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HEM AWARE

The Bleeding Disorders Magazine

 NATIONAL HEMOPHILIA FOUNDATION | hemaware.org



ADVICE AND TIPS
TO HELP FAMILIES
NAVIGATE THE EARLY
YEARS OF THEIR
CHILD'S DIAGNOSIS

**Parents
Helping
Parents**

p.22



BeneFix[®]
Coagulation Factor IX (Recombinant)
 Room Temperature Storage
 *BeneFix was approved February 11, 1997.



Scan to register
and stay informed.

BeneFix has been supporting individuals with hemophilia B for 25 years—and our support continues



ONCE-WEEKLY PROPHYLAXIS
AND **ON DEMAND** WITH
INDIVIDUALIZED DOSING



25 YEARS OF
CLINICAL EXPERIENCE



20 TRIALS, INCLUDING
MORE THAN **1500 PATIENTS**



**99% OF COMMERCIALY
INSURED PATIENTS**
HAVE ACCESS TO BeneFix

1997

BeneFix becomes **the first** recombinant factor IX (rFIX) treatment for hemophilia B approved by the US Food and Drug Administration (FDA).

2020

The FDA approves **BeneFix** **once-weekly** prophylactic use in addition to its on-demand indication.

2022

On February 11, BeneFix proudly became the only rFIX **supporting individuals with hemophilia B for 25 years.**

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.

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

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

Are you up to date on the latest?

Talk to your doctor or register at benefix.com/sign-up to stay informed.

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-0464-14.0, revised September 2021.



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to struggle to get answers
and adequate treatment.**

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Photography by Elyse Butler





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In Focus

PRESIDENT AND CEO LEONARD A. VALENTINO, MD



**We're
committed to
giving patients
and families
the tools
they need to
manage their
health.**

Parents Helping Parents

Did you know that the National Hemophilia Foundation was founded in 1948 by parents of a young boy with hemophilia? When Robert and Betty Jane Henry's son, Lee, was born, the life expectancy of a person with the disease was about 24 years. The Henrys were determined to do everything they could to make life better for their son.

Today, nearly 75 years later, we're committed to giving patients and families the tools they need to manage their health, make informed decisions about their care and live healthy, productive lives.

This kind of information is especially important for parents who learn, often unexpectedly, that their newborn has a blood or bleeding disorder. As a doctor who has treated many people with hemophilia and

von Willebrand disease, I know how overwhelming this can be for parents.

Our cover story features advice for parents of newly diagnosed children from parents who have been in their shoes and know exactly what they're going through. These seasoned parents share their best tips for navigating day-to-day living with a child who has a bleeding disorder—including the best ways to advocate for your child, how to prepare for possible emergencies and where to turn when you encounter billing and insurance problems.

When you read these parents' stories, you'll see a common thread. For many families, their connection to their local NHF chapter has proved to be an invaluable source of resources, community and support. To find the chapter nearest you, visit hemophilia.org/chapters.

Online | hemaware.org

National Hemophilia Foundation: hemophilia.org
Facebook: facebook.com/NationalHemophiliaFoundation
Twitter: twitter.com/NHF_Hemophilia
Instagram: instagram.com/nhf_hemophilia
YouTube: youtube.com/NHFvideo

Advocacy: hemophilia.org/advocacy
Steps for Living: stepsforliving.hemophilia.org
Victory For Women: victoryforwomen.org
Better You Know: betteryouknow.org



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Healthy Start

STATS, FACTS AND NEWS YOU CAN USE

Stress Busters

We asked you on social media, "What do you do to relieve stress and anxiety?"
Here are some of your answers:



I listen to music and read novels

—@HRUSHIKESHMANKAR_1701



HYBRID_UGP/UNSPASH

Meditation

—@RSTARKS54

Crazy dance

—@_LINDSAYREYES

*Knowledge is power!
The more I know, the more
comfortable I feel.*

—@ABBBS_18

Exercise

—@LUCASTAYLOR

1970

*First patients
ever receive
gene therapy*

1997

*First rFIX
products
approved
by FDA*

1999

*First gene
therapy
trial in hem B*

2018

*Late-stage
trials for
gene therapy
in hem B
underway*



EVERY STEP HAS BEEN EVOLVING THE SCIENCE OF GENE THERAPY IN HEMOPHILIA B

We're working to make gene therapy a reality for you.

**HemEvolution**

Explore the advancing science behind gene therapy at [HemEvolution.com](https://www.HemEvolution.com)



Biotherapies for Life® **CSL Behring**

COVID-19 Vaccines Are Safe for People with Bleeding Disorders

Some people in the inheritable blood and bleeding disorders community have avoided the COVID-19 vaccine because they've heard that it may cause a rare clotting disorder, but people with bleeding disorders can safely get COVID-19 vaccines that are available in the US.

Scottish researchers found that the AstraZeneca COVID-19 vaccine is associated with a slightly increased risk of the autoimmune bleeding disorder immune thrombocytopenic purpura (ITP), affecting 11 people out of 1 million. ITP didn't specifically affect people with bleeding disorders, but some people who heard about the research are refusing to get vaccinated.

"This is a reason some patients have given as to why they do not want the vaccine," says pediatric hematologist Tung Wynn, MD, an associate professor in the Division of Pediatric Hematology/Oncology at the University of Florida.

"It is not possible for anyone to get the AstraZeneca vaccine here since it's not approved for use in this country."

One vaccine available in the US has been associated with thrombocytopenia syndrome (TTS), a very rare condition causing blood clots and low platelet levels. Fifty-four people out of 14 million who received the Johnson & Johnson/Janssen COVID-19 vaccine developed TTS. In April 2021, the US Food and Drug Administration (FDA) paused distribution of this vaccine for 10 days to investigate its association with TTS. In December, FDA revised its fact sheets about the J&J vaccine, but it didn't recommend against the vaccine for people with bleeding disorders.

