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DATELINE FEDERATION

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Dear Friends,

Oh, what a year 2020 has been! As we reflect on the hardships and stress this year has brought, I'd encourage you to also find space for gratitude. I know for myself this year has brought uncertainty and chaos, but also time to slow down and enjoy the little things that I might have otherwise missed. Switching many community events to the virtual setting has been challenging, but I know HFA and our member organizations have found creative ways to still support and bring joy to our community and I'm so grateful for that!

This issue features results of a survey on women's perspectives. For me, as a woman with a bleeding disorder, it can be challenging and feel as if we are supposed to have all the answers. In such an uncertain world it can feel as if we are supposed to have the answers for our families and friends, and give support when we may not have much to give. It can also feel as if we are supposed to have all the knowledge and wisdom about our health, except sometimes there aren't many good options that fit your needs.

Know that HFA is here for you. Our programs aim to provide spaces of vulnerability and safety to unpack the hard questions and acknowledge that we do not have all the answers. And our advocacy aims to bring more awareness around issues with access and treatment that women face. Our patient-centered research aims to help bring knowledge and tools so that women can be better supported.

We're here for all of you during this challenging time. Here's to 2020 and hoping for a better 2021!

Allie Ritcey

Board Chair

Hemophilia Federation of America™

Assisting, educating, and advocating for the bleeding disorders community since 1994.

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Connect with us on social media for daily posts and updates about what's happening at HFA.

ARTICLES

SPECIAL FEATURES

ON THE COVER.
Hemophilia Federation of America surveyed Blood Sisterhood and Families program participants, like Britnee Vieira who has vWD, to better understand knowledge and awareness of bleeding disorders in women. We break down the results of the survey and what we discovered. **Read more on page 20.**

The Fun of the Summer Camp Experience Found Online

While bleeding disorders families across the country may have missed the summer ritual of camp due to pandemic restrictions, Hemophilia Federation of America partnered with Beyond Recreation on a three-part webinar series, A Taste of Camp. The virtual camp experience features songs, skits, campfires and fun right at home. Camp directors, camp staff and former campers share ways to prepare children to get the most out of the camp experience.

Find the series in the A Taste of Camp series playlist at **www.youtube.com/HemophiliaFederationofAmerica**.

Learning to Parent in a Whole New World

An unexpected side effect of the pandemic has been struggles (and wins!) in parenting. A Whole New World: Parenting in a Pandemic is a three-part webinar series featuring Dr. Juliana Bloom, a licensed psychologist and pediatric neuropsychologist. In these webinars, Dr. Bloom discusses parenting during a pandemic with topics specific to age, whether newborns to preschoolers, school-aged children or adolescents, teens and young adults. Dr. Bloom also shares valuable resources for parents to access.

Find the series in the Whole New World playlist at **www.youtube.com/HemophiliaFederationofAmerica.**

Sangre Latina Program Hosts Monthly Talks for Spanish-Speaking Community

Once a month Hemophilia Federation of American will host a virtual chat to help the Spanish-speaking bleeding disorders community stay connected during a health emergency. The informal conversations allow attendees to discuss difficult situation many are experiencing. Attendees are encouraged to share stories and techniques on how to deal with the situation.

Register for the monthly chat at **bit.ly/HFAHablandoContigo**.

HAPPENING **HIGHLIGHTS**

WFH
WORLD FEDERATION OF HEMOPHILIA
FÉDÉRATION MONDIALE DE L'HÉMOPHILIE
FEDERACIÓN MUNDIAL DE HEMOFILIA

World Federation of Hemophilia Publishes Third Edition of Guidelines

The third edition of the World Federation of Hemophilia Guidelines for the Management of Hemophilia features up-to-date guidance and practical recommendations on the diagnosis and management of hemophilia, including the management of musculoskeletal complications and inhibitors, updates to laboratory diagnosis and genetic assessments and new recommendations on outcome assessments.

Led by Dr. Alok Srivastava and Dr. Glenn Pierce Ph.D., WFH Vice President-Medical, WFH says the guidelines come from evidence and is supplemented with expert opinion and patient preference, including more than 300 practical recommendations developed by consensus using the "Trustworthy Consensus-Based Statement" process to ensure reliable guidance. The guide is meant to bridge the gap between treatment practices and care around the world.

Download the third edition of the WFH Guidelines for the Management of Hemophilia at **bit.ly/WFH3rdGuidelines**.

Natural Disasters Do Not Discriminate

Hemophilia Federation of America has established the Disaster Relief Fund for community members displaced or impacted by a natural disaster and in need of immediate financial relief. Dealing with wildfires, earthquakes or other natural disasters is challenging for anyone but when someone has a bleeding disorder, it presents an entirely more complicated scenario. Our community has a long history of rallying around families in need and this fund, originally founded in 2017 as a response to the nation's unprecedented number of national disasters taking place, expands upon our legacy of helping bleeding disorders community members experiencing financial hardship during a time of crisis.

Learn more, apply or make a donation to the fund at **www.hemophiliafed.org/disasterrelief**.

HFA SCHOLARSIHP *winners announced*

Each year, Hemophilia Federation of America awards scholarships to promising students in the bleeding disorders community seeking post-secondary education at a college, university or trade school. **We are pleased to award scholarships to the following students.**

OLIVIA **HOFF**

Attending Saint Mary's College in Notre Dame, Indiana

WINNER OF THE MEDICAL/HEALTH SCIENCES SCHOLARSHIP OF $4,000

I am very appreciative to be selected as a scholarship recipient from the Hemophilia Federation of America. The scholarship will allow me to complete my last year of schooling at Saint Mary's College. I will receive a Bachelor of Science. I am majoring in biology with a minor in chemistry. After graduation I plan to attend medical school in hopes to become a pediatric hematologist. Living with a bleeding disorder and seeing the positive impact my own hematologist has made on my journey, has sparked my long-term career choice. I am very appreciative of accepting this scholarship!

OLIVIA **WALDENBURG**

Attending Spokane Falls Community College in Spokane, Wash.

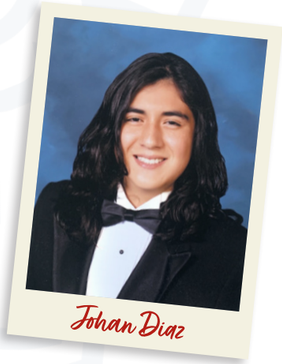
WINNER OF HFA EDUCATIONAL SCHOLARSHIP OF $2,000

I am so excited and grateful to be receiving this scholarship from the Hemophilia Federation of America. It means a lot to be receiving a scholarship that hits so close to home. The support of the HFA has allowed me to attend the Spokane Falls Community College and transfer to the University of Montana during the winter semester with a major in business management. My goal is to become a business owner and help other people just as people have helped me through my struggles and challenges. My blood condition has helped shape me into the person I am today and am excited to see where the future takes me. I feel so grateful to be included in this organization.

