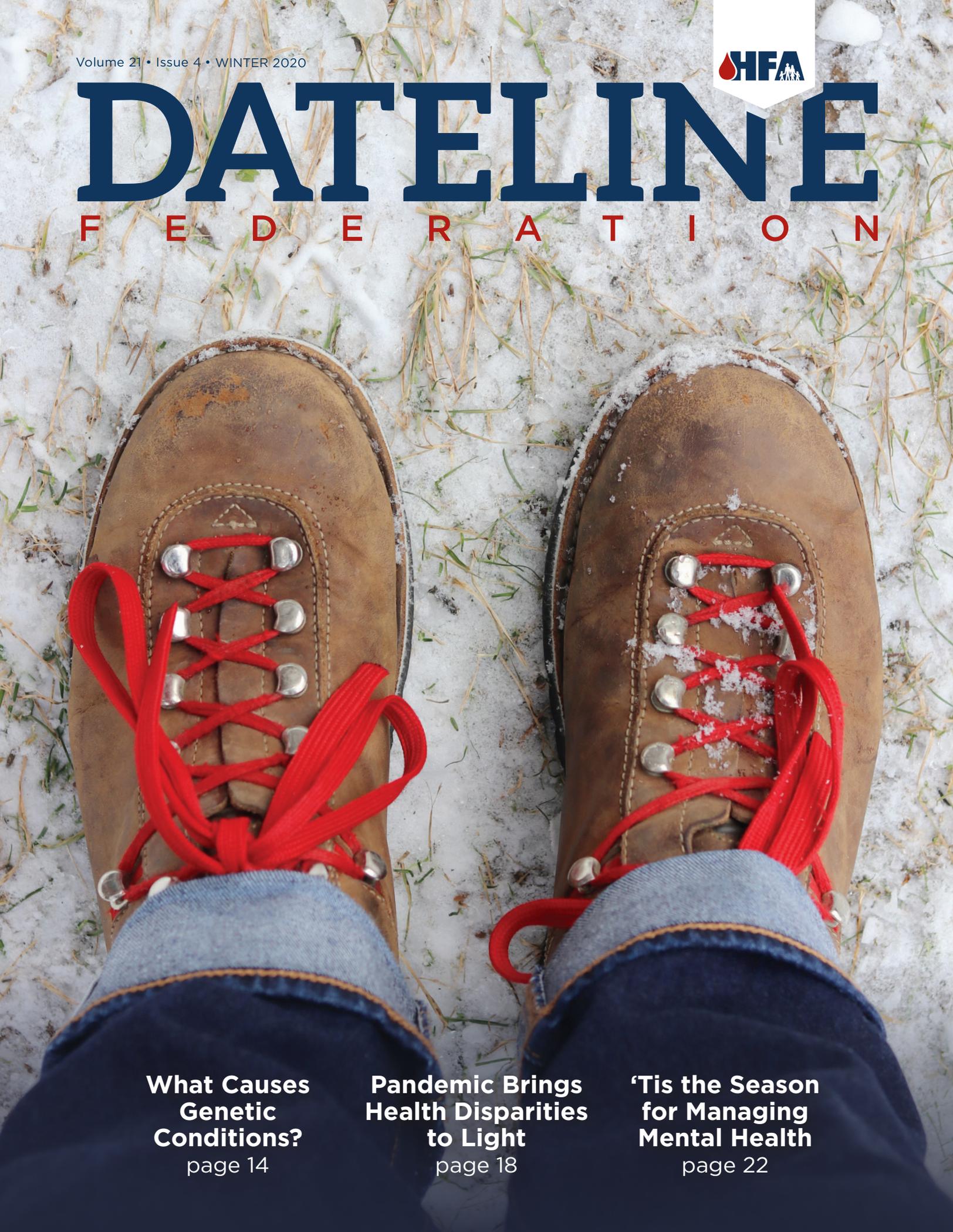




DATELINE

F E D E R A T I O N



**What Causes
Genetic
Conditions?**
page 14

**Pandemic Brings
Health Disparities
to Light**
page 18

**'Tis the Season
for Managing
Mental Health**
page 22

Dear Friends,

As we begin a new year, it is important to take a moment to reflect on 2020. Our staff understands how hard the past year has been. Personally, we've lost family and friends to the virus and experienced the angst of exposure. We've changed our routines; our children are learning in our dining rooms instead of a classroom, and we're sharing our workspaces with spouses. We've worked hard to slow the spread of the virus by protecting ourselves and our loved ones.

Organizationally, we closed our office in D.C. while everyone worked from home and halted travel. We moved Symposium to a virtual event, responded quickly to cultural and societal changes, and maintained all of our programs and services despite the challenges. We strengthened our advocacy efforts throughout the year as we worked toward product safety and access to quality, affordable care and coverage. We started a diversity and inclusion working group that encompasses all areas of the organization and worked hard to help individuals and families in the bleeding disorders community who are dealing with job loss and stress due to the virus. Our Helping Hands COVID-19 Relief Fund has approved more than 540 applications for individuals in desperate need with housing, utilities and transportation payments, disbursing more than \$330,000 into our community. Despite the hardships and challenges of 2020, our commitment to our mission to assist, educate and advocate for the bleeding disorders community was stronger than ever.



As we look ahead to 2021, after such a tough year, we want to reflect on what has makes HFA special. Our vision is to focus the organization by embracing our roots, increase the “family” feel of HFA, build trust and solicit constructive feedback from the community on how we can better serve your needs. As we strategically plan our future, we hope to smartly grow while never forgetting our history – what a privilege it is to serve so many resilient and wonderful people in the bleeding disorders community.

In closing, we hope you enjoy this final edition of Dateline for 2020. We also hope 2021 brings you much joy, gratefulness and happiness. Please remember to stay connected because we are always stronger together!

Dr. Sharon Myers, CFRE

Sharon Myers
President & CEO



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

Volume 21 • Issue 4 • Winter 2020

EXECUTIVE LEADERSHIP

Board Chair • Allie Ritchey
President & CEO • Dr. Sharon Meyers, CFRE

PRODUCTION TEAM

Editor • Emily Roush-Bobolz
Art Direction • Michael DeGrandpre
Design & Layout • Ashley Williams
Copy Editor • Kit Salisbury
Informative Communications Manager
Kyle McKendall

Contributing Writers

Ashley Smith, HFA Staff
Kimberly Ramseur, JD, M.P.H., HFA Staff
Emily Roush-Bobolz, HFA Staff
Sue Geraghty, Guest Writer

Contributing Photographers

Michael DeGrandpre, Gina Richards

The information printed in Dateline Federation is provided for general purpose use. HFA does not give medical advice or engage in the practice of medicine and recommends that you consult with your physician or local treatment center before beginning any form of treatment.

Requests for permission to translate or reprint contents of Dateline Federation should be directed to info@hemophiliafed.org.

999 N. Capitol Street NE • Suite 201
Washington, DC 20002
202.675.6984
info@hemophiliafed.org

©2020 Hemophilia Federation of America.
All rights reserved.

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



ARTICLES



12

Community Members Serving Community Members — Read more about the 2020 Symposium award winners.



14

What Causes a Genetic Condition? — We know bleeding disorders are genetic conditions, but what *really* causes a genetic condition?

22

Cover Story: 'Tis the Season for Seasonal Affective Disorder — Mental health of the bleeding disorders community, including seasonal depression, is something we should address.

SPECIAL FEATURES

4

Happening Highlights — Recent grant awards have given Hemophilia Federation of American an opportunity to bring more research and services to the bleeding disorders community.