"The rate of blood clots is much, much higher in people who develop COVID and get severely ill," Wynn says. "The vaccines are proven to be the best way to protect everyone from getting a severe infection from COVID-19."



Checking In with Chris Bombardier

A lot has happened since we featured Chris Bombardier in our Winter 2018 issue for becoming the first person with hemophilia to summit Mount Everest.

Soon after our cover story came out, *Bombardier Blood*—a critically acclaimed documentary about his climb—was released. He also became executive director of Save One Life, the nonprofit that he raised money for while climbing; got a master's degree in global health; and became a father (son Carter is 1). We recently chatted with Bombardier to learn more.

Q WHAT DO YOU DO AT SAVE ONE LIFE, WHICH HELPS PEOPLE WITH BLEEDING DISORDERS IN DEVELOPING COUNTRIES?

My role is making sure all our programs are operating well in the developing countries that we work in, and also making sure that we have the funds to do that. With the pandemic, that's been a little bit challenging, but we actually did better fundraising in both 2020 and 2021 than we were anticipating.

Q HOW HAS FATHERHOOD AFFECTED YOUR ADVENTUROUS SPIRIT?

It's true that I've definitely slowed down since Everest, but my wife and I were able to go hiking a bunch last year; during our hikes, we carried Carter in the backpack.

Q ARE YOU PLANNING TO WORK AGAIN WITH BELIEVE LIMITED, THE COMPANY THAT PRODUCED BOMBARDIER BLOOD?

Yes, we're collaborating on a seven-episode podcast for the five-year anniversary of Everest, and we're going to be announcing an Everest base camp trek as a fundraiser for Save One Life in spring 2023.



FOLLOW CHRIS ON INSTAGRAM
@adventures_of_a_hemo



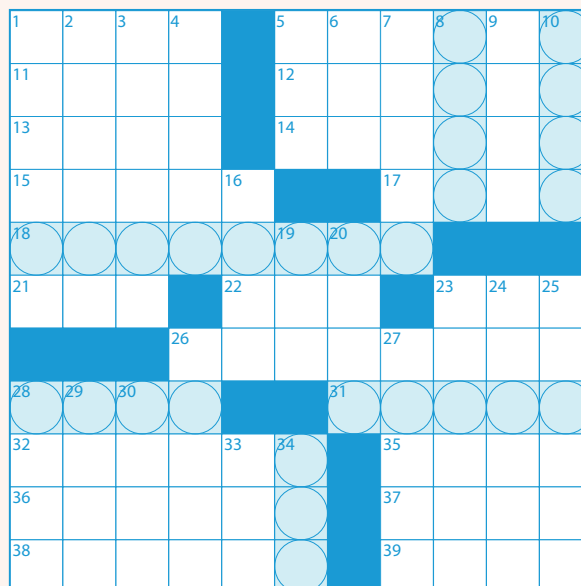
TOP TO BOTTOM: PMI IMAGES/GETTY IMAGES; COURTESY OF CHRIS BOMBARDIER.

CAN YOU SOLVE

FOR A DIFFERENT HEMOPHILIA A

TREATMENT?

Test your HEMLIBRA knowledge



ACROSS

1. Wine barrel
5. Deep fissures
11. Mideast gulf port
12. District
13. Ripped
14. Familiar with
15. Mean
17. Roost
18. The #1 prescribed prophylaxis for people with hemophilia A without factor VIII inhibitors*

*According to IQVIA claims data from various insurance plan types from April 2020 - May 2021 and accounts for usage in prophylaxis settings in the US.

21. Calendar divs.
22. Regret
23. Banquet hosts (abbr.)
26. International travel necessity
28. Check out the _____ treated bleeds data with HEMLIBRA
31. Number of dosing options HEMLIBRA offers

† Number of people with hemophilia A treated as of October 2021.

32. Small hole in lace cloth
35. Central Plains tribe
36. Melodic
37. Towering
38. Reduce
39. Spanish cheers

DOWN

1. Memorable, as an earworm
2. Devotee
3. Medical fluids
4. Prepare to propose, perhaps
5. PC's "brain"
6. Owns
7. Concert venue
8. See Medication Guide or talk to your doctor about potential _____ effects
9. Winter hrs. in Denver and El Paso
10. HEMLIBRA is the only prophylactic treatment offered this way under the skin

16. Pre-Euro currency in Italy
19. Subway alternative
20. Relax
23. Human
24. New Orleans cuisine
25. Mentally prepares
26. Collared shirts
27. Instagram post
28. Ardent enthusiasm
29. Brontë heroine Jane
30. Old Portuguese coins
33. Opposite of WNW
34. More than _____ thousand patients have been treated with HEMLIBRA worldwide†

SOLUTIONS

Across: 1. cask, 5. chasms, 11. Aden, 12. parish, 13. tore, 14. used to, 15. cruel, 17. nest, 18. HEMLIBRA, 21. yrs, 22. rue, 23. MCGs, 26. passport, 28. zero, 31. three, 32. eyelid, 35. Owe, 36. arose, 37. tall, 38. lessen, 39. oles
Down: 1. catchy, 2. adorn, 3. serums, 4. kneel, 5. CPU, 6. has, 7. arena, 8. side, 9. MSTs, 10. shot, 16. lire, 19. bus, 20. rest, 23. mortal, 24. Creole, 25. steels, 26. polos, 27. photo, 28. zeal, 29. Eyre, 30. Reis, 33. ESE, 34. ten

Discover more at [HEMLIBRA.com/answers](https://www.hemlibra.com/answers)

INDICATION & IMPORTANT SAFETY INFORMATION

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**.



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.

Your body may make antibodies against HEMLIBRA, which may stop HEMLIBRA from working properly. Contact your healthcare provider immediately if you notice that HEMLIBRA has stopped working for you (eg, increase in bleeds).

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

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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: 12/2021



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Genentech
A Member of the Roche Group

ASK A SOCIAL WORKER

Whenever you have a question or concern about your bleeding disorder, the social worker at your hemophilia treatment center (HTC) is an excellent resource to rely on.