JOHAN **DIAZ**

Attending University of Southern California in Los Angeles

WINNER OF HFA EDUCATIONAL SCHOLARSHIP OF $2,000



It is such an honor to be a recipient of the Hemophilia Federation of America scholarship. The funds from this award will be directly sent to my school, the University of Southern California. Being selected to be awarded this scholarship not only helps support me financially, but also mentally. Funding my education will be one less thing to worry about because of this award. I will be majoring in biomedical engineering with a pre-med emphasis. I chose this field of engineering not only to focus on preparing myself for medical school, but to be able to earn a degree that could secure me a stable job, given that medical school turns out to not be right for me. This field of engineering still helps many people with medical issues, by providing prosthetics and devices for different parts of the human body. I always dreamt of helping people with medical complications because I always looked up to the medical professionals that took care of me. My education has always been my number one priority because I could never have a career in athletics, due to my blood disorder. Yet, my hemophilia has been a blessing in disguise because it has led me to graduate high school with honors and got me into one of the best private schools in the nation.

LAUREN **SURBROOK**

Attending University of North Carolina Wilmington in Wilmington, N.C.

WINNER OF THE PARENT/SIBLING/CHILD EDUCATIONAL SCHOLARSHIP OF $2,000

I am so grateful to be a recipient of a scholarship from the Hemophilia Federation of America. This scholarship is going towards my tuition at the University of North Carolina at Wilmington. I will be finishing my bachelor's degree in Business Administration with a concentration in Accountancy in May of 2021. I plan to continue my education with a master's degree and become a CPA. In high school, I was not sure what to expect from the future. I had been hospitalized for a blood disorder condition that my family was unaware that ran in the family. Even though it was slow and took some time, I was so lucky to have an amazing health team to help me recover. Even though it was a painful experience, I am thankful that it happened. We learned that both of my bothers also had the same blood disorder condition. I have come so far since that time in my life. With this opportunity that HFA has given me, it will grant me the chance to continue to make strides towards a brighter future.

Congratulations!

MAKING RESEARCH ACCESSIBLE TO ALL

Access to research allows researchers and the bleeding disorders community to better understand each other

The Research Team at Hemophilia Federation of America would like to thank all the wonderful presenters who chose to share their research during HFA's Annual Symposium Poster Session at the 2020 virtual Symposium in August, as well as all those who visited the first-ever virtual poster session, voting for their favorite posters. Here are some words from our People's Choice winners about why they do the work they do:

FIRST PLACE

Gender Differences in Parenting Stress and Social Support Among Hemophilia Families

Presented by Carletha Gates, PH.D., HS-BCP

"I am honored to once again be awarded the People's Choice award for HFA's annual symposium research poster session. It means a lot to be recognized for the work I hold so near and dear to my heart. I am also elated that HFA has begun to expand their research efforts, giving a platform for researchers to expose the bleeding disorders community to the many studies that exist that are of benefit for our community, particularly those in the psychosocial discipline. My research study on parenting stress and social support was a true labor of love. As a hemophilia mother of four affected children, I know from first-hand experience that parenting stress and having appropriate support is a real concern for parents as they face the many challenges of raising a child with hemophilia. Hemophilia not only concerns the affected child, but the entire family unit, particularly the parents.

The main goal of my research was to raise awareness of gender differences related to hemophilia patients' parental stress and how they can better utilize social support networks as they raise their chronically ill children. Understanding the link among gender, stress, and perceptions of social support is important to help parents develop coping strategies to meet the unique challenges of caring for their children with hemophilia.

My research concluded that all hemophilia parents experience some level of stress and there is a need for gender-specific social support regardless of the child's hemophilia severity. There is also a need for more in-depth dialogue about the seriousness of parenting stress. Parents need to be engaged in programs and services that can help decrease their stress levels and increase social support use to improve the health, wellness, and overall quality of life of the hemophilia family."

esperoct®

antihemophilic factor (recombinant), glycopegylated-exei

Brief Summary information about ESPEROCT® [antihemophilic Factor (recombinant), glycopegylated-exei]

This information is not comprehensive.

- Talk to your healthcare provider or pharmacist
- Visit www.novo-pi.com/esperoct.pdf to obtain FDA-approved product labeling
- Call 1-800-727-6500

Patient Information

ESPEROCT®
[antihemophilic factor (recombinant), glycopegylated-exei]

Read the Patient Information and the Instructions For Use that come with ESPEROCT® before you start taking this medicine and each time you get a refill. There may be new information.

This Patient Information does not take the place of talking with your healthcare provider about your medical condition or treatment. If you have questions about ESPEROCT® after reading this information, ask your healthcare provider.

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

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

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

What is ESPEROCT®?

ESPEROCT® is an injectable medicine used to replace clotting Factor VIII that is missing in patients with hemophilia A. Hemophilia A is an inherited bleeding disorder in all age groups that prevents blood from clotting normally.

ESPEROCT® is used to treat and prevent or reduce the number of bleeding episodes in people with hemophilia A.

Your healthcare provider may give you ESPEROCT® when you have surgery.

Who should not use ESPEROCT®?

You should not use ESPEROCT® if you

- are allergic to Factor VIII or any of the other ingredients of ESPEROCT®
- if you are allergic to hamster proteins

If you are not sure, talk to your healthcare provider before using this medicine.

Tell your healthcare provider if you are pregnant or nursing because ESPEROCT® might not be right for you.

What should I tell my healthcare provider before I use ESPEROCT®?

You should tell your healthcare provider if you:

- Have or have had any medical conditions.
- Take any medicines, including non-prescription medicines and dietary supplements.
- Are nursing.
- Are pregnant or planning to become pregnant.
- Have been told that you have inhibitors to Factor VIII.

How should I use ESPEROCT®?

Treatment with ESPEROCT® should be started by a healthcare provider who is experienced in the care of patients with hemophilia A.

ESPEROCT® is given as an infusion into the vein.

You may infuse ESPEROCT® 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 hemophilia treatment center or healthcare provider. Many people with hemophilia A learn to infuse the medicine by themselves or with the help of a family member.

Your healthcare provider will tell you how much ESPEROCT® to use based on your weight, the severity of your hemophilia A, and where you are bleeding. Your dose will be calculated in international units, IU.

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

If your bleeding is not adequately controlled, it could be due to the development of Factor VIII inhibitors. This should be checked by your healthcare provider. You might need a higher dose of ESPEROCT® or even a different product to control bleeding. Do not increase the total dose of ESPEROCT® to control your bleeding without consulting your healthcare provider.

Use in children

ESPEROCT® can be used in children. Your healthcare provider will decide the dose of ESPEROCT® you will receive.

If you forget to use ESPEROCT®

If you forget a dose, infuse the missed dose when you discover the mistake. Do not infuse a double dose to make up for a forgotten dose. Proceed with the next infusions as scheduled and continue as advised by your healthcare provider.

If you stop using ESPEROCT®

Do not stop using ESPEROCT® without consulting your healthcare provider.

If you have any further questions on the use of this product, ask your healthcare provider.

What if I take too much ESPEROCT®?

Always take ESPEROCT® exactly as your healthcare provider has told you. You should check with your healthcare provider if you are not sure. If you infuse more ESPEROCT® than recommended, tell your healthcare provider as soon as possible.

What are the possible side effects of ESPEROCT®?