6

Inspiring Impact — A first time Gears ... for Good participant learns what it means to become part of the HFA "family."

35

Member Organization Spotlight Local organizations address the unique needs of the communities they serve.



ON THE COVER.

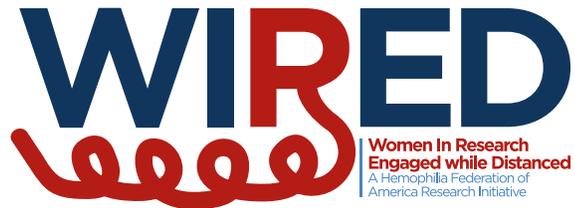
Anyone can experience Seasonal Affective Disorder, but people living with chronic illness and chronic pain have a stronger propensity to developing the temporary depressive event. We break down the symptoms, causes and ways to find help starting on **page 22**.

Photo: Michael DeGrandpre

HAPPENING HIGHLIGHTS



HFA Awarded PCORI Grant to Better Connect Women During Pandemic



Hemophilia Federation of America has received a Eugene Washington PCORI Engagement Award from the Patient-Centered Outcomes Research Institute for its WIRED (Women in Research Engaged While Distanced) Project, to improve women's engagement in patient-centered research while social distancing.

When many states in the United States gave stay-at-home orders because of the global pandemic caused by COVID-19, it put a halt to the usual participation in and

dissemination of research. With travel restrictions in place and the risk traveling places on the patient community, connecting patients, specifically women, became challenging. It caused research stakeholders to rethink the usual forms of engagement and to create opportunities for teams to connect remotely.

To deliver training effectively, virtual and distance methods of engagement needed to be identified, explored and evaluated to understand which methods result in higher continued community building, and to be best prepared for future emergencies or situations that require social distancing.

To solve this problem, HFA identified promising approaches for sustaining engagement of women affected by rare diseases in times of social distancing due to COVID-19, and developed WIRED Academy.

Women selected to participate in WIRED Academy received information about bleeding disorders, the research process and the importance of advocating for more research on women with bleeding disorders through modules available on HFA's Learning Central e-learning platform and through live virtual seminars with experts in these fields. Women also built relationships between their Blood Sisters as they discussed course topics together and shared their experiences as women with bleeding disorders.

PCORI awards the Eugene Washington PCORI Engagement Awards to support projects that encourage active integration of patients, caregivers, clinicians and other healthcare stakeholders as integral members of the patient-centered outcomes research/clinical effectiveness research (PCOR/CER) initiative.

Learn more about WIRED at www.hemophiliafed.org/WIRED.

HAPPENING HIGHLIGHTS

New Quarterly Email to Cover State Issues

In 2020 Hemophilia Federation of America began sending a quarterly e-newsletter called State of the States. With an increased awareness of policy and advocacy issues on a national level, HFA wanted to keep the bleeding disorders community informed about the work happening on the ground at the local level. Policy and advocacy work is increasingly important at the state level and is often a more accessible way for bleeding disorders patients and their families to become involved in self-advocacy with their local member organizations.

The information will also be available on the website at www.hemophiliafed.org/news.



HFA Seeks Internship Applications

Hemophilia Federation of America is accepting applicants until March 15 for its annual 10-week Policy and Government Relations Internship, which will take place from May to August 2021. HFA will provide leadership and guidance for two college students or recent graduates with bleeding disorders or students related to a person with a bleeding disorder. Those chosen will develop a core passion in health care policy and self-advocacy, helping to shape the next generation of advocacy leaders, and may attend relevant virtual events, provide support for webinars, assist with PAGE programs and draft bi-weekly blog posts.

Learn more and apply at www.hemophiliafed.org/internship.

Welcome TO THE FAMILY!

Virtual event allows new bleeding disorders families to join the family-like atmosphere

BY ASHLEY SMITH, STAFF WRITER

The coronavirus made 2020's Gears for Good National Ride, part of Hemophilia Federation of America's Team Resilience endurance fundraising team, look completely different from past years.

HFA intended for cyclists and the support team to be together in person, cycling through the scenic landscape and small towns of West Virginia to Washington, D.C., to celebrate the 10th anniversary of the ride's creation. When the ride was turned virtual, it was a great disappointment to those who planned to participate in this annual tradition — a tradition that would have brought blood brothers and sisters together for an opportunity to welcome one family within the community.

This family of new participants — Meagan Murray and her 10-year-old son, TJ, of Rhode Island — had been unable to participate in the Gears for Good National Ride previously, but this year they knew they could make it happen because the event transitioned to a virtual challenge.

They chose to accept the challenge and rode 156 miles in their

hometown. With Gears for Good being a long-distance ride, the 2020 virtual option allowed riders to log miles at their own pace throughout the course of an entire week, rather than three days, offering some welcome flexibility for those who participated. TJ said he wanted to join the virtual challenge with his mom to get exercise and because he had been stuck inside due to COVID-19.



Lindsey Russell and husband Rob participated as a family of two, to raise money for HFA's Helping Hands program.

“My mom and I had some really good conversations while biking. I wanted to join to help others with bleeding disorders and because it looked fun,” said TJ.

Riders were challenged with riding 156 actual miles, and when asked the hardest part of the challenge, TJ exclaimed, “Biking! We did so much in a day. My little kid muscles didn't like it very much. Also, I wiped out a few times and had to take my blood medicine more than I wanted to. By the last night, we had 8.9 miles left, so we decided to go out with our lights on and finish. It was the first time riding in the cold and I wish I had gloves.”

Gears for Good helps raise awareness of bleeding disorders and funds for HFA's Helping Hands program, which provides

INSPIRING IMPACT

I stopped because I was tired in front of a lady's house. She asked why I biked so much with my mom and I told her about having vWD. Luckily I was wearing my HFA shirt and she gave me \$20 for the cause!

-TJ Murray

emergency financial assistance to the bleeding disorders community in their time of need. Every chance a story from Gears for Good or another Team Resilience event can be shared, it educates people outside the community. One day while TJ and Meagan were on their ride, he was able to provide some education to a neighbor.

"I stopped because I was tired in front of a lady's house. She asked why I biked so much with my mom and I told her about having vWD," said TJ. "Luckily I was wearing my HFA shirt and she gave me \$20 for the cause!"

Meagan had shared with TJ that Helping Hands is HFA's Emergency Assistance Program. "I feel really proud I was able to help others," he said.

Even though riders did not get to join together in person and create the camaraderie that typically fills the September weekend, the 2020 riders were able to collectively raise more than \$24,000 for Helping Hands.

HFA hopes to keep the virtual option available for anyone who is unable to attend the in-person event in 2021, as being able to experience and demonstrate the resilient behavior that one needs to complete this type of endurance challenge is powerful and motivating. It echoes the characteristics of those living with bleeding disorders daily.

TJ plans to train hard to prepare for the ride next year. He hopes

others will consider training and joining him on the trails, either in person or virtually.

"It took us a week and we barely made it," he said. "I don't know how the rest of the team does this in three days."

Another great aspect of the virtual challenge was riders could share photos from across the nation, showing different trails and neighborhoods where they logged miles. Riding by the ocean was TJ's favorite part of the challenge.



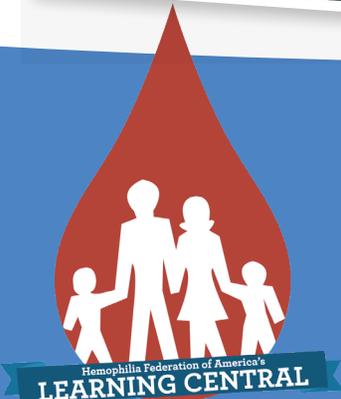
Learn more about Team Resilience and future events at www.TeamHFA.org.