For decades, HTC social workers have been helping patients and their families cope with life with inheritable blood and bleeding disorders. Social workers offer advice, practical information, mental health counseling and other support. They may help you cut through your health insurance company's red tape, get

answers from your health-care providers between appointments and help you feel more connected to the bleeding disorders community at large.

To gain insights into important topics that may affect your life, you can read our new Ask a Social Worker column online. Each month, different social workers from NHF's Social Work Working Group answer questions from members of the bleeding disorders community. They address issues including mental health, family life, medication and more.

VISIT: hemaware.org to read the Ask a Social Worker column.

VAL D. BIAS'S LEGACY

Here is more about Val D. Bias' extraordinary impact on the bleeding disorders community:

Years that Val was associated with NHF

27

Number of NHF chapters that launched while Val was CEO

17

Number of bleeding disorders groups in which Val held leadership positions

4

Years that Val worked with Camp Hemotion, a camp for kids with bleeding disorders in California

30

The Remarkable Legacy of Val D. Bias

On December 30, former NHF CEO Val D. Bias passed away at age 63. He served as CEO for nearly 12 years before retiring in 2019. Previously, as an NHF lobbyist, he influenced the passage of the Ricky Ray Hemophilia Relief Fund Act of 1998, which compensated people who contracted HIV from tainted blood products.



SEE: page 40 for more on Val's legacy.

TOP TO BOTTOM: KLAUS VEDFELT/GETTY IMAGES, GUERIN BLASK



AIMING HIGH
Garrett Hayes says Jason Fulton's story inspires him.

Helping to Realize a Dream

The first recipient of the Jason Fulton Memorial Scholarship plans for a career in healthcare

Garrett Hayes, now 23, grew up thankful that he was born in the era of modern medicine. Even with severe hemophilia A, he has lived a very active life. “I can do so many things that my predecessors in the community were unable to do,” he says. “Today’s treatment modalities are very safe and effective—a fact that’s not lost on me.”

One of those predecessors was Jason Fulton. He also had severe hemophilia A, and when Jason was only a teenager, he learned that the blood products he had taken to treat his disease had infected him with HIV. Despite this diagnosis, he continued to dive headfirst into his advocacy and studies—focused on hematology and pharmacy—and lived his life to the best of his ability. Tragically, Jason

died in 1995 at age 24 of complications from the HIV infection.

Today, Garrett’s and Jason’s lives have intertwined. Garrett recently received the first Jason Fulton Memorial Scholarship from the National Hemophilia Foundation (NHF), awarded to young leaders who demonstrate the same determination, passion and work ethic in support of the bleeding disorders community.

Karen Fulton Holine, Jason’s mother, says Garrett is the perfect candidate to receive the inaugural award. “We were pretty clear that we wanted someone with the same kind of ambitions as my son in terms of postgraduate work in public health and in medicine,” she says. “Not only does Garrett have those goals,

“Bringing the patient perspective to medicine is really valuable.”

he’s also very athletic and competitive, which my Jason was too, so that’s sort of the cherry on top for me.”

When Garrett learned he had been given this honor, he delved into Jason’s story. “I was taken aback by his perseverance,” says Garrett. “Unfortunately, some aspects of Jason’s story aren’t unique. So many people in our community had to shoulder a terrible burden in order for us to get to where we are today. But what is unique about Jason’s story is that he was able to shoulder this burden with such a good attitude and continue to follow his dreams of working in healthcare, and that’s something I aspire to do as well.”

The award should help Garrett do that. He is pursuing a master’s in public health at The University of North Texas Health Science Center at Fort Worth and plans to attend medical school. “I think bringing the patient perspective to medicine is really valuable,” he says.

The scholarship helps fund internships in public health, clinical and community settings for current and former members of NHF’s National Youth Leadership Institute (NYLI). “Giving young people the opportunity to build skills and professionalism and work as part of a team is so important,” says Garrett. “I hope to bring the abilities I’ve developed in the NYLI program to help my future professional endeavors.”

Karen could not be happier. “Garrett is just a charming young man, like Jason. And like my son, he’s clear about pursuing medicine and public health, and that just makes my heart sing. He is a perfect choice.”

—By Leslie Pepper



DONATE TO THE JASON FULTON MEMORIAL LEADERSHIP FUND: hemophilia.org/jasonfultonfund

SAY HELLO TO JAMES

He has hemophilia A and has gone through two major surgeries while keeping to his factor regimen with the support of his hemophilia care team

“RECOVERY WAS TOUGH,
BUT I LEARNED I HAD
MORE SUPPORT THAN
I THOUGHT POSSIBLE.”



Read stories like James' in
Hello Factor magazine:
BleedingDisorders.com





STRETCH YOUR THINKING

Inactivity can lead to lost strength and flexibility and raise the risk of falls.

5 Ways to Improve Your Balance

Falls are dangerous, especially for people with hemophilia. Here's how to protect yourself.

As we age, our risk of falling increases—our bodies lose strength, our vision and hearing decline, and some medications can make us feel woozy. Having a bleeding disorder only exacerbates the issue.

“With hemophilia, it’s common to lose range of motion in your ankles or knees” after repetitive joint bleeds, says Jennifer Newman, a physical therapist at the UNC Hemophilia and Thrombosis Center in Chapel Hill, North Carolina. “And that makes it more difficult to clear surfaces—even small ones—like catching your toe on an area rug or uneven flooring.”

The complications associated with falls are greater for people with hemophilia, too. Not only can a fall trigger a bleed, but it’s also more likely to result in a bone fracture, given that people with bleeding disorders tend to have low bone density.

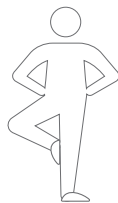
So, how can you protect yourself from a fall? Follow these five tips.



Eye Checks



Movement



Balance

If you have any near falls, tell your doctor or HTC provider.

1 HAVE YOUR EYES CHECKED

“If your vision is off at all, it will affect your balance,” Newman says.