Common Side Effects Include:

- rash or itching
- swelling, pain, rash or redness at the location of infusion

Other Possible Side Effects:

You could have an allergic reaction to coagulation Factor VIII products. **Call your healthcare provider right away or get emergency treatment right away if you get any signs of an allergic reaction, such as:** hives, chest tightness, wheezing, dizziness, difficulty breathing, and/or swelling of the face.

Your body can also make antibodies called "inhibitors" against ESPEROCT®, which may stop ESPEROCT® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

These are not all of the possible side effects from ESPEROCT®. Ask your healthcare provider for more information. You are encouraged to report side effects to FDA at 1-800-FDA-1088.

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

What are the ESPEROCT® dosage strengths?

ESPEROCT® comes in five different dosage strengths. The actual number of international units (IU) of Factor VIII in the vial will be imprinted on the label and on the box. The five different strengths are as follows:

Cap Color Indicator	Nominal Strength
Red	500 IU per vial
Green	1000 IU per vial
Gray	1500 IU per vial
Yellow	2000 IU per vial
Black	3000 IU per vial

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

How should I store ESPEROCT®?

Prior to Reconstitution (mixing the dry powder in the vial with the diluent):

Protect from light. Do not freeze ESPEROCT®.

ESPEROCT® can be stored in refrigeration at 36°F to 46°F (2°C to 8°C) for up to 30 months until the expiration date stated on the label. During the 30 month shelf life, ESPEROCT® may be kept at room temperature (not to exceed 86°F/30°C) for up to 12 months, **or** up to 104°F (40°C) for no longer than 3 months.

If you choose to store ESPEROCT® at room temperature:

- Record the date when the product was removed from the refrigerator.
- Do not return the product to the refrigerator.
- Do not use after 12 months if stored up to 86°F (30°C) **or** after 3 months if stored up to 104°F (40°C) **or** the expiration date listed on the vial, whichever is earlier.

Do not use this medicine after the expiration date which is on the outer carton and the vial. The expiration date refers to the last day of that month.

After Reconstitution:

The reconstituted (the final product once the powder is mixed with the diluent) ESPEROCT® should appear clear and colorless without visible particles.

The reconstituted ESPEROCT® should be used immediately.

If you cannot use the reconstituted ESPEROCT® immediately, it must be used within 4 hours when stored at or below 86ºF (30°C) or within 24 hours when stored in a refrigerator at 36°F to 46°F (2°C to 8°C). Store the reconstituted product in the vial.

Keep this medicine out of the sight and out of reach of children.

What else should I know about ESPEROCT® and hemophilia A?

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

Revised: 10/2019

ESPEROCT® is a trademark of Novo Nordisk Health Care AG.

For Patent Information, refer to: http://novonordisk-us.com/patients/products/product-patents.html

More detailed information is available upon request. Available by prescription only.

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US19ESP00168 December 2019

novo nordisk®

SECOND AND THIRD PLACE

Pharmacovigilance of Von Willebrand Factor/Coagulation FVIII Complex (Human)- 10 Years' Experience

Decreased Inhibitor Formation in Previously Untreated Patients with Severe Hemophilia A Exposed to a Fourth Generation rFVIII Product Derived from a Human Cell Line – Simoctocog Alfa, NUWIQ,

Both presented by Claudio Sandoval, M.D.

Prior to joining Octapharma I was an academic pediatric hematologist-oncologist. My first encounter with patients with hemophilia was during medical school in 1985 as a third-year student. At that time the most feared complication of hemophilia therapy was infection with hepatitis B and HIV. Today, the most feared complication is inhibitor formation, which affects approximately 30 to 40% of patients. It was an Octapharma innovation that made plasma-derived proteins safe for use in humans. Octapharma scientists developed a recombinant factor 8 product derived from human cells to reduce the frequency of inhibitor formation in previously untreated patients. It was my pleasure to present our data, which showed that our product, NUWIQ, reduced the frequency of high-titer inhibitor formation. It is paramount to educate patients and families about the attributes of our product so they can make informed decisions on their therapy.

Continuing the theme of patient safety, our product Wilate was produced for patients with von Willebrand disease. Wilate's formulation and pharmacologic properties mimic those of natural, endogenous von Willebrand factor. More important, our product is safe and effective, and can be used for patients with both von Willebrand disease and factor 8 deficiency.

FOURTH PLACE

Cell Therapy in Hemophilia: What the Future Holds

Presented by Jelena Garafalo, Ph.D.

I am currently senior director in the Medical Affairs Department of Sigilon Therapeutics. I am a scientist by training, and I always like to learn and understand how things work. I did this research to educate myself on cell therapies and what their place is in the context of existing therapies, especially when thinking of novel therapies. Sigilon Therapeutics is using an innovative approach to cell therapy, where the engineered cells are shielded by an alginate-based sphere which nurtures the cells and protects them from the host's immune system. **Unshielded cells are seen as foreign by the host's immune system and destroyed, which is why it is important to protect them. This is one of the greatest challenges of cell therapy and if we are successful in the clinic, we have the potential to apply this technology across many chronic diseases.**

We are already looking forward to 2021! To continue to make research more accessible to patients and caregivers, HFA invites researchers to disseminate their findings at Symposium 2021 in San Antonio in an atmosphere that encourages questions and dialogue. **For information on presenting a poster and to be notified when the call for abstracts is posted, email research@hemophiliafed.org.**

To view all the posters and their abstracts, visit **www.hemophiliafed.org/research**.

THE RISK OF DEVELOPING A COSTLY MEDICAL COMPLICATION

HFA develops an inhibitor education initiative to address findings of a survey of patient and caregiver awareness of inhibitors

DEBRAN TARVER, PH.D., STAFF WRITER

People with hemophilia or von Willebrand disease type three, who treat with clotting factor concentrates, are at risk for developing an inhibitor. Inhibitor development makes it more difficult to treat bleeds. It is a serious and costly medical complication.

Hemophilia Federation of America has an opportunity for the bleeding disorders community to learn more about inhibitor development, testing and treatment through our free, online learning platform, Learning Central. The following provides a brief overview of how the inhibitor courses were developed and what participants can expect to see during their Learning Central experience.

WHAT WE LEARNED

Based on findings from a survey of men enrolled in Blood Brotherhood and caregivers in the Families Programs, HFA concluded that additional education was needed for individuals to be able to be able to identify if they or their child are at risk for an inhibitor and if so to:

- Correctly identify the inhibitor testing method name
- Correctly identify all the risk factors for inhibitor development
- Promote individuals at risk to get tested for an inhibitor
- Encourage provider-patient conversations surrounding inhibitor development and testing

As a result, HFA designed the inhibitors courses in Learning Central to increase knowledge and awareness about the signs, symptoms, risk factors and testing for inhibitors for people at risk.

LEARNING CENTRAL DEVELOPMENT

The primary initiative was to develop and launch an educational course including the following topics:

- Inhibitor Basics
- Risk Factors for Developing an Inhibitor
- Signs and Symptoms of Inhibitors
- Testing and Diagnosis of Inhibitors
- Treatment of Inhibitors
- Quality of Life with Inhibitors

HFA partnered with advisors from the Centers for Disease Control and Prevention, a learning technologies firm, a hematologist and a hematologist nurse in the bleeding disorders community to shape content into a digitally interactive and memorable learning experience.