Find interactive online learning on:

- Bleeding Disorders Basics
- Types of Bleeding Disorders
- Inhibitors
- Research & Clinical Trials
- Current, New & Emerging Therapies
- Joint Health
- Employment Rights

Please join us at www.HFALearning.org to find out more!



When it comes to your hemophilia A treatment

Move beyond the threshold^a

Esperoct[®] can give you high factor levels for longer.^b

Extend half-life beyond the standard 22-hour average half-life in adults^c

FOR ADULTS AND ADOLESCENTS

Switching made easy with a standard 50 IU/kg dose every 4 days

-50% fewer infusions if you previously infused every other day

-40% fewer infusions if you previously infused 3x a week

High factor levels

At or above 3% for 100% of the time^{d,e}

At or above 5% for 90% of the time^{d,f}

Flexible on the go

The ONLY extended half-life product that can be stored up to 104^{°F}

Please see Brief Summary for complete storage instructions.

Safety Proven across 5 studies, the largest and longest EHL clinical trial program

^aOf 1% trough factor levels for standard half-life (SHL) products in adults and adolescents.

^bCompared with SHL products.

^cData shown are from 42 adults who received a pharmacokinetic (PK) assessment around the first Esperoct[®] 50 IU/kg dose.

^dTrough level goal is 1% for prophylaxis.

^eData shown are from a study where 175 previously treated adolescents and adults received routine prophylaxis with Esperoct[®] 50 IU/kg every 4 days.

Pre-dose factor activity (trough) levels were evaluated at follow-up visits. Mean trough levels for adolescents (12-<18 years) were 2.7 IU/dL.

^fSteady-state FVIII activity levels were estimated in 143 adults and adolescents using pharmacokinetic modeling.

^gFor up to 3 months.

What is Esperoct[®]?

Esperoct[®] [antihemophilic factor (recombinant), glycopegylated-exei] is an injectable medicine 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

- Esperoct[®] is not used to treat von Willebrand Disease

IMPORTANT SAFETY INFORMATION

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[®] or if you are allergic to hamster proteins

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
- 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

What should I tell my healthcare provider before using Esperoct[®]?

- Before taking 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, pregnant or planning to become pregnant, or have been told that you have inhibitors to factor VIII
- Your body can make antibodies called "inhibitors" against Esperoct[®], which may stop Esperoct[®] from working properly.

Call your healthcare provider right away if your bleeding does not stop after taking Esperoct[®]

What are the possible side effects of Esperoct[®]?

- Common side effects of Esperoct[®] include rash or itching, and swelling, pain, rash or redness at the location of infusion

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

Discover more at [Esperoct.com](https://www.esperoct.com).



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

Esperoct[®] is a registered trademark of Novo Nordisk Health Care AG.

Novo Nordisk is a registered trademark of Novo Nordisk A/S.

© 2020 Novo Nordisk Printed in the USA. US20ESP00014 February 2020

esperoct[®]

antihemophilic factor (recombinant), glycopegylated-exei

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 86°F (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.

Manufactured by:
Novo Nordisk A/S
Novo Allé

DK-2880 Bagsværd, Denmark

For information about ESPEROCT® contact:

Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA
1-800-727-6500

© 2019 Novo Nordisk

US19ESP00168 December 2019



novo nordisk®



HFA'S ANNUAL SYMPOSIUM AWARDS

Each year at Symposium, Hemophilia Federation of America recognizes individuals in the bleeding disorders community who have made a positive impact. While Symposium was turned into a virtual educational conference in 2020, HFA still honored those who made a difference.



VOLUNTEER OF THE YEAR

Tracy Cleghorn

Selected by the HFA staff in recognition of tremendous national spirit and remarkable volunteerism with HFA for the bleeding disorders community.

Tracy Cleghorn is one of the first people to volunteer to help. As she transitioned from board chair, she found other ways to lead and continues to serve the community through her work on the Symposium committee from 2019 to 2020, governance committee, and CEO search committee, among others. Her time, efforts and visible leadership do not go unnoticed. HFA and the bleeding disorders community are stronger as a result of Tracy's consistent style, passion and leadership!

Carl Weixler

For extraordinary lifetime service that encompasses national volunteerism, professionalism and leadership.

As the board president for the Committee of Ten Thousand (COTT), Carl Weixler has been a strong advocate for his fellow blood brothers and sisters since he was a child. At the tender age of 4, Carl met with the governor to discuss a "hemophilia program" for the state of Kentucky. And it didn't stop there.

Carl's entire life has been full of advocacy efforts at both state and national levels. He has sat on numerous advisory boards for governmental agencies and manufacturers, is a past HFA board chair, and has represented the bleeding disorders community across the globe. He welcomes each and every blood brother that comes through the door, with open arms, and is always looking out for the safety and well-being of others. He really has been a true advocate and supporter of our community since the very beginning.



CHARLES STANLEY HAMILTON LEGACY AWARD



IT TAKES A VILLAGE AWARD

Harvey Gates Sr.

Provided to an individual who goes above and beyond in uniting the community and has demonstrated a unique ability to link those in need, from the experienced bleeding disorder veteran to the newly diagnosed family.

For more than 30 years, Harvey Gates Sr. has been an active servant leader in hemophilia and bleeding disorders communities throughout the country. His visions are to unite and empower families and provide a platform for the much-needed social support as families face the challenges of living with a bleeding disorder. As a native Georgian, he knew of the gap in service for the bleeding disorders community in his home state, and he endeavored to do outreach to bridge the gap and meet the needs of the Georgia bleeding disorders community, co-founding United Hemophilia Foundation in 2016.

His fervor is also driven by the tragedy that struck the community in the 1980s, which resulted in the death of his father-in-law, mother-in-law, and a nephew due to hemophilia related complications. Since that time, he has worked to highlight awareness and to ensure access to quality healthcare to those affected by Hemophilia.



RON NEIDERMAN HUMANITARIAN AWARD

Rich Pezzillo

For extraordinary and inspirational service via one's professional work or volunteer service, in memory of Ron Neiderman.

Rich Pezzillo, Executive Director of the New England Hemophilia Association, goes above and beyond for the bleeding disorder community. Rich does everything in his power to help community members from feeling alone. Whether it's lunch with a new family or a game of corn hole with a shy child at Family Camp, Rich makes every single person he meets feel important. From New Family and Spanish Heritage weekends, to Couples Retreats and Family Camp, Rich has made sure that his community has the programs and services they need to thrive.

Laveane Lovelady

In recognition of special, behind-the-scenes volunteer work.

HFA is pleased to honor Laveane Lovelady with this award in recognition of dedicated service on the committee for HFA's Helping Hands Program. Laveane volunteered countless hours over the course of many years during her time on the committee.

Her empathy, compassion and advocacy for the bleeding disorders community was integral to the Helping Hands Program in providing support to our most vulnerable community members.



SPECIAL SERVICE RECOGNITION

Chris Bombardier

Awarded for exceptional commitment to supporting HFA in its national efforts to encourage health, nutrition and wellness behaviors in the bleeding disorders community.