Adults older than 60 should have a dilated eye exam every one to two years, but some people—such as African Americans and those with diabetes—might need one more frequently, according to the National Eye Institute. Talk to your doctor to see whether you’re at high risk.

2 INVEST IN GOOD FOOTWEAR

Wearing sturdy, well-fitting shoes can keep you from slipping and falling. Look for pairs that offer support and the cushioning you need for your joints.

“We rely heavily on sensations in our feet to assist with balance,” Newman says. “Always opt for shoes with an enclosed heel and adequate tread, and take extra care when walking on slippery or icy surfaces.”

3 MOVE MORE

Studies show that as many as 20% of people with hemophilia restrict their activities out of fear of falling, but inactivity leads to lost muscle strength and flexibility that makes people unsteady on their feet.

Newman recommends checking with your doctor

and the physical therapist on your hemophilia treatment center (HTC) care team before adopting any new exercise routine or activity but says walking and low-impact water aerobics are good places to start.

4 PERFORM BALANCE EXERCISES

There are several simple exercises you can do at home to improve your balance. Try this: Stand on one foot near a kitchen counter. Begin by holding on to the counter; then, let go. Next, add a distraction, such as brushing your teeth. Or, try closing your eyes. Balance on each foot with every exercise.

Always practice balance exercises when someone else is home. At the very least, keep your phone near you at all times in case you do fall.

5 REPORT NEAR FALLS TO YOUR PHYSICIAN

Almost falling now could lead to actually falling later. If you have any near falls, tell your doctor or HTC provider. He or she may recommend physical therapy to help you prevent a future fall or help you find a therapist who specializes in working with people with hemophilia.

—By Shelley Flannery



YOUR GUIDE TO STRENGTH TRAINING: Building muscle and strengthening bones can help you avoid a fall, but you might need a modified exercise program. Visit hemaware.org/mind-body/your-guide-strength-training

When Your Child Has a Fear of Needles

Four ways to make infusions easier for everyone

Many young children are afraid of needles, and many parents may have anxiety about giving their child infusions, especially in the beginning and when a child needs infusions several times a week. Fortunately, there are strategies to make the experience less stressful for all involved. Here, experts share four ways to combat needle nervousness:

DEMYSTIFY

Getting anxious about needle pokes can actually cause the veins to constrict, making the infusion process even harder, says Christi Humphrey, LCSW, a social worker at Hemophilia of Georgia. Educating your child about the process and why it's necessary can make infusions less intimidating.

"I suggest that parents talk about hemophilia throughout their week, not just at infusion time," Humphrey says. "Talking about the condition normalizes it. Families can also write their own story and ask the children to draw pictures about the infusion process. The more families are open to discussing hemophilia, what it looks like, and how it makes you feel, the less a child has a stress response to the condition."

PRACTICE

Jim Munn, RN, MS, program coordinator at the University of Michigan Hemophilia and Coagulation Disorders

"Talking about the condition normalizes it."

Program, stresses the value of hands-on experience for both kids and their parents. "Start to involve kids early in the process with having them assist with getting things ready for infusions, helping with pushing the medication into the vein once accessed, and completing the documentation of the infusion," he says.

Practicing giving needle pokes is especially important, says Carrie Starnes, a child life specialist at the Indiana Hemophilia and Thrombosis Center. She suggests using actual medical equipment—a tourniquet, alcohol pads, pressure dressings, and a butterfly or syringe without a needle—on a stuffed animal or doll. "This helps educate a child about the sights, sounds and sequence of the procedure."

DISTRACT

Find things that your child can do during the infusion process to keep their mind off it. "Distraction can be a very powerful tool for children and adults



BRING IT UP

Talking openly about infusions before they happen can counteract your child's fear.

as well," Starnes says. "If you can find something that your child likes and will engage in, then use it." She recommends blowing bubbles, counting, watching a video or playing games on a tablet device.

Physical interventions such as numbing creams, cold sprays and a product called Buzzy, a small vibrating device that helps reduce pain with infusions, are also useful.

CALM

Maintaining a calm environment during the infusion process helps reduce stress and relax the body. You might dim the lights and reduce noise in the room if your child finds that comforting, for example. A warm blanket and warm drink might also be helpful.

Humphrey also recommends using mindfulness techniques, such as imagining a favorite place or focusing on a mantra. "These all help to address the in-the-moment stress response to needles," she says.

—By Christina Frank



LEARN MORE: For more tips on infusion, visit stepsforliving.hemophilia.org/basics-of-bleeding-disorders/treatment-basics/infusion-sites



Help the hemophilia community by taking part in a research study

Sponsored by **B:OMARIN**

The aim of this study is to find out how common it is for people with hemophilia A to have existing antibodies against adeno-associated virus (AAV) and what it could mean for the development of future treatments.

By participating, you can help us understand how the body may develop antibodies against the naturally occurring AAV.

You may qualify for the **SAAVY (270-701)** study if:

- You are 18 years of age or older; and
- You have a diagnosis of hemophilia A

How does the study work?

- Participants will be asked to complete 2 blood draws and answer questions on a convenient mobile app.
- You'll receive compensation after each blood donation for your time and participation.
- No medication, therapy, or experimental procedures are part of this study.

What value will the results of the study bring to the hemophilia community?

Different types of AAV are frequently used in clinical trials for gene therapy. Understanding the presence of AAV antibodies will help guide researchers in developing innovative therapies for people with hemophilia A.

Scan the code
to sign up at
saavy-study.com



Hear what the experts are saying

Learn more about the value of this important research from NHF leadership and experts in the community.