Each topic includes the following:

- **Guide:** A member of the HFA Community who guides the learner through clinical content, so the learner feels as though they are learning from a friend.
- **Show what you know:** An opportunity for participants to assess their knowledge at the beginning of each topic.
- **Content:** Information addressing the topic (e.g., treatment of inhibitors). With guidance from our partner learning designers, hematologists, and CDC. Our goal was to display factual information in clear, and interactive ways.
- **Knowledge Check:** An opportunity for participants to assess their knowledge at the end of each topic.
- **Summary:** A brief description of what was discussed and available resources for more information.

The courses also include a series of videos highlighting community members' experiences with inhibitor signs and symptoms, testing, diagnosis, treatment and quality of life. These videos were developed with the intention of increasing awareness and knowledge of inhibitors from the direct voices of community members who have experienced inhibitors first-hand. The videos address the challenges of living with inhibitors and the importance of being knowledgeable about the content provided in Learning Central. Being knowledgeable about inhibitors allowed these community members to be able to advocate for their, or their family member's, health and get the needed support from providers.

A LOOK INTO LEARNING DEVELOPMENT

An example of content presented in the courses is an examination of myths related to inhibitors. Some of the myths dispelled are:

MYTH: You can only develop an inhibitor as a baby

FACT:
An inhibitor typically occurs within the first 30 to 50 exposures to factor treatment, so those who are severe or bleed often tend to get diagnosed very young. However, for those who infuse less frequently, it can happen much later in life.

MYTH: Only those with severe hemophilia will develop an inhibitor

FACT:
While inhibitors develop more often among those with severe hemophilia, anyone who infuses clotting factor can develop inhibitors, even those with mild or moderate hemophilia.

MYTH: Product switching causes inhibitors

FACT:
Switching from one clotting factor product to another does not cause or increase your risk of developing an inhibitor.

Below are quotes from the videos of community members discussing their challenges with inhibitors.

INHIBITOR KNOWLEDGE

I didn't know too much about inhibitors at that time. I was told that it was possible that he may get an inhibitor at some time because of my brother (who has an inhibitor)—the family history. It was scary.

— Veronica, mom of son with hemophilia and tolerized inhibitor

INHIBITOR TREATMENT

A setback for the bypassing agent we would use is that the life span (half-life) of it is only two hours and regardless of how much you stack it up, if you combine and give him two doses to what the physician would consider 100%, he's still only getting coverage for two hours, not an extended 6 hours. You can't extend it when its (half) life is only two hours. So, we're giving him more, but it's only lasting two hours. So, if he did have bleeds, we would be infusing every two hours. It puts time constraints on social activities, time constraints on getting him to school, getting to work, practically having a life outside of our infusion schedule.

— Marquita, mom of son with hemophilia and inhibitors

SIGNS SYMPTOMS TESTING + DIAGNOSIS

There was a year during his second-grade year that he was bound to a wheelchair because of his joint issues—a bleed that just didn't seem to ever get resolved in his knee. And so he was out of school more than he was in school that year and it proved to be very difficult for him and the entire family. But the inhibitor truly did get some of the best of my son and our family at times in those early years when he was in second and third grade."

— Cazandra, mom of son with hemophilia and inhibitors

QUALITY OF LIFE

My inhibitor has certainly impacted my work. I found a job where I can work from home which I think for me is essential... Even as someone that works from home, there are days when I can't even do that because of a bleed and so I use a lot of sick time and I have to write a lot of emails to my co-workers saying I'm really sorry, but I won't be able to get to this today and maybe not tomorrow either because I have a really bad bleed going on....It's the single biggest influence on my work life."

— Michael, adult male with hemophilia and inhibitors

Visit Learning Central at www.hfalearning.org to learn more about inhibitors and hear directly from the experience of community members.

While these courses are designed for people diagnosed with hemophilia A or B or vWD Type 3 who have infused with a clotting factor treatment product and their parents/ caregivers, the courses are open to anyone who wants to learn more.

This work was supported by Cooperative Agreement Number, NU27DD001151-05, funded by the Centers for Disease Control and Prevention. Its contents are solely the responsibility of the authors and do not necessarily represent the official views of the Centers for Disease Control and Prevention or the Department of Health and Human Services.

ADVATE
[Antihemophilic Factor(Recombinant)]

Important facts about

ADVATE [Antihemophilic Factor (Recombinant)]

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

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

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 ADVATE so that your treatment will work best for you.

What is ADVATE?

ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia). The product does not contain plasma or albumin. Hemophilia A is an inherited bleeding disorder that prevents blood from clotting normally.

ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A.

Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

Who should not use ADVATE?

You should not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

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

How should I use ADVATE?

ADVATE is given directly into the bloodstream.

You may infuse ADVATE 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 ADVATE by themselves or with the help of a family member.

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

You may have to have blood tests done after getting ADVATE 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 ADVATE.

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

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

What are the possible side effects of ADVATE?

You can have an allergic reaction to ADVATE.

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.

Side effects that have been reported with ADVATE include:

cough	headache	joint swelling/aching
sore throat	fever	itching
unusual taste	dizziness	hematoma
abdominal pain	hot flashes	swelling of legs
diarrhea	chills	runny nose/congestion
nausea/vomiting	sweating	rash

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 ADVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADVATE 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 ADVATE 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 ADVATE for a condition for which it is not prescribed. Do not share ADVATE 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 ADVATE. The FDA-approved product labeling can be found at www.ADVATE.com or 1-877-825-3327.

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.

Patented: see https://www.takeda.com/en-us/patents/

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ENDING THE CYCLE OF FINANCIAL STRUGGLE

New program provides a way for community members who often struggle financially to take steps to get ahead, stay ahead

BY ALLISON HARRISON, M.S.W.,
CERTIFIED FINANCIAL SOCIAL WORKER, STAFF WRITER

As a member of the Helping Hands team, I speak every day to members of the bleeding disorder community who are stuck in a cycle of financial insecurity. Hemophilia Federation of America's Helping Hands team, staffed by social workers, connects with community members in need, delivers financial assistance and educates applicants about support that may strengthen their ability to succeed while living with a bleeding disorder.

While members of the bleeding disorders community are some of the most resilient and resourceful individuals, they also face a set of formidable challenges, such as high expenses due to medical appointments, procedures, medications, transportation to appointments, and items that are not covered by insurance like wheelchairs and protective gear. Many have faced "surprise billing" issues when, in an emergency, they received care not covered by their insurance provider.

Employment

Some community members face challenges with income and financial stability. Because of physical needs or caregiving duties, people in the community may have a hard time finding work flexible enough to accommodate their physical needs. A bleeding disorder may also make it difficult for caregivers and patients to complete higher education that could open doors to higher wages.

We have seen many community members who are under-employed (hours or pay are too low to meet financial needs), find it hard to keep a job, find low-wage hourly work the only option, are overlooked for promotions or raises, or regularly miss out on wages due to caregiving or medical needs.