Living with severe hemophilia has not stopped Chris Bombardier from reaching his goals. Goals that are quite literally some of the “highest” one can set. Chris is the first person living with hemophilia to climb the Seven Summits, the highest mountains on each of the seven continents – Mount Everest, Aconcagua, Denali, Kilimanjaro, Mount Elbrus, Vinson Massif and Carstensz Pyramid, raising funds for Save One Life Inc. each step of the way. Chris is an incredible role model for those living with a bleeding disorder and is now one of only 450 climbers in history to complete the seven summits – and the only one with hemophilia. Throughout his journey, he has raised awareness of what living with Hemophilia means in developing countries and is an inspiration to people living with bleeding disorders across the globe. It’s an honor to present the Terry Lamb Health and Wellness Award to Chris Bombardier.



**TERRY LAMB
HEALTH AND
WELLNESS
AWARD**



**MEMBER
ORGANIZATION
SPOTLIGHT
AWARD**

Hemophilia of South Carolina, Sue Martin, Executive Director

Honors an HFA Member Organization that has created an environment within their membership that engages members, continually promotes education and consistently encourages self-advocacy.

Hemophilia of South Carolina continually goes above and beyond for their members, especially given that they only have one staff person – Executive Director Sue Martin. Their South Carolina Hemophilia and Bleeding Disorders Advocacy Coalition has allowed HSC to advocate across their large state. Hemophilia of South Carolina travels state-wide to meet with their members and provide services and events. They are known to be the most family-like chapter, greeting with hugs and support for each other while teaching self-advocacy with their ambassador programming, education and supportive services.

HFA also recognizes former board chair, Josh Hemann, for his service and commitment to the board. Josh, who has served on the HFA board for eight years, and his wife, Heather, have three children and their two daughters have von Willebrand Disease. He has been a part of the HFA’s Dads in Action program since attending his very first Symposium and just completed his second year as board chair, now serving as past chair.





WHAT CAUSES GENETIC CONDITIONS?

PROVIDED BY AND PRINTED
WITH PERMISSION BY BIOMARIN

**DID YOU KNOW THERE ARE
APPROXIMATELY 20,000 GENES IN
THE HUMAN GENOME?**

A mutation or permanent variation, in just one gene can lead to a genetic condition. Knowing what causes a genetic condition is the first step in understanding how ongoing research in gene therapy might enable physicians to treat these conditions differently. No gene therapies for hemophilia A or B have been approved for use or determined to be safe or effective.

**GENETIC CONDITIONS ARE THE
RESULT OF MUTATIONS**

Genetic conditions are the result of mutations, or variations, in the make-up of a gene. These mutations are most often passed down from biological parents but can sometimes happen spontaneously. Cystic fibrosis and hemophilia are examples of genetic conditions.

In hemophilia A, the gene responsible for producing factor VIII is mutated. This gene is located in the X chromosome. Males have only one X chromosome, which means that one copy of the mutated gene is enough to cause hemophilia, making it more common in this population.

While it is possible for females to have hemophilia, it is rare for them to present with symptoms of the disease because the disease affects just one X chromosome. Since females have 2 X chromosomes, both chromosomes would need to have a mutation for them to be severely affected by the disease. However, females who do not show signs of hemophilia are often referred to as “carriers” because they can still pass on the mutated gene to their children, even though they have no symptoms of the condition.

MUTATIONS CAN AFFECT YOUR GENETIC INSTRUCTIONS

A mutation can affect the genetic instructions in the body. The instructions can be missing or incorrect, changing the way proteins are produced. This can result in the production of a protein that does not work properly, or in some cases the protein is not produced at all. Mutations can take the form of changed nucleotide pairings, extra DNA where it doesn't belong, missing DNA, or repeated DNA.

In people with hemophilia A or B, the genetic mutation affects the body's ability to produce a protein called factor VIII or factor IX, respectively. These proteins are critical for blood to clot.

THERE ARE 3 TYPES OF GENETIC CONDITIONS

1

Monogenic conditions—like hemophilia—are caused by a mutation in a single gene. Other examples include cystic fibrosis and Huntington disease.

2

Multifactorial inheritance conditions, or multi-gene conditions, develop from multiple small genetic mutations and can lead to some of the more common diseases we're familiar with, such as heart disease and diabetes.

3

Chromosome disorders are caused by changes to the number or structure of chromosomes. Down syndrome is the most common disorder related to this type of abnormality.

GLOSSARY



GENE

A part of a DNA molecule that tells your body how to make a protein. A mutation within certain genes can, for example, lead to deficient protein, which can lead to genetic conditions. Gene therapy is under investigation to evaluate the risks and whether it can help the body to produce the protein it needs.



GENOME

An organism's complete set of genetic material. It contains all of the instructions needed for the organism to function. In people, every cell that has a nucleus contains a copy of the entire genome.



MONOGENIC CONDITIONS

Monogenic conditions, such as hemophilia, are caused by a single gene not working properly. The cause of the malfunction may be present in one or both copies of chromosomes inherited from the parents.

(note: Factor VIII and IX and definition of hemophilia removed because our readers tend to know this already)



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.

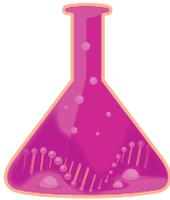


No gene therapies for hemophilia have been approved for use or determined to be safe or effective.

B:OMARIN

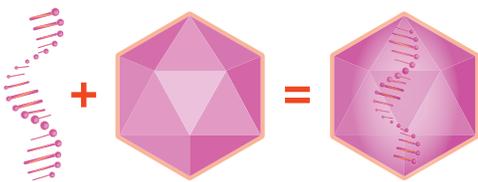
THE 5 STEPS OF INVESTIGATIONAL GENE TRANSFER

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.



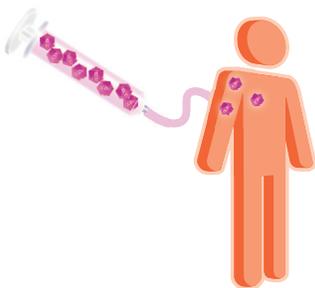
STEP 2 BUILDING A THERAPEUTIC VECTOR

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



BEHIND THE VEIL

**Challenging year brings systematic health
disparities in various groups to light**

BY KIMBERLY RAMSEUR, JD, M.P.H., STAFF WRITER

For much of 2020, the world has been forced to grapple with COVID-19 and its ongoing effects, but while this year proved to be challenging for everyone, not everyone has shared the same set of challenges.



For much of 2020, the world has been forced to grapple with COVID-19 and its ongoing effects, but while this year proved to be challenging for everyone, not everyone has shared the same set of challenges.

We have socially distanced ourselves for months, lost homes, jobs and even loved ones. It's as if life as we know it has come to an utterly strange halt at the same time it has unveiled many hard truths—most pointedly, that health care is not as widely available for all who need it. Despite what some may have believed, healthcare is not one-size-fits-all.

The bleeding disorders community is no stranger to the challenges of maintaining unfettered access to quality, affordable coverage and health care. Yet, there is another subset of our rare community that is met with such challenges.

COVID-19 is not the only challenge that 2020 presented us – for the past several months, many have also been overwhelmed with emotions ranging from sadness to horror and even rage, as Americans witnessed the killing of George Floyd while still grieving the tragic losses of Breonna Taylor,

Ahmaud Arbery, and many more.

Sadly, these are only the most recent examples of racism. Many communities have been faced with systemic racism for years, even generations, subjecting populations to trauma that has often led to socioeconomic, physical and mental health disparities, and has had significant social and economic costs both to individuals and societies.

HFA's Bleeding Disorders Health Disparities Council will:

- ⇒ Conduct a needs assessment using focus groups and publish findings.
- ⇒ Establish a working group to work toward the goal of equal treatment in health care.
- ⇒ Offer patient-focused solutions.
- ⇒ Assist HFA's member organizations and hemophilia treatment centers by publishing a best practices guidebook with local diversity, inclusion and equity tools.