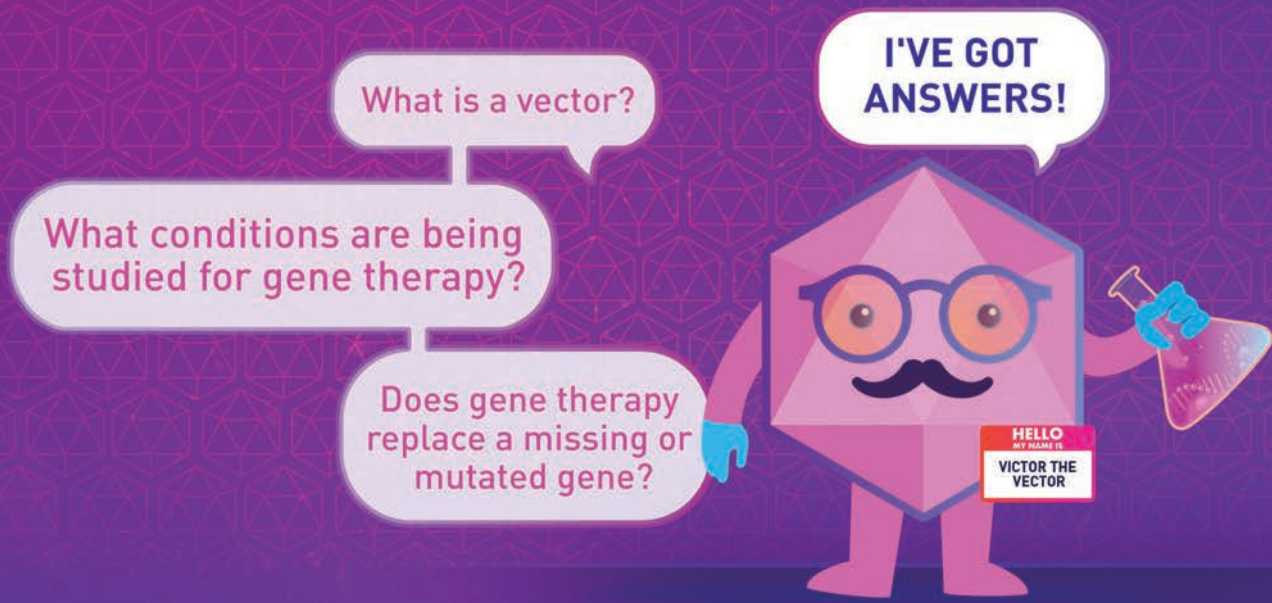
Kim Schafer,
MSN, NP-C
Nurse Practitioner,
Hemostasis and
Thrombosis Center,
UC Davis Health



Dawn Rotellini
Chief Operating
Officer, NHF

Watch the video at saavy-study.com

DO YOU HAVE QUESTIONS ABOUT GENE THERAPY RESEARCH?



DID YOU KNOW

There are **5,000 to 8,000 genetic conditions caused by a single gene mutation** (monogenic conditions). Hemophilia, Huntington's Disease, and Cystic Fibrosis are just a few of them. In fact, BioMarin has been researching monogenic conditions for close to 25 years. One way we show that commitment is through the *BioMarin Gene Therapy Learning Academy*, where we can help you better understand the possibilities of gene therapy research.



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at [HemDifferently.com](https://www.hemdifferently.com).

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**FILL 'ER UP**

Eating a variety of foods and nutrients keeps your gut happy and healthy.

Go with Your Gut

Keep your microbiome balanced for better health. Here's how.

A lot more goes on in your gut than you might realize. Trillions of microorganisms, including bacteria, viruses, fungi and parasites, call your gut home. When they live together in harmony in what is called the microbiome, they help keep you healthy.

“If the balance is good, we’ll have fewer infections and illnesses, and our immune systems will be optimal,” says Emily Ostrowski, a registered dietitian at Sparrow Health System in Lansing, Michigan, who works with patients at the Michigan State University Center for Bleeding and Clotting Disorders. “When it’s not in balance, it can affect everything, from your heart to your mental health.”



EASY WAYS TO ADD FRUITS AND VEGGIES: Getting the recommended daily amounts of good-for-you foods isn't hard, but most of us still struggle with it. Find helpful tips at hemaware.org/mind-body/eating-rainbow

“If the balance is good, we’ll have fewer infections and illnesses, and our immune systems will be optimal.”

START WITH YOUR DIET

What you eat plays the biggest part in gut health. In a large study published in 2021, researchers reported that poor diets—those featuring dairy-based desserts and processed foods, including high-fat meats such as bacon—promoted gut microorganisms linked with high cholesterol, high blood sugar and inflammation. These combine to increase a person’s risk of diabetes, heart disease and other chronic diseases. Other research ties gut inflammation to depression.

A mostly plant-based diet of fiber-rich fruits and vegetables, fish, nuts and seeds supports gut microorganisms that may lower the risk of chronic disease, researchers have discovered.

ADJUST AS NEEDED

Kevin Tomlin is learning that now. Until recently, the 45-year-old focused on a high-protein meaty diet to serve his passion of weightlifting. But that raised his bad cholesterol and his risk of fatty liver disease, so he began a more diverse diet of greens and fiber. Plant-based protein, from sources such as lentils and beans, is beneficial for his gut and helps him build muscle in the gym.

Tomlin, who has hemophilia A and lives in Lansing, says liver disease runs in his family. His new diet will help reduce his

risk. “I’m ready for this new challenge,” he says.

“The influence of gut health on bleeding disorders is not well known, but we do know that one form of vitamin K, known for its role in blood clotting, is produced by gut bacteria,” Ostrowski says. She says anyone with a chronic disease, including hemophilia, will benefit from a gut-healthy diet.

“You want to eat a variety of foods and nutrients to keep your gut in balance and maintain the diversity of its microorganisms,” she says. “Eat fiber-rich foods and limited amounts of processed foods.”

CONSIDER PROBIOTICS

The gut microbiome also can be maintained with probiotics. These are good bacteria that help balance the disease-causing ones. Probiotics can be found in yogurt—make sure the label says it contains live and active cultures—as well as fermented foods, such as sauerkraut, kimchi, kefir and kombucha.

Probiotics also come in supplement form, but Ostrowski says more research is needed to determine their effectiveness and the best strains for the desired clinical outcomes. Talk to your doctor or dietitian before adding supplements to your diet.

