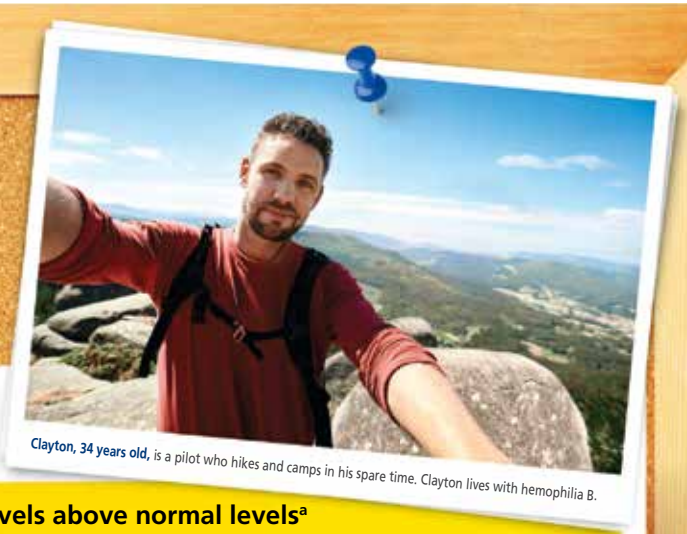
HEMLIBRA®
emicizumab-kxwh
injection for subcutaneous use
150 mg/mL

Genentech
A Member of the Roche Group

In hemophilia B

TAKE CONTROL TO A HIGH LEVEL WITH REBINYN®

NOW AVAILABLE



Clayton, 34 years old, is a pilot who hikes and camps in his spare time. Clayton lives with hemophilia B.

Rebinyn® elevates factor levels above normal levels[a]

+94% Factor IX (FIX) levels achieved immediately after an infusion[b]

17% FIX levels sustained after 7 days[a]

With a single dose of Rebinyn® 40 IU/kg in adults with ≤2% FIX levels[a]

[a]In two phase 3 studies, factor levels were evaluated for 1 week after the first dose of Rebinyn® 40 IU/kg. The average levels after 7 days were 16.8% in 6 adults, 14.6% in 3 adolescents, 10.9% in 13 children ages 7 to 12 years, and 8.4% in 12 children up to age 6 years.

[b]Based upon a 2.34% increase in factor levels per IU/kg infused in adults.

Image of hemophilia B patient shown is for illustrative purposes only.

INDICATIONS AND USAGE

What is Rebinyn® Coagulation Factor IX (Recombinant), GlycoPEGylated?

Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat and control bleeding in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for routine prophylaxis or for immune tolerance therapy.

IMPORTANT SAFETY INFORMATION

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

- **Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.** Carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing Rebinyn®.

Who should not use Rebinyn®?

Do not use Rebinyn® if you:

- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my health care provider before using Rebinyn®?

Tell your health care provider if you:

- have or have had any medical conditions.
- take any medicines, including non-prescription medicines and dietary supplements.
- are nursing, pregnant, or plan to become pregnant.
- have been told you have inhibitors to Factor IX.

How should I use Rebinyn®?

- Rebinyn® is given as an infusion into the vein.
- **Call your healthcare provider right away if your bleeding does not stop after taking Rebinyn®.**
- Do not stop using Rebinyn® without consulting your healthcare provider.

What are the possible side effects of Rebinyn®?

- **Common side effects include** swelling, pain, rash or redness at the location of the infusion, and itching.
- **Call your healthcare provider right away or get emergency treatment right away if you get any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- **Tell your healthcare provider about any side effect that bothers you or that does not go away.**
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

Please see Brief Summary of Prescribing Information on the following page.

Rebinyn® is a prescription medication.

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.

Learn more at rebinyn.com

novo nordisk®

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

rebinyn®
Coagulation Factor IX (Recombinant), GlycoPEGylated

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

Pfizer