According to the World Health Organization, health inequities are systematic differences in different population groups' health status. Factors such as education, employment status, income level, co-morbidities, race, gender, and ethnicity play a critical role in health status by posing barriers to care.

At Hemophilia Federation of America, our mission has always been to assist, educate and advocate on behalf of all people living with a bleeding disorder. To begin to address the barriers to care and inequality that exists within the community, HFA is embarking on a new opportunity – the Bleeding Disorders Health Disparities Council. The council will strengthen the care of persons with blood disorders by identifying areas of health disparities and inequities for members of the community: assessing needs, developing action items and initiating change.

The BDHDC will enable working groups to distribute community data to hemophilia treatment centers, industry, specialty pharmacies, member organizations, and other health care providers and stakeholders.

To learn more about the Bleeding Disorders Health Disparities Council, contact Senior Manager for Policy and Advocacy, Kimberly Ramseur, at k.ramseur@hemophiliafed.org.

GO SEEK. GO EXPLORE.
GO AHEAD.

PEOPLE LIKE YOU. STORIES LIKE YOURS.
Explore more at HEMLIBRAjourney.com



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. People who use activated prothrombin complex concentrate (aPCC; Feiba®) to treat breakthrough bleeds while taking HEMLIBRA may be at risk of serious side effects related to blood clots.

These serious side effects include:

- **Thrombotic microangiopathy (TMA)**, a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs
- **Blood clots (thrombotic events)**, which may form in blood vessels in your arm, leg, lung, or head

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


HEMLIBRA
emicizumab-kxwh | 150
injection for subcutaneous use | 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

HEMLIBRA® is a registered trademark of Chugai Pharmaceutical Co., Ltd., Tokyo, Japan
©2018 Genentech, Inc. All rights reserved.

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® is a registered trademark of Chugai Pharmaceutical Co., Ltd., Tokyo, Japan.
The HEMLIBRA logo is a registered trademark of Chugai Pharmaceutical Co., Ltd., Tokyo, Japan.
The Genentech logo is a registered trademark of Genentech, Inc.
All other trademarks are the property of their respective owners.
©2020 Genentech USA, Inc. All rights reserved. M-US-00007357(v1.0) 09/20

Genentech
A Member of the Roche Group



'TIS THE SEASON

Shorter days, cooler weather can usher in winds of change in the mental state of those with bleeding disorders

BY EMILY ROUSH-BOBOLZ, STAFF WRITER

Also featuring writing by Andy Anderson, guest writer/former HFA staff



Life with a chronic illness can bring many challenges, including an impact on the mental health of someone with a bleeding disorder.

Millions of Americans currently have or have experienced Seasonal Affective Disorder at some point in their life. SAD, or winter depression, is a type of depression related to changes in seasons. It begins and ends at about the same time every year – symptoms typically start in the fall and continue into the winter months, affecting energy and mood.

Chronic illness and chronic pain can exacerbate changes in mental health during the winter. People in the bleeding disorders

community living without an accurate diagnosis or effective treatment are at a higher risk for negative impacts on mental health. However, whether one has a diagnosis and effective treatment or not, it's a good idea to check in on mental health during winter months.

WHAT CAUSES SAD?

While research on SAD is ongoing, researchers have discovered a change in one's serotonin and melatonin levels as well as changes in the body's biological clock/circadian rhythm, due to changes in the amount of sunlight and vitamin D, may have an impact on mental health.

Serotonin, a key brain chemical/hormone in stabilizing mood, feelings of well-being and happiness, may drop when sunlight is reduced, triggering depression. The neurotransmitter enables brain cells and the nervous system to communicate and helps with sleep and digestion, so when levels change, changes to sleeping and eating habits may occur.

Melatonin, a naturally occurring hormone which is also available in over-the-counter pill form, regulates sleep and wake cycles. The more light or sunlight a person is exposed to, the more awake and alert the body is. Darkness causes the body to produce more melatonin, which signals the body to prepare for sleep, so shorter days and less exposure to light can trigger a feeling of sleepiness and depression.

In addition to changes in the levels of serotonin and melatonin, a deficiency in vitamin D may worsen the changes in mood. Vitamin D, which bodies naturally produce when exposed to sunlight, is believed to promote serotonin activity. Less daylight in winter means lower vitamin D levels, which may further hinder serotonin activity and lead to SAD.

People living with chronic illness and chronic pain should be more aware of the possibility of SAD during winter months. Some studies have shown a connection between chronic pain and a propensity for SAD. Low energy, feeling hopeless or worthless, and changes in weight, appetite and sleep can be signs of depression. Other signs and symptoms of SAD may include:

- ❄ Feeling depressed most of the day, nearly every day
- ❄ Losing interest in activities you once enjoyed
- ❄ Having low energy
- ❄ Having problems with sleeping
- ❄ Experiencing changes in your appetite or weight
- ❄ Feeling sluggish or agitated
- ❄ Having difficulty concentrating
- ❄ Feeling hopeless, worthless or guilty
- ❄ Having frequent thoughts of death or suicide

Continued on pg. 24 >



Sunday 4:07 pm
Enjoying a game of checkers with his wife
Alan, living with hemophilia A with inhibitors

Not an actual patient

WE'RE IN THIS TOGETHER.

Let's make today brilliant.

Takeda is here to support you throughout your journey and help you embrace life's possibilities. Our focus on factor treatments and educational programs, and our dedication to the bleeding disorders community, remain unchanged. And our commitment to patients, inspired by our vision for a bleed-free world, is stronger than ever.

bleedingdisorders.com



Copyright © 2020 Takeda Pharmaceutical Company Limited. All rights reserved. TAKEDA and the TAKEDA logo are trademarks or registered trademarks of Takeda Pharmaceutical Company Limited. US-NON-0136v1.0 03/20



FALL AND WINTER SAD

Symptoms more specific to winter-onset SAD may include:

-  Oversleeping
-  Appetite changes, especially a craving for foods high in carbohydrates
-  Weight gain
-  Tiredness or low energy



SPRING AND SUMMER SAD

While SAD during spring and summer is more rare than fall and winter SAD, it can happen. Symptoms specific to summer-onset SAD may include:

-  Trouble sleeping (insomnia)
-  Poor appetite
-  Weight loss
-  Agitation or anxiety

Visit with your hemophilia treatment center if you are experiencing symptoms of SAD. There are options for treatment, such as therapy, light therapy, medication and vitamin D supplements.

If you or a loved one are experiencing mental health challenges, visit HFA's Patient Assistance Portal for valuable mental health resources.

www.hemophiliafed.org/patientassistanceportal

If you need immediate help, please contact your doctor or text or call the mental health resources available on page 24.

While efforts are made to ensure accuracy of the content, this article is not intended to be construed as medical advice or the official opinion/position of HFA, its staff, or its Board of Directors. Readers are strongly encouraged to discuss their own medical treatment with their healthcare providers.

TIPS FOR COMBATING DEPRESSION... SEASONAL OR OTHERWISE!



KEEP A ROUTINE

The circadian rhythm plays a big role in our brain's ability to stay balanced. Going to sleep and waking up at the same time every day can support the brain during these seasonal shifts.



GET AS MUCH SUNLIGHT AS POSSIBLE

Sitting by a window, taking a walk, or sipping your coffee on the front porch are great (free!) ways to maximize your exposure to UV rays. A lightbox or sun lamp is a good option for those who live in areas with short daylight hours in winter.