—By Matt McMillen

Women and Alcohol

Excessive drinking can be dangerous—particularly for those with bleeding disorders

Media and memes convey that regular alcohol consumption is normal adult behavior, especially as we cope with a growing number of stressors. Women in particular are seeking solace in alcohol—one recent study showed an increase in alcohol misuse among women during the pandemic. This has experts worried.

“It is a disturbing trend, especially because we know that alcohol impacts women more severely than it does men in a lot of different ways,” says social worker Nick Szubiak, MSW, LCSW, of NSI Strategies.

Research shows that women experience greater physical and behavioral health consequences from alcohol than men do, and those with bleeding disorders should be especially mindful of the risks.

RISKS OF ALCOHOL

Alcohol affects men and women differently. Women's bodies have proportionally less water and more fat than men's bodies. Because water dilutes alcohol and fat holds onto it, women's organs have greater alcohol exposure over time. Plus, women have less of the enzyme alcohol dehydrogenase, which metabolizes, or breaks down, alcohol.

In the short term, excessive alcohol can lead to alcohol poisoning, risky sexual behavior, miscarriage and stillbirth, and injuries. Violence is also connected with excessive alcohol use.

Over time, drinking too much alcohol can cause high blood pressure, heart disease, stroke, liver disease, digestive problems, various cancers, mental health issues such as depression and anxiety, and job- or family-related problems.

For people with bleeding disorders, alcohol use is concerning because of the risks tied to liver disease and high blood pressure as well as falls and injuries, since alcohol affects the blood's ability to clot.

WHAT IS TOO MUCH?

The Dietary Guidelines for Americans advise that women consume no more than one drink (see sidebar) per day.

Excessive drinking for women can be binge drinking (four or more drinks on an occasion) or heavy drinking (eight or more drinks per week), says the Centers for Disease Control and Prevention.

WHAT TO WATCH FOR

Professionals use specific criteria to diagnose substance use disorders, which

range from mild to severe. Signs that drinking is becoming a problem include:

- Feeling that you should cut down
- Feeling annoyed when others criticize your drinking
- Feeling guilty about your drinking
- Drinking first thing in the morning to steady your nerves or combat a hangover

Some people can cut back and make healthy changes by themselves, but if you struggle on your own, help is available.

“It's not because you're a failure. It's not because you're not trying hard enough,” Szubiak says. “It may be because you have a substance use disorder. And that means you may need some outside help.”

—By Stephanie Conner

What Is 1 Drink?

- 12 ounces of beer (5% alcohol content)
- 8 ounces of malt liquor (7% alcohol content)
- 5 ounces of wine (12% alcohol content)
- 1.5 ounces of 80-proof (40% alcohol content) distilled spirits or liquor

KNOW

Help is available for those struggling with alcohol.



FIND RESEARCH-BASED INFORMATION
ON DRINKING AND YOUR HEALTH:
rethinkingdrinking.niaaa.nih.gov

LIFE HAPPENS

AND ADVATE WILL BE THERE WHEN IT DOES

ADVATE has over 15 years of treatment experience in the real world and provides clinically proven bleed protection* for patients with hemophilia A.¹


[Antihemophilic Factor (Recombinant)]
REAL LIFE. REAL BLEED PROTECTION.*

AdvateRealLife.com

*In clinical trials, ADVATE demonstrated the ability to help prevent bleeding episodes using a prophylaxis regimen.

Not an actual patient.

Prophylaxis with ADVATE prevented bleeds¹

The ability of ADVATE to treat or prevent bleeds was evaluated in a clinical study using a standard prophylaxis, pharmacokinetic driven prophylaxis, and on-demand treatment. 53 previously treated patients (PTPs) with severe to moderately severe hemophilia A were analyzed. For the first 6 months of the study, patients received on-demand treatment. For the following 12 months of the study, patients received either standard prophylaxis every 48 hours or a pharmacokinetic-driven prophylaxis every 72 hours. The primary goal of the study was to compare annual bleeding rates between those receiving prophylaxis treatment and those receiving treatment on-demand. The number of bleeds per year for the 2 prophylaxis regimens were comparable.

- Those patients experienced a median of 1 overall bleed per year on either prophylaxis treatment vs 44 overall bleeds per year with on-demand treatment.¹ This represented a 98% reduction in overall bleeds per year.
- Zero bleeds were reported in 42% of patients (22 out of 53 patients) during 12 months on prophylaxis

¹Median is the middle number in a group of numbers arranged from lowest to highest.

ADVATE Important Information

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

DETAILED IMPORTANT RISK INFORMATION

Who should not use ADVATE?

Do not use ADVATE if you:

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

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

What should I tell my HCP before using ADVATE?

Tell your HCP 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.

What should I tell my HCP before using ADVATE? (continued)

- Are or 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 important information do I need to know about ADVATE?

- You can have an allergic reaction to ADVATE. Call your HCP 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.
- Do not attempt to infuse yourself with ADVATE unless you have been taught by your HCP or hemophilia center.

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. Talk with your HCP to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

What are possible side effects of ADVATE?

- 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, and rash. Tell your HCP about any side effects that bother you or do not go away or if your bleeding does not stop after taking ADVATE.

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

Please see Important Facts about ADVATE on the following page and discuss with your HCP.

For Full Prescribing Information, visit www.ADVATE.com.

Reference: 1. ADVATE Prescribing Information.

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[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.

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US-ADV-0030v1.0 02/20



K

KNOWLEDGE

IS

POWER

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**FOSTER
CONFIDENCE**

"I've taught my son from a very early age to own the fact that he has hemophilia and be empowered by it," says Lauren Holomalia, photographed with her son Mason by Elyse Butler near their home in Wai'anae, Hawaii.

Learning that your child has an inheritable blood or bleeding disorder can cause stress and anxiety for many parents. Your mind can race from what life will look like for them and how you'll care for them to what the future holds. HemAware asked parents for their best advice on what has helped them navigate their children's bleeding disorders in the first few years after diagnosis.