In HFA's CHOICE Survey, a survey conducted by HFA several years ago to ask patients about their experiences, respondents with hemophilia A reported missing an average of 12 days of work or school because of their bleeding disorder. Respondents living with hemophilia A with inhibitors missed an average of 23 days. The American Journal of Hematology published a study in 2015 stating nearly 25 percent of patients with bleeding disorders ages 25 to 34 were unemployed compared to 6 percent in the general population in the same year.

Family Impact

Bleeding disorders like hemophilia and vWD are usually hereditary, putting

community members at risk of facing the intergenerational impacts of both trauma and financial insecurity. Families with bleeding disorders have faced traumatic experiences, including long and painful bleeding episodes when treatments weren't as effective, permanent complications, negative interactions with medical institutions, bankruptcy and debt caused by medical bills and insurance restrictions, the contamination of the blood supply, and stigma from neighbors, schools and employers.

While treatments have advanced and individuals with bleeding disorders are able to live long and productive lives, living with a chronic disorder still produces emotional, social medical, and financial challenges.

Some Helping Hands applicants face a short-term emergency, like insufficient savings to cushion the blow of temporarily lost income or an unexpected expense. With financial assistance, they soon get back on their feet. Other applicants face a financial emergency but are also in a cycle of debt or poverty due to employment challenges, medical/insurance setbacks, debt and credit problems, or any number of issues outlined above.

Since its creation in 1997, Helping Hands has continuously evolved to meet the needs of our most vulnerable community members. The program started by providing bleeding disorder families with funds offering a brief respite from the daily grind of chronic illness. Over the years, Helping Hands has furnished thousands of families with assistance with for urgent basic living expenses, medically necessary items, fitness support, medical travel and tutoring support. The program has also provided financial support to the bleeding disorders community affected by natural disasters such as hurricanes and floods through the Disaster Relief Fund help to those impacted by the pandemic. But there is more to do.

Plan to End the Cycle of Financial Struggle

Our community members are experiencing physical, financial and mental stress on a daily basis. Many find themselves maxing out physically and emotionally with little left to field daily phone calls from bill collectors, re-apply for public benefit programs, research financial assistance programs, search for a job or job training, or strategize for financial sustainability.

The Helping Hands team proposed to—in addition to lending a "helping hand" to community members in crisis—develop a program to address the root causes of financial insecurity applicants face year after year, leading to the creation of the Helping Forward program, a program to empower community members to move forward from crisis into a sustainable future through career planning and financial management.

The current focus of Helping Forward is education, including:

> The Helping Forward webpage includes a robust set of resources to help community members understand their rights, options and resources related to employment and finances.

> The Employment Rights and Self-Advocacy courses on HFA's Learning Central featuring videos, quotes and stories from 18 different community members. Lessons include an introduction to the topic of employment for people with bleeding disorders, the Americans with Disabilities Act (ADA), the Family and Medical Leave Act (FMLA) and tips for self-advocacy in the workplace.

Visit the website to sign up for the Helping Forward Network so you can learn and grow with the program!

"In my previous job, the company did not allow me to grow, and I feel it's because they never understood what I needed. My mental health was greatly affected....I wanted to stay and was fearful of losing my job, but at the same time my absences were unpaid, and it had a huge impact to my budget. There were times I struggled to buy groceries and to pay bills. In that job I learned to make sure I could have a secure income while I was dealing with my personal and health problems."

– Fabiola, HFA Community Member

NEED HELP NOW OR MOVING FORWARD?

Helping Forward:
www.hemophiliafed.org/HelpingForward

Helping Hands:
www.hemophiliafed.org/helpinghands

Patient Assistance Portal:
www.hemophiliafed.org/patientassistanceportal

WHAT WE LEARNED AND WHERE WE NEED TO GO

BY JANET CHUPKA, R.N., BSN, STAFF WRITER

Approximately one percent or as many as two million women in the United States may have an undiagnosed bleeding disorder. Despite these women experiencing serious gynecologic/obstetric and other challenges related to bleeding throughout their lives, most bleeding disorders remain undiagnosed for years and some women are never diagnosed.

Hemophilia Federation of America reviewed the literature and studies showed an underrecognition of diagnosis and severity of bleeding among women because of a persisting belief among medical providers that only men have bleeding disorder. HFA held discussion groups with women diagnosed with a bleeding disorder and women reported feeling brushed off or told that their symptoms were "normal" when they tried to speak with their providers about bleeding disorder symptoms. Many women are not aware their symptoms are abnormal and do not seek medical advice.

Several years ago HFA developed a survey and collected information from women over the age of 18 who were participants in the Blood Sisterhood and Families program to better understand the knowledge and awareness of bleeding disorders in women and their experience (among HFA female participants).

The 86 women who were enrolled reported blood disorder diagnoses or symptoms of a blood disorder (including women who are carriers of bleeding disorders):

- 83% indicated they had a diagnosis for a bleeding disorder
- 15% indicated they were not diagnosed with a bleeding disorder
- 2% either were unsure or preferred not to answer

The disease groups represented in this survey were hemophilia A (22%), von Willebrand's disease (20%), carriers of hemophilia A (15%), platelet disorder (9%), hemophilia B (8%), carriers of hemophilia B (6%), factor V (5) deficiency (4%), factor V-VIII (5-8) deficiency (2%), and factor VII (7) deficiency (2%), while 12% of participants did not respond with a disease type.

The following highlights what program participants reported about female bleeding disorders and gaps in knowledge and awareness that are being addressed in upcoming HFA educational programming.

Knowledge and Awareness of Signs and Symptoms of Bleeding Disorders in Women

To learn more about participants' current knowledge of signs and symptoms of bleeding disorders in women, we assessed if respondents were able to identify the signs and symptoms of bleeding disorders in women, specifically heavy periods, a family history of bleeding, frequent nosebleeds, and bleeding after giving birth or a miscarriage. And whether they knew that frequent headaches are not a symptom of a bleeding disorder.

- More than half of the women (62%) correctly identified all of the symptoms of bleeding disorders in women. However, there appeared to be a gap in knowledge among the women 18-29 years old as the majority, 60%, answered incorrectly.
- We looked at individual symptoms and found almost all women, 93%, recognized heavy periods as a symptom of bleeding disorders in women, and 85% of the women correctly answered that the definition of menorrhagia is menstrual bleeding that lasts more than seven days.

Knowledge and Awareness of Diagnostic Testing

We examined how familiar women were with the clinical laboratory tests that are common during the diagnosis process.

- 33% of women without a BD diagnosis and 62% of women with a BD diagnosis knew that the Factor 8 Clotting Test measures the amount of factor 8 protein in the blood. There was increased awareness about the purpose of the von Willebrand Factor Antigen Test, as 50% of the women without a BD diagnosis and 79% of women with a BD diagnosis answered this question correctly (including 94% of women diagnosed with VWD).
- Women older than 29 years demonstrated more knowledge about laboratory tests for a BD diagnosis. Only 21% of this age group answered the question incorrectly compared to approximately 50% of the women between 18-29 years old who answered this question incorrectly. As older women from our sample were not more likely to have a diagnosis, it is likely there are other factors that influence the knowledge level about laboratory tests among older women.