A BODY IN MOTION

Studies have shown time and again that physical activity is beneficial to our stress response and mental health. You don't have to take up marathon running to reap the benefits of physical activity. Walking the dog, making time for stretching, and dancing around the house are easily accessible ways to get moving! Make time for what feels good for your body.



EAT WELL

While we tend to enjoy those holiday goodies and have a tendency to make poor food choices in the winter, they're not always the best for regulating the bodies ups and downs. Chose healthier foods, less sugary foods and consume healthy beverages to regular the body and help improve mood.



EXPERIENCE MATTERS

BeneFix is FDA approved for once-weekly prophylaxis and on-demand use to fit your dosing needs—from the only recombinant factor IX supporting individuals with hemophilia B for more than 20 years.*

Not actual patients.



More than 20 years* of experience—the first recombinant treatment for individuals with hemophilia B



Dosing options to meet your needs—for once-weekly prophylaxis and on-demand use



Designed with viral safety in mind. More than 150 quality control tests are done on each batch of BeneFix



The convenience of the BeneFix Rapid Reconstitution (R2) Kit with a range of vial sizes



What Is BeneFix?

BeneFix, Coagulation Factor IX (Recombinant), is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Your doctor might also give you BeneFix before surgical procedures.

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

Important Safety Information

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash, or hives.
- Your body can make antibodies, called “inhibitors,” which may stop BeneFix from working properly.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness, and rash.

Please see the Brief Summary for BeneFix on the next page.

ASK YOUR DOCTOR WHICH BENEFIX DOSING OPTIONS MAY BE RIGHT FOR YOU



BeneFix

Coagulation Factor IX (Recombinant)
Room Temperature Storage

*BeneFix was approved February 11, 1997.

R_x 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.



SAVE THIS PAGE

CRISIS TEXT LINE

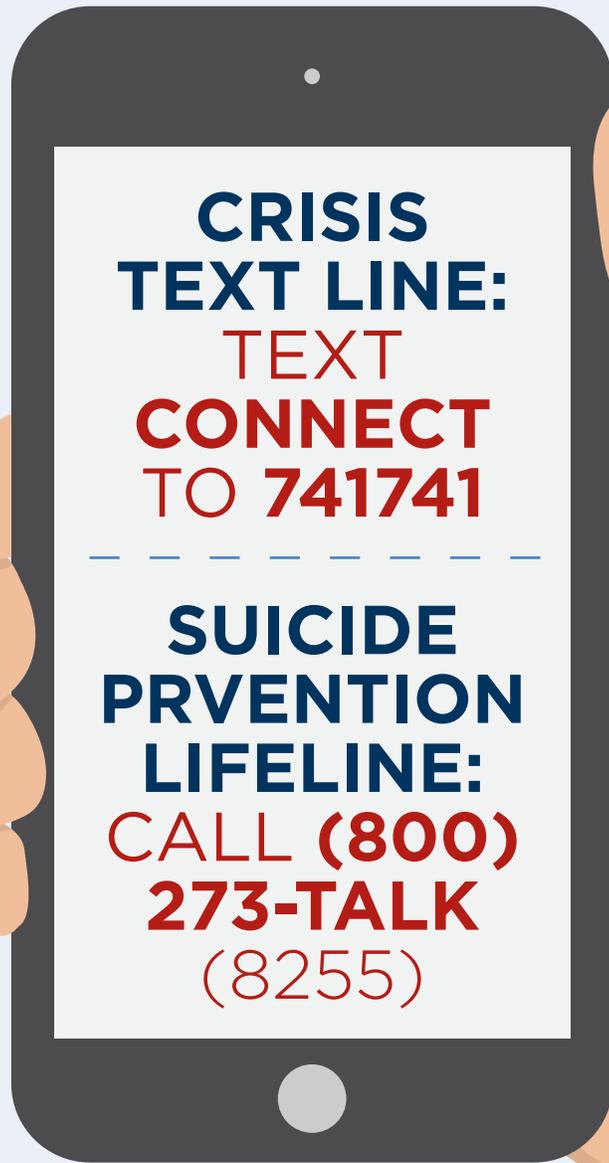
Crisis Text Line is a free 24/7 texting only support service. You are connected to a live, trained crisis counselor to help move you from a hot moment to a cool calm while helping you create a plan to stay safe and healthy.

→ www.crisistextline.org

SUICIDE PREVENTION LIFELINE

The lifeline provides 24/7, free and confidential support for people in distress, as well as prevention and crisis resources for you or your loved ones.

→ www.suicidepreventionlifeline.org



Other Reputable Resources:

www.mentalhealthfirstaid.org

www.twloha.com

www.bethe1to.com

→ **The National Suicide Prevention Lifeline website** also has information on providing support on social media, such as how to report a suicidal post on Facebook, self-harm on Twitter or safety concern on SnapChat. They have information on engaging and supporting someone online.

THE IMPACT ON PATIENTS AND FAMILIES

Discovering how a global pandemic has had an effect on the bleeding disorders community

BY EMILY ROUSH-BOBOLZ, STAFF WRITER

In the spring of 2020, the usual way of life for the entire nation changed.

While Hemophilia Federation of America quickly responded by providing resources and information to the bleeding disorders community, the organization also wanted to better understand how the community was affected.

HFA conducted an online survey among bleeding disorders patients and their families to discover the impact on mental health, access to care and education, and more. The results were eye opening. The open survey (now closed) and results were shared on HFA's Facebook page.

Nationally many schools and businesses closed or limited hours in the spring in order to stop the spread of the novel virus. Among the bleeding disorders community, nearly half had children

suddenly learning at home or the adult patients or caregivers discovered they too would be learning at home. In addition to education, employment changed due to closings – in the bleeding disorders community 28% lost their job.

In addition to the changes to education and employment, the changes in access to care for patients and their families was perhaps more troublesome. Fourteen percent were unable to get the medication or treatment they needed and 33% were unable to see their provider. Most healthcare providers quickly shifted to telehealth appointments.

The largest concern with the global pandemic was on the mental health of U.S. citizens and perhaps even more on patients with chronic illness, who may sometimes be at greater risk of mental health concerns. The survey showed nearly 75% experienced mental health issues. But to help manage these issues, patients and caregivers turned to connecting with friends online, taking care of their bodies with exercise, reconnecting with hobbies and contacting mental health providers.

HFA's COVID-19 Hub features:

- Product availability & updates
- Information on insurance coverage & concerns
- Fraud alerts
- Financial assistance resources
- Mental health resources

www.hemophiliafed.org/covid19

COVID-19 Relief Fund Available for Bleeding Disorders Families

HFA is providing financial relief to members of the bleeding disorders community grappling with a significant loss of income due to COVID-19 with the **HFA COVID-19 Relief Fund**. It provides emergency financial assistance for payment toward essential household bills such as mortgage, rent, utilities or car payment.

Visit www.hemophiliafed.org/COVID-Fund for eligibility information and to apply.