WRITTEN BY RITA COLORITO
PHOTOGRAPHY BY ELYSE BUTLER,
LIZ PUTNAM & STACY HOWELL

LAUREN HOLOMALIA

Wai'anae, Hawaii

SON MASON, 10

Diagnosed with hemophilia before birth

When my grandmother found out, she told my mother that I was going to have a hard life. My uncle who had hemophilia died in his early 50s. My grandmother remembers the struggles of taking care of him as a child. Their comments really scared me.

My son's hematologist was very encouraging in letting me know that technology and treatments have come a long way. She also offered to speak to my family. That's what really turned them around on the whole situation. Through being educated, they became much more supportive of me.

If you're in a similar situation, educating your family members is important. Invite them to chapter meetings and NHF family events so they can learn about hemophilia.

I've also taught my son from a very early age to own the fact that he has hemophilia and be empowered by it. We embrace any opportunity to educate others so that it's not a shameful thing. It's not a secret. I know some people are more private, but that has helped my son really be more aware of things that he shouldn't do and know what he can do safely. It's all about being aware and building confidence.

Talk to your child's school. Go in calmly and explain the situation. When you're comfortable, they're comfortable. It helps defuse the panic they feel when they hear "bleeding disorder." I'm a teacher, so I created a binder that I gave to the school nurse. It has a picture of him, his name, date of birth and very specific steps to follow in case of an emergency. I learned very early on that not all hospitals or doctors know very much about hemophilia or how to infuse.

JODI RUDELL

Cheyenne, Wyoming

DAUGHTERS HANNAH, 23, and EMMA, 19

Diagnosed with von Willebrand disease at ages 12 and 8, respectively

Our older daughter's fifth period is what triggered the diagnoses for our family. I have it too, and my husband was a carrier, but we didn't know it.

Ultimately, what helped us was reaching out and connecting with a local chapter and community through our hemophilia treatment center (HTC) in Denver. That's my biggest piece of advice to new parents: Connect with your HTC and local chapter. They have the best resources.

Be involved with your bleeding disorder community. Without it, I don't know where I'd be. While my best friends are my support system in life, they don't understand bleeding disorders. And that's the difference. It's a huge stress to have a chronic, rare disorder. My "bleeder moms" understand what I'm going through more.

Our medical bills were outrageous. I, fortunately, had never been in a position to ask for money before but I found that our chapter had a patient assistance program. So, I wrote a five-page letter explaining what was going on. When Hannah had that heavy period, nobody knew what was happening. There was not a hematologist in Cheyenne at the time. Hannah was in the ICU here and had to be transferred to the ICU at Children's Hospital in Denver, which is connected to the HTC there. I asked for \$500 for the ambulatory fees, and the chapter gave us more, which was absolutely helpful.

Cristel Peake

Northfield, New Hampshire

Son Seth, 17

Diagnosed with hemophilia A before birth, and diagnosed with an inhibitor at age 2

I didn't know very much about how inhibitors occurred. It took a few years to learn he had inhibitors. He's had issues with venous access, joint surgeries, you name it. He uses a wheelchair now. Had I known how serious it could become, I would have monitored him more for inhibitor formation.

I didn't know to press the doctors to check for inhibitors. That's something I think parents should be aware of. If caught early, new treatment options for inhibitors can reduce the risk of serious joint issues.

Always follow your instincts. You know your child best. If the doctor doesn't believe you, press it and fight.

Make sure to have your other children tested, even if they're older and their symptoms are unclear. My 26-year-old daughter, Jess, was recently diagnosed with mild hemophilia A.





+ GET INVOLVED
"Connect with your HTC and local chapter. They have the best resources," says Jodi Rudell, photographed with her husband James and daughter Hannah by Liz Putnam near their home in Cheyenne, Wyoming.

+
**NORMALIZE
INFUSING**

"Showing our daughter videos of other kids getting infused took the fear out of it for her," says Samantha Javorka, photographed with her daughter Daphne by Stacy Howell near their home in Summerville, South Carolina.



“

To handle the unknowns of emergency room visits, ask to set up a tour of the ER.

”

SAMANTHA JAVORKA

Summerville, South Carolina

DAUGHTER DAPHNE, 8

Diagnosed with von Willebrand disease type 2B and thrombocytopenia at age 2

Learning to treat with prophylaxis at home was the best piece of advice we got. At the time [of diagnosis], that was uncommon for people with von Willebrand disease. We had a really open-minded doctor in Michigan, where we moved from, who was up on all the current treatments and who supported our decision. In the past six months, NHF has released new guidelines for people with von Willebrand disease saying that there are positive results with prophylactic treatment.

Get advice from someone who lives with the condition every day—a parent or a child. When my daughter had pain from the car seat strap over her port, parents who experienced similar situations gave us different suggestions.

Showing our daughter videos of other kids getting infused took the fear out of it for her. We’ve also learned that sucking on a lemon wedge helps. There’s something about the connection between your brain that focusing on the sour takes priority over focusing on the pain.

To handle the unknowns of emergency room visits, ask to set up a tour of the ER. We took our daughter when she was not having a bleed, so she could meet the nurses, see the facility and feel comfortable if she ever needed to go in. The lead nurse updated my daughter’s online chart so now it flashes red with information to call our hematologist. Before that, if we went to the ER, I would have to call my doctor and hand over my cellphone to make sure they called. I’d overheard nurses joking before, “It’s just a bloody nose. Who comes to the ER for a bloody nose?” But the flashing screen validates us.

MAGGIE CARRUTH

Jackson, Wyoming

Son **TEDDY TAYLOR, 17 months**

Diagnosed with severe hemophilia A at birth



the most helpful thing for us after our son’s diagnosis was meeting with other hemophilia families. Our local NHF chapter connected us to several parents who had older boys. It helped to talk to them about what kind of treatment options worked for them, and the pros and cons between getting a port for prophylaxis or using medication subcutaneously.