Patient-Provider Relationships

In order to better understand the level of care women with a reported diagnosis for a bleeding disorder receive, we asked questions about whether they see a healthcare provider for their disorder, whether they have a treatment plan in place, and whether they talked about or received diagnostic testing during their most recent visit.

- When we asked about diagnostic testing, 42% of the women with symptoms of a BD or were carriers, but did not

BLOOD SISTERHOOD

A Summary of HFA's Blood Sisterhood Survey Data

7%
of diagnosed women do not routinely see a healthcare provider

62%
correctly identified all symptoms of bleeding disorders in women

Out of the last 30 days, eight were not good physically; nine were not good mentally; seven were so physically or mentally difficult that usual activities could not be completed

23%
of diagnosed women do not have a prescribed treatment plan in place

58%
"always" or "frequently" experience joint problems

69%
feel "extremely" or "very" confident in being a BD self-advocate

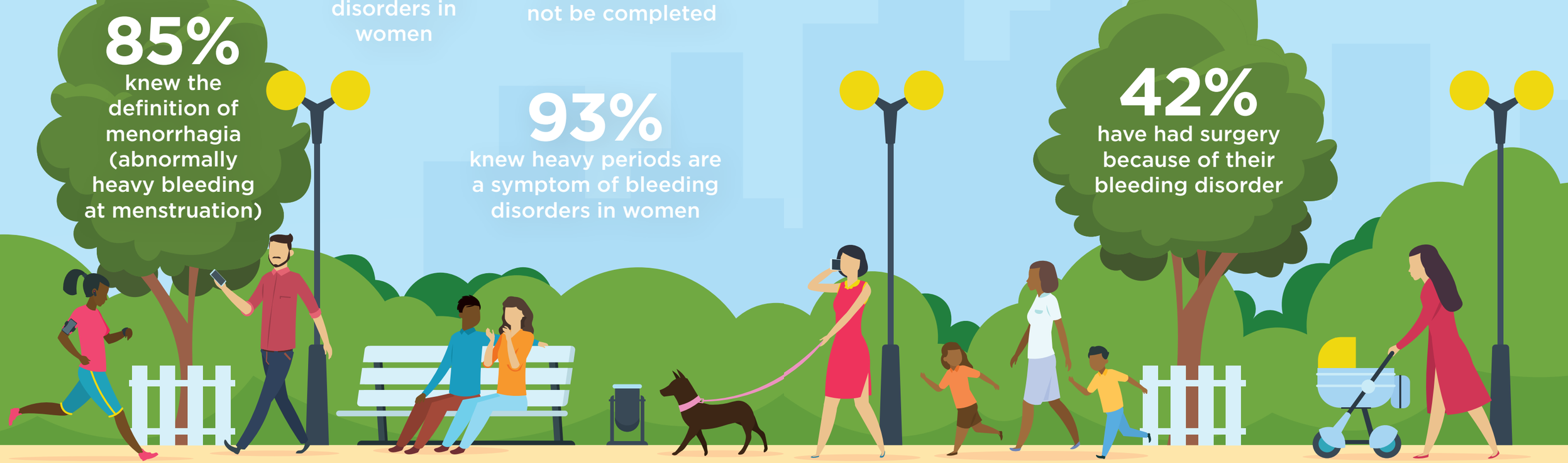
Average age at bleeding disorder diagnosis:
24

64%
feel their self-advocacy knowledge is "very high" or "above average"

85%
knew the definition of menorrhagia (abnormally heavy bleeding at menstruation)

93%
knew heavy periods are a symptom of bleeding disorders in women

42%
have had surgery because of their bleeding disorder



Hemdifferently

Exploring the science behind gene therapy research

Gene therapy research has the potential to bring an entirely new option to people with specific genetic conditions. Many gene therapies are in clinical trials to evaluate the possible risks and benefits for a range of conditions, including hemophilia. HemDifferently is here with gene therapy education, providing accurate information on the basics and beyond.

Want to stay in-the-know and receive the latest information to your inbox, including invitations to events?

Scan the QR code now or visit **HemDifferently.com** to sign up for updates.

No gene therapies for hemophilia have been approved for use or determined to be safe or effective.

BIOMARIN

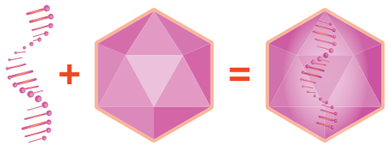
THE 5 STEPS OF INVESTIGATIONAL GENE TRANSFER

One method of gene therapy currently being explored in clinical trials is called gene transfer. This approach aims to introduce a working gene into the body to determine if it can produce a needed protein.

STEP 1

CREATING A WORKING GENE

A working, or functional, copy of a mutated gene is created in a laboratory.



STEP 2

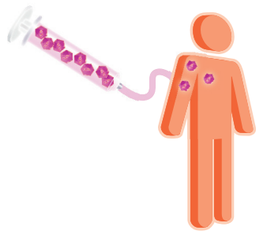
BUILDING A THERAPEUTIC VECTOR

To protect and deliver the working gene, scientists place it inside an empty viral shell.

STEP 3

DETERMINING ELIGIBILITY

As part of gene therapy research, eligibility requirements may be considered, including age, gender, and organ health. In addition, some patients may have immunity to the therapeutic vector, which could be determined by a blood test and could make them ineligible for a trial.



STEP 4

DELIVERING THE WORKING GENE

A single, one-time infusion in an appropriate clinical setting delivers large numbers of therapeutic vectors into the body.

Once in the body, the working gene is designed to provide instructions for the body to make the protein it needs on its own.

=Therapeutic vectors with working gene

STEP 5

MONITORING SAFETY AND EFFICACY

Clinical trial participants are regularly monitored to better understand the safety of the gene transfer and to evaluate its effect on the body, including whether it is creating the needed proteins.

=New proteins

report a diagnosis, indicated that they had talked about diagnostic testing with their provider at their last visit, and 17% of this group indicated that they received a diagnostic test at their last visit.

- Approximately 7% of the women who self-identified as having a diagnosed bleeding disorder said that they do not routinely see a healthcare provider, all of whom were under the age of 50. In addition, we saw differences by the geographic region where women lived, as about 20% of the women with a diagnosis from the Northeast or the West did not see a provider regularly, while all of diagnosed women from the South or the Midwest indicated regularly seeing a provider. In addition, 23% of the women with a diagnosed bleeding disorder do not have a prescribed treatment plan in place with their provider. Once again, women from the Northeast and West have less treatment plans in place (54% reported having a plan) than women from the South or Midwest (86% reported having a plan).

Medical History of Bleeding Disorder

For women who reported having a bleeding disorder diagnosis, we also asked questions about the women's age at diagnosis and medical interventions to better understand the impact their bleedings disorders have had on their medical history.

- The average age at diagnosis was 24 years, with the youngest being 0 years and the oldest being 55 years of age at diagnosis.
- When asked about surgery, 42% of the women surveyed indicated having had surgery as a result of their bleeding disorders. The percentage of women having had surgery generally increased with the age of the women.