SURVEY SAYS

In the spring of 2020, participants coped with the pandemic by:



77%
Connecting with friends and family via technology

56%
Exercising

44%
Spending time on hobbies

23%
Using mindfulness techniques

16%
Accessing therapy/counseling

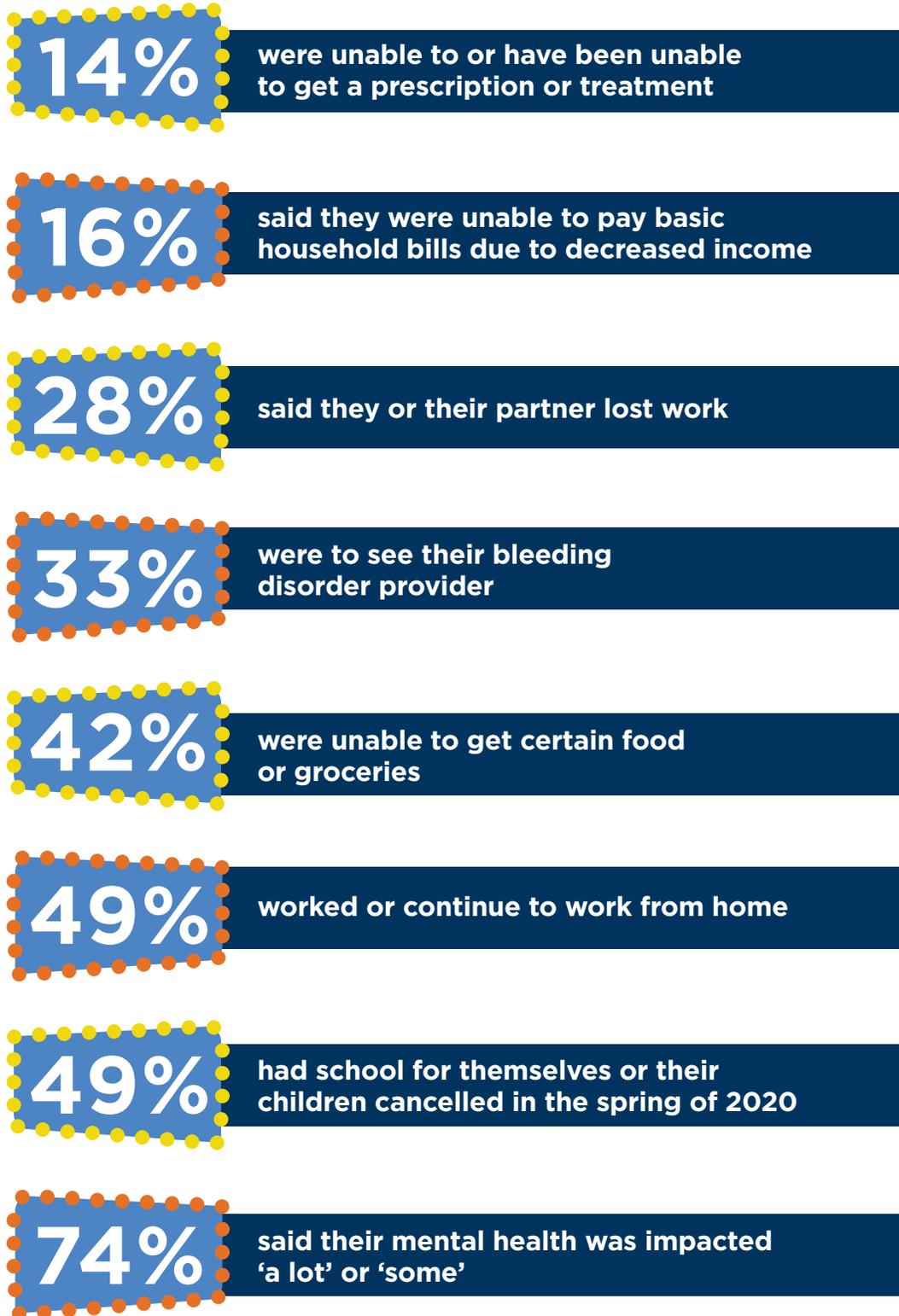


Survey conducted by



Hemophilia Federation of America's 2020 COVID-19 Mental Health & Coping Survey

When the COVID-19 pandemic hit the United States in the spring of 2020, an HFA survey of bleeding disorders patients and their families showed the impact the pandemic had on survey participants:



THE STIMATE RECALL

AND WHAT YOU NEED TO KNOW

BY SUE GERAGHTY, R.N., GUEST WRITER

In August of 2020, Ferring Pharmaceuticals Inc. extended the recall of Stimate® Nasal Spray (desmopressin or DDAVP) to the consumer level. Patients with vials of Stimates listed in the chart below should stop taking it and make arrangements to return to the distributor.

Many consumers with mild factor VIII deficient hemophilia, mild to moderate von Willebrand disease and mild platelet function defects rely on this medication to treat bleeding episodes on demand when they occur or on a prophylactic basis for monthly menstrual cycles or prior to physically demanding activities, like sports.

Stimate was recalled due to superpotency, or higher amounts of desmopressin in the vial than the label indicates. The risks of too much desmopressin include hyponatremia (a low sodium level) and fluid retention. Mild symptoms can include headache, fatigue and nausea. More severe symptoms include confusion and seizures.

In early October, CSL Behring, the distributor of Stimate, sent this announcement: “Ferring Pharmaceuticals Inc. initiated a

voluntary, consumer-level recall of all batches of Stimate® Nasal Spray (desmopressin acetate) 1.5 mg/mL, which is distributed and sold by CSL Behring LLC. At this time, Ferring does not anticipate resupply before 2022.”

What does this mean to patients or family members who relied on Stimate to treat a bleeding disorder? Patients should have a serious conversation with their healthcare provider or hemophilia treatment center as soon as possible to determine a new treatment plan. Do not accept generic DDAVP as replacement from your pharmacy, it is not the same strength as Stimate.

For patients with mild hemophilia A, there are many factor VIII products that may meet their needs – patients should discuss with a healthcare provider.



DO NOT USE THE FOLLOWING VIALS OF STIMATE

Arrange to return to CSL Behring, the distributor of Stimate at (800) 676-4266

PRODUCT	NDC	BATCH	PRODUCT
Stimate*	0053-6871-00	R15953C	30-SEP-22
		R14667A	31-AUG-22
		R14101A	31-JUL-22
		R13648A	30-JUN-22
		R13271A	30-APR-22
		R11845A	30-APR-22
		P13756P	31-APR-21
		P13755A	30-JUN-21
		P13212H	30-JUN-21
		P13209L	30-APR-21
		P11326C	28-FEB-21
		P11326AA	28-FEB-21
		N17445N	31-DEC-20
		N15378G	30-SEP-20
		N14134C	31-JUL-20

For patients with von Willebrand disease who use Stimate, some products containing von Willebrand factor may meet their needs. Below is a table of those products:

MANUFACTURER	PRODUCT	TYPE
CSL Behring	Humate-P	Plasma-derived Clotting Factor
Ferring Pharmaceuticals	DDAVP (Desmopressin)	Intravenous Injection-Factor catalyst/factor booster/factor precipitator
Grifols	Alphanate	Plasma-derived Clotting Factor
Octapharma	Wilate	Plasma-derived Clotting Factor
Takeda	Vonvendi	Recombinant von Willebrand Clotting Factor

For those who have been using Stimate for platelet disorders, their healthcare provider can make appropriate recommendations.

In addition to the factor products available on the market, there is an IV (intravenous) form of DDAVP which has been in use since 1978. This product can be administered prior to surgeries, dental work or other procedures. It is usually done in a hospital or clinic, but some consumers can be trained to do this at home. It will up to the healthcare provider or HTC to make that recommendation.

Lastly, there are two oral medications that can be used either alone or with one of the above treatments to control bleeding:

MANUFACTURER	PRODUCT	TYPE
Akorn	Amicar (amniocaproic acid - oral solution and tablets)	Oral Solution and Tablets
Ferring Pharmaceuticals	Lysteda (tranexamic acid tablets)	Tablet

The year 2020 has been a year of adaptation and change for all of us. This is not the first time the bleeding disorder community has had to deal with a recall and more than likely it will not be the last. Just like everything else in 2020, we need to make the best of the situation. With help and guidance from a medical team, patients and their family will make it through.