Staying off Google helped. I’m not joking. It can create unnecessary anxiety. Talk to the professionals—the nurses and doctors who treat hemophilia—instead.

One piece of advice that has stuck with me: They don’t necessarily bleed faster. They just bleed longer. I had this vision there would be blood gushing everywhere. The reality is that it takes much longer for their blood to clot, depending on the severity.

Learning as much as I can about my son’s condition has helped me advocate for him. If we do find ourselves in an emergency room, we know what should happen. We have a care plan. We’re prepared.



CRISTINA RUIZ

West Allis, Wisconsin

SON YADIR, 10

Diagnosed with severe hemophilia B before birth

iI had already been involved with the hemophilia community because of my nephews. The community support helped me so much during those first few months and years after diagnosis. The hemophilia moms would meet once a month for breakfast and talk about anything on our minds.

One thing parents need to keep in mind: Even if your child has other family [members] with hemophilia, they all bleed differently.

Keep the lines of communication open and check in with your kids. Some kids don’t let you know when they’re hurting or bleeding. We tell our son, “It’s OK to feel what you’re feeling.” I think he thinks it’s a bad thing that he needs to get infused. We tell him it’s not his fault; it’s just something we have to do.

Managing financial logistics, like health insurance, can be challenging. The information I got through the hemophilia community helped. I also let my hematologist know if there were ever any billing and insurance issues.

Advocate for your child, especially in the ER. They just aren’t very aware of what they should be doing. If you see something that doesn’t seem right or doesn’t look right, you should speak up.

STRIVING *to be* SEEN *and* HEARD



BLACK WOMEN WHO HAVE INHERITABLE BLOOD AND BLEED
THEIR JOURNEY—FROM DIAGNOSIS TO TREATMENT TO ACCE



“
IT IS IMPORTANT TO
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PHYSICAL, EMOTIONAL AND
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MANAGING OUR CARE.

”

—CONNIE MONTGOMERY

NG DISORDERS OFTEN FACE OBSTACLES ALL ALONG
SS TO HEALTH INSURANCE AND MORE

WRITTEN BY ANDREA COLLIER
PHOTOGRAPHY BY MATTHEW LAVERE,
NICOLE LOEB & KEVIN TITUS
ILLUSTRATION BY JOHN B. HANSEN

+
**STRONG
ADVOCATE**

Ronia Cole was
photographed by
Matthew LaVere
at her home in
Redford Township,
Michigan.

“
NEVER GIVE UP AND CONTINUE
TO FOLLOW YOUR DREAMS!
”

—RONIA COLE

Ronia Cole, 52, of Redford Township, Michigan, has lived with a bleeding disorder since she was a toddler. “I’d take a fall, bruise easily and my mom couldn’t understand why,” she says. Her pediatrician had no answers or real treatment for her. Many doctors struggled to find ways to treat her prolonged nosebleeds and excessive gum bleeds.

When she turned 5, Cole was diagnosed with von Willebrand disease (VWD). During the mid-1970s, this inherited bleeding disorder was extremely rare among African Americans. In fact, her family was told she may have been the first in her state to be diagnosed with VWD after undergoing tests at the University of Michigan Medical Center.

“After my diagnosis, my mom began to build a better rapport with our family doctor, my hematologist, and my ear, nose and throat specialist, in order to help me maintain a normal hemoglobin,” Cole says. In her mid-20s, Cole struggled with heavy periods and had to seek various ways to control excessive bleeding episodes.

It is estimated that nearly 3 million Americans are living with VWD, and it is the most common bleeding disorder among women and girls in the US. “I tell other women with bleeding disorders, ‘You are not alone. We have many resources to help you during your hardest times. Never give up and continue to follow your dreams!’” says Cole.



MULTIPLE BARRIERS

Tammuella Chrisentery-Singleton, MD, chief of hematology for the Louisiana Center for Advanced Medicine in New Orleans, says it’s common for Black women with bleeding disorders to struggle to get answers.

“Overall, it’s challenging to diagnose a bleeding disorder because primary care physicians don’t have significant training in diagnosing and treating them,” says Singleton. But for Black women, there are also significant cultural and systemic factors that can complicate diagnosis and treatment. It’s a complex and multifaceted issue.

“As an example, if you’re white, nine times out of 10 you’re going to a doctor regularly, and that doctor knows you and understands you from a cultural standpoint,” she says. “But a lot of Black people often don’t feel comfortable going to the doctor because they haven’t really established a relationship with a provider.

“As a Black physician, I have a bit of an advantage when I’m talking to other Black people, many of whom are feeling disenfranchised from the medical system,” Singleton says. When she is working with people who have limited financial resources, such as patients who are on Medicaid, she understands that they need some extra help.

“I know that many of them may not fully understand what’s happening, so I’ll spend some extra time explaining and trying to provide some additional resources,” she says, adding that those kinds of connections often are not made for Black women who have bleeding disorders.

Connie Montgomery, 51, of Pawleys Island, South Carolina, has lived with

factor VII deficiency since birth, but she didn’t get a diagnosis until her mid-30s. “I went through my adolescent years with heavy, painful periods and often bled through my clothes at school,” she says. Her mother took her to pediatricians, but she says nobody listened to them. “And culturally, it was difficult—it was stressed that you didn’t talk about it outside of the home. My mother said that you just deal with it,” she says.

As Montgomery got older, she continued to seek help from several ob/gyns about her pain and heavy periods. “Doctors would tell me that it can’t be that bad,” she says.

Even after having two cesarean section births and heavy postpartum bleeding for a month and a half, Montgomery says she was repeatedly misdiagnosed or not diagnosed at all. “When I was 36 years old and was in a terrible car accident, I came across a compassionate emergency room doctor who listened to what I had to say and helped me get a real diagnosis through a hematologist.”

Keri L. Norris, PhD, the National Hemophilia Foundation’s (NHF) vice president of health equity, diversity and inclusion, says that “it can be difficult to diagnose or treat chronic conditions—including