Joint Health

With bleeding disorders, joint damage due to bleeding episodes in joints is the most common complication. We asked a series of questions to all the women (both those diagnosed and those still undiagnosed with a BD) about their joint health.

- Overall, 58% of the women indicated that they "always" or "frequently" experienced problems with their joints, such as pain, stiffness, loss of motion, or weakness. Among the women who chose these categories, 96% were over 29 years old.
- More than 75% of women with hemophilia B, VWD, and women who are carriers for hemophilia A reported "always" or "frequently" having joint problems.

We asked how often their joint problems, such as pain, stiffness, loss of motion, or weakness, limit their daily activities, such as going to work or school, doing recreational activities, or taking care of themselves.

- Approximately one in four said "frequently" and one in 10 said "always;" the largest proportions of women who answered were hemophilia A carriers (39%), women with hemophilia A (32%), women with hemophilia B (33%), women with platelet disorders (43%), and women with VWD (47%).

Age of Participants

More than half of the women surveyed were between 30 and 49 years of age.

Age	Number of Participants (%)
18-29 yrs.	10 (12.2%)
30-39 yrs.	26 (31.7%)
40-49 yrs.	20 (24.4%)
50-59 yrs.	15 (18.3%)
60+ yrs.	11 (13.4%)
Total	82

Quality of Life

To better understand the quality of life these women experience, we asked all the women (with and without a diagnosis) to estimate how many days out of the last 30 days they felt their pain made it hard for them to do their usual activities.

- About half (52%) of the women said they experienced limiting pain like this for either 0 or one to five of the last 30 days. However, 22% of the women indicated having limiting pain for more than two weeks during the last 30 days.

We also asked during the last 30 days, how many days they felt their physical health and they felt their mental health was not good.

- The average response was that physical health was not good for about eight of the last 30 days, with the average number of not-good-days increasing with age.
- On average, mental health was not good for about nine of the last 30 days, with hemophilia A carriers and women with hemophilia B reporting the highest average number of days when mental health was not good.
- The women estimated that poor mental or physical health prevented them from completing their usual activities for on average between six to seven days of the last 30 days, with the number of days again increasing with age.
- Interestingly, when asked about their health in general, most women felt that they were either "good" (47%) or "very good" (31%), although none of the women felt they were "excellent." In addition, we asked how often they felt overwhelmed by bleeding disorder concerns, to which 51% responded "never" or "hardly ever," and 34% responded "sometimes."

Finally, we asked a series of questions about interpersonal relationships to gauge how much social support these women have. Overall, responses to these questions indicated that most of these women have the relationships and social support they need.

Self-Advocacy and Sources of Bleeding Disorders Information

We asked questions to better understand how comfortable all of the women who participated in the survey were with advocating for themselves and where they received information about bleeding disorders.

- The women expressed, for the most part, that they were confident in being self-advocates for bleeding disorders, as 36% said they were "extremely confident"

Continued on Page 30

Region in which Participants Reside

The region of the United States where participants lived wasrepresented fairly equally between regions with slightly more women living in the south, Region 3, and slightly less women living in the west coast, Region 4.

Region**	Number of Participants (%)
Region 1 Northeast (CT, ME, MA, NH, RI, VT, NJ, NY, PA, PR)	21 (24.4%)
Region 2 Midwest (IL, IN, MI, OH, WI, IA, KS, MN, MO, NE, ND, SD)	21 (24.4%)
Region 3 South (DE, FL, GA, MD, NC, SC, VA, DC, WV, AL, KY, MS, TN, AR, LA, OK, TX)	27 (31.4%)
Region 4 West (AZ, CO, ID, MT, NV, NM, UT, WY, AK, CA, HI, OR, WA)	17 (19.8%)
Total	**86**

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GO AHEAD.

PEOPLE LIKE YOU. STORIES LIKE YOURS.

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Discover your sense of go. Discover HEMLIBRA®.

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, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMLIBRA may cause serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including thrombotic microangiopathy (TMA), and blood clots (thrombotic events). 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.

Please see Brief Summary of Medication Guide on following page for Important Safety Information, including **Serious Side Effects**.

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

Medication Guide
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. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (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, ages newborn and older, with hemophilia A with or without 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.
- **Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis.**
- **You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.**
- 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.
- You will receive HEMLIBRA 1 time a week for the first four weeks. Then you will receive a maintenance dose as prescribed by 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 as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. **Do not** give two doses on the same day 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 a total of 7 days or at a temperature greater than 86°F (30°C).
- 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-kxwh

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
Revised: 10/2018

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

Genentech
A Member of the Roche Group

and 33% said they were "very confident." When asked to rate their own knowledge and awareness on self-advocacy related issues, 34% felt their knowledge was "very high" and 30% felt they were "above average", with women older than 29 tending to rank their knowledge higher than women 18-29 years of age.

- The most popular sources of bleeding disorders information were state/local chapters and HFA. Among women who reported seeking information daily, 50% of the women used state/local chapters and 25% used HFA. Among women who reported seeking information weekly, 36% of women used HFA and 31% used state or local chapters. Among women who reported seeking information on a monthly basis, 37% used state/local chapters and 25% used HFA. Many women also reported getting information from NHF, as 34% of women seeking information on an annual basis indicating using NHF, followed by 30% using HFA and 21% using state/local chapters.

We also asked about which HFA specific activities the women participated in to get information about women and bleeding disorders. The majority of women indicated they used HFA's social media, with more than half (55%) saying they used it either "a great deal" or "a moderate amount." HFA's Newsletter, Dateline Federation, is also a commonly used source, as 57% of the women used that either "a great deal" or "a moderate amount." About 40% of the women said they used HFA's website "a great deal" or a moderate amount," and 33% saying they used it "occasionally." Many women also reported utilizing information gained during HFA's Symposium, as about 40% of the women said they used it "a great deal" or "a moderate amount;" however, there is still room for growth , as 26% of the women indicated that they "never" get their information from Symposium. Other HFA activities that were used less frequently as sources of information were HFA's Webinar, which 52% of women said they "never" or "rarely" used, and the Blood Sisterhood program, which 48% of women said they "never" or "rarely" used.

Finally, we asked the women a little more about their opinions on HFA's Blood Sisterhood Program related to social support.

- Overall, most women felt that it is "extremely helpful" (45%) or "very helpful" (29%) to be able to talk about issues related to their condition with other women with bleeding disorders.
- About four in 10 women indicated that the program has increased their communication with other women in the bleeding disorders community. Although six in 10 women did not feel this way (31%) or were unsure (29%).
- We also asked whether the Blood Sisterhood Program provides social support to those who participate, to which 48% of the women indicated "Yes," while 11% said "No" and 41% were unsure.

Conclusions

Our findings indicate that more education targeting younger women between the ages of 18 to 29 years old may be needed on knowledge topics such as signs and symptoms of bleeding disorders in women and the purposes of different diagnostic tests, as well as the need to routinely see a provider and have a prescribed treatment plan after diagnosis. More support for women 30 years and older dealing with joint problems and physically limiting pain is also warranted.