References: NHF - MASAC Medical Advisory #427: Stimate Recall Update, 10/19/2020; CSL Behring Urgent: Voluntary Product Recall, 8/11/2020; Current Products on the Market – Dateline Special issue 2019-2020; Benson, Gary, et al. Diagnosis and care of patients with mild haemophilia: practical recommendations for clinical management. Blood Transfusion, 11/16/2018

Thank You

TO OUR CORPORATE PHARMACEUTICAL AND BIOTECH SPONSORS!

Without the generous support from the following companies who invest needed resources into our community nonprofits, we wouldn't be able to serve individuals and families living with a bleeding disorder in the capacity we do. While 2020 presented challenges, the following programs were able to happen much in thanks to the support from our sponsors.

ADVOCACY EDUCATION

Silver Takeda

Bronze Genentech
Pfizer

GENE THERAPY ATTITUDES SURVEY

Platinum BioMarin
Spark Therapeutics

LEARNING CENTRAL

Silver BioMarin
Novo Nordisk

Bronze Sanofi Genzyme
Takeda

ADVOCACY LEADERSHIP COUNCIL

Platinum Sanofi Genzyme

Gold Pfizer

Bronze Genentech
Spark Therapeutics

HELPING HANDS

Platinum Novo Nordisk

PATIENT FLY-IN

Platinum Sanofi Genzyme

Gold Genentech

Silver CSL Behring
Pfizer
Spark Therapeutics
Takeda

POLICY & GOVERNMENT RELATIONS INTERNSHIP

Platinum Takeda

PROJECT CALLS

Gold Sanofi Genzyme

Silver Genentech
Takeda

TEAM RESILIENCE

Platinum CSL Behring
Sanofi Genzyme

Gold CVS Health
Medexus

Bronze Spark Therapeutics
Novo Nordisk

PROGRAMS

Diamond— CVS Health
All Programs Sanofi Genzyme
Takeda

Platinum BioMarin—*Blood Brotherhood*
Novo Nordisk—*Blood Sisterhood*

Gold CSL Behring—*Blood Sisterhood*
Novo Nordisk—*Families*

Silver Genentech—*Families*

POLICY & GOVERNMENT RELATIONS INTERNSHIP

Gold Genentech
Sanofi Genzyme
Takeda

Bronze CSL Behring
Pfizer

IXINITY[®]
coagulation factor IX
(recombinant)

**Discover more
about IXINITY[®]**

Visit IXINITY.com

**MEDEXUS
PHARMA**

Aptevo BioTherapeutics LLC, Chicago, IL 60606
IXINITY[®] [coagulation factor IX (recombinant)] and any and all Medexus Pharma, Inc. brand, product, service and feature names, logos, and slogans are trademarks or registered trademarks of Aptevo BioTherapeutics LLC and Medexus Pharma, Inc. in the United States and/or other countries.
©2020 Medexus Pharma, Inc. All rights reserved. CM-FIX-0112

Thank You

TO OUR 2020 MEMBERS!

Our organization thrives on the spirit and energy of community members from across the country and are able to assist, educate and advocate for so many due to the support from our membership. Beyond the nonprofit organizations and corporate members that you see listed, we'd like to thank the many individuals and families who were official 2020 members.

MEMBER ORGANIZATIONS

We're pleased to have partnered with the following 50 Member Organizations this year to serve the bleeding disorders community:

Alaska Hemophilia Association
Arizona Bleeding Disorders
Asociación Puertorriqueña de Hemofilia y Condiciones de Sangrado (Puerto Rico)
Bleeding Disorder Foundation of Washington
Bleeding Disorders Alliance Illinois
Bleeding Disorders Alliance of North Dakota
Bleeding Disorders Association of Northeastern New York
Bleeding Disorders Association of the Southern Tier
Blood Bond Bleeding Disorder Network
Central California Hemophilia Foundation
Connecticut Hemophilia Society
Eastern Pennsylvania Hemophilia Foundation
Florida Hemophilia Association
Gateway Hemophilia Association
Great Lakes Hemophilia Foundation
Hemophilia Alliance of Maine

Hemophilia Association of New Jersey
Hemophilia Association of New York, Inc.
Hemophilia Association of the Capital Area
Hemophilia Foundation of Arkansas
Hemophilia Foundation of Maryland
Hemophilia Foundation of Michigan
Hemophilia Foundation of Minnesota / Dakotas
Hemophilia Foundation of Northern California
Hemophilia Foundation of Southern California
Hemophilia of Indiana
Hemophilia of Iowa
Hemophilia of North Carolina
Hemophilia of South Carolina
Hemophilia Outreach of El Paso
Lone Star Bleeding Disorders Foundation
Louisiana Hemophilia Foundation
Mary M. Gooley Hemophilia Center
Midwest Hemophilia Association
New England Hemophilia Association

New York City Hemophilia Chapter
Northern Ohio Hemophilia Foundation
Oklahoma Hemophilia Foundation
Pacific Northwest Bleeding Disorders
Rocky Mountain Hemophilia & Bleeding Disorders Association
Sangre de Oro, Inc., Bleeding Disorders Foundation of New Mexico
Snake River Hemophilia & Bleeding Disorders
Southwestern Ohio Hemophilia Foundation
Tennessee Hemophilia & Bleeding Disorder Foundation
Texas Central Bleeding Disorders
United Hemophilia Foundation
Utah Hemophilia Foundation
Virginia Hemophilia Foundation
Western Pennsylvania Chapter of NHF
Wisconsin Bleeding Disorders Network

CORPORATE PHARMACEUTICAL AND BIOTECH MEMBERS

Bayer
Sanofi Genzyme
Spark Therapeutics
uniQure

SPECIALTY PHARMACY CORPORATE OR 340B HTC MEMBERS

Accredo
BioTek reMEDys
CVS Specialty
Nationwide Children's Hospital

Enroll or renew your membership for 2021 today!

Visit www.hemophiliated.org/membership today; membership dues are just \$35 and go a long way in strengthening our organization.

Utah Hemophilia Foundation

Utah Hemophilia Foundation, located in Salt Lake City, has been serving the bleeding disorders community in Utah since 1959. They currently provide resources and programs for around 400 families, including programs for men, women, children and families.



“Utah is a very tight knit community,” said Jan Western, Executive Director for UHF. “There is great support and camaraderie whenever our community gathers. New members are always welcomed, embraced and mentored in our great community.”

Utah’s “Everybody’s Here” program is one of their most popular programs. Families with bleeding disorders gather for the purpose of learning from one another with open-ended family activities that allow members to get to know one another and “check in” with each other.

FACTS ABOUT UHF

400 APPROXIMATE
MEMBERS SERVED BY UHF

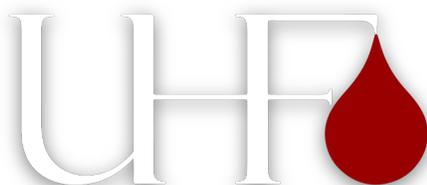


**PROGRAMS FOR MEN,
WOMEN, CHILDREN, FAMILIES**

SPECIAL SERVICE



**FINANCIAL ASSISTANCE
FOR MEDICAL EXPENSES
RELATED TO TREATMENT**





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

Visit Facebook @HemophiliaCoRes