To support both diagnosed and undiagnosed women HFA created a new module on Learning Central that has information on bleeding disorders in women. Visit Learning Central at **www.hfalearning.org**. Topics covered include: Understanding Yourself, Signs and Symptoms of Bleeding Disorders in Women, Testing and Treatment Options, and Life Balance and Health Promotion.

In the future, HFA also plans to examine providers knowledge of bleeding disorders in women and if there may be gaps in knowledge among providers that HFA can address. HFA would like to express our appreciation and gratitude to the women who took the time to participate in the survey.

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INSPIRED

Singer-songwriter with hemophilia finds music an outlet for his creativity

BY EMILY A. ROUSH-BOBOLZ, STAFF WRITER

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We all have our favorite things — the things we go to for comfort and familiarity. It might be that cozy sweater on a snow day or rainy Sunday afternoon. Or that mug that fits our hands just right and seems to make the coffee taste better.

It might be a special person. The one we go to when we've had a bad day and just need a shoulder to cry on. The one we share all our hopes and dreams with. That was the inspiration for a song written by bleeding disorders community member and singer-songwriter Trevor Martin of Nashville.

"I really wanted to impress a girl," said Martin, 23, who was diagnosed with severe hemophilia A as an infant. At 13, Martin had his eyes on a girl, and began writing songs using love stories as inspiration. For one of his most recent song releases, "Go-To," the message is about a girl who knows what she likes. At the end of the day, he/the singer hopes he will be one of her go-tos.

The road from writing songs for a girl at 13 to releasing songs that are now on YouTube and music streaming services started when Martin attended an event for Tennessee Hemophilia and Bleeding Disorders Foundation while in college. During the THBDF event, Martin's family met music producer Adam B. Smith, who has a son with hemophilia. Smith is a successful producer who has worked with internationally-known music artists such as Andy Grammer and Colbie Caillat. Smith and his wife, Tara, invited Martin for dinner where he had the chance to meet Arlo, the Smith's son with hemophilia. Martin says it was factor night and the Smiths expressed the young boy had some fear.

"I showed him where my port was, and he showed me his. It was a huge moment to calm him down," said Martin. "I sat down with him and watched [his infusion] and he did it like a champ!"

Martin and the Smiths developed a hemophilia connection that year, but life and school got in the way and a musical connection would come later. After graduation from Belmont University, Smith and Martin reconnected and began working together regularly, writing more than 20 songs together. In May he released "Go-To" with Smith's help. Music has been an outlet for Martin who, despite loving sports, couldn't participate in contact sports due to hemophilia.

"It's a way to express myself without being too hard on joints," said Martin. "I want kids to know you can live a life of hemophilia that's fun and exciting!"

In addition to recently released singles, Martin has written theme songs for movies and has done some acting. He placed second in the 2018 Music for a Cause hemophilia-based songwriting competition and won first place in the 2019 competition for his song "Way Back Home." He continues to be active in the hemophilia community and is grateful for his connection with Adam Smith.

"Not only has the music aspect been really cool, but the hemophilia aspect has been cool. It's a great connection with Adam and Arlo," said Martin. "The hemophilia community has been great to me. There have been a lot of people who were inspirational to me and I hope to be an inspiration to someone too."

TREVOR'S RECENT SINGLES

"GO-TO"
Available on Spotify, Apple Music, Amazon Music
Written in collaboration with hemophilia dad, Adam B. Smith

"CALLING IT FRIENDS"
Available on Spotify, Apple Music, Amazon Music

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MUSIC VIDEOS ON YOUTUBE:
TREVOR MARTIN MUSIC

BeneFix®

Coagulation Factor IX (Recombinant)

Room Temperature Storage

℞ only

Brief Summary

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-438-1985.

Please read this Patient Information carefully before using BeneFix and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

What is BeneFix?

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Your doctor might also give you BeneFix before surgical procedures.

BeneFix is **NOT** used to treat hemophilia A.

What should I tell my doctor before using BeneFix?

Tell your doctor and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal medicines.

Tell your doctor about all of your medical conditions, including if you:

- have any allergies, including allergies to hamsters.
- are pregnant or planning to become pregnant. It is not known if BeneFix may harm your unborn baby.
- are breastfeeding. It is not known if BeneFix passes into the milk and if it can harm your baby.

How should I infuse BeneFix?

The initial administrations of BeneFix should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

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

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

Call your doctor right away if bleeding is not controlled after using BeneFix.

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

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

wheezing	fast heartbeat
difficulty breathing	swelling of the face
chest tightness	faintness
turning blue (look at lips and gums)	rash
	hives

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

Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFix may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFix is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

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

How should I store BeneFix?

DO NOT FREEZE the BeneFix kit. The BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Throw away any unused BeneFix and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

BeneFix does not contain a preservative. After reconstituting BeneFix, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFix if the reconstituted solution is not clear and colorless.

What else should I know about BeneFix?

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

If you would like more information, talk with your doctor. You can ask your doctor or pharmacist for information about BeneFix that was written for healthcare professionals.

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-12.0, revised June 2020.

Pfizer

INSPIRING IMPACT

We're Stronger WITH Your Support!

There are many ways you can make a contribution to Hemophilia Federation of America as the end of year approaches. Donations are tax-deductible and support our efforts to advocate for the bleeding disorders community.

Annual Giving

Nov. 15 to Dec. 31

Donate online at www.hemophiliafed.org or send a check to 999 N Capitol St NE, Ste 201, Washington, D.C. 20002.

All donations will support Helping Hands, our financial assistance program.

Check if your employer will match your donation by using our simple search tool on hemophiliafed.org/matchinggifts.
It's a great way to double your gift!

Giving Tuesday

Dec. 1

Take part in this annual, international day of giving as the holiday season kicks off and show your support for HFA.

Donate online at www.hemophiliafed.org.

Annual Membership

Renew or initiate an individual, professional, or corporate membership today! Individual memberships start at just $35.00.
Visit www.hemophiliafed.org/membership.

Cyber Monday

Nov. 30

Participate in one of the biggest online shopping days of the year, and support HFA by making your purchases using Amazon Smile.

When you shop on Amazon Smile, you'll find the same low prices and vast selection as on Amazon, with the added bonus that Amazon will donate a portion of the purchase price to HFA. All it takes is three easy steps:

- Sign up for Amazon Smile through your Amazon account at smile.amazon.com.
- Designate Hemophilia Federation of America as your charity of choice.
- Make purchases on Amazon Smile and a percentage automatically goes to HFA.

Amazon Smile is available year-round so be sure to designate HFA as your charity of choice today.

Other Ways to Give

- When making holiday gift purchases, ask if the store will donate a portion of the sale to a charity.
- In lieu of giving a physical present, consider making a Tribute Gift in the name of a loved one.
- Ask your employer if they match employee charitable gifts.

With your help, HFA will be able to deliver educational programming, help families experiencing a financial crisis, advocate for the bleeding disorders community and so much more. Thank you in advanced for your generosity and support.

MoRe CoRe.

Our new CoRes.

The Sanofi Genzyme Community Relationship and Education (CoRe) team is growing. With our larger team, we'll be able to bring a higher level of personalized attention to patients affected by rare blood disorders.

To us, it's personal.

We're here for you. Let's connect.
Call us at 855-693-5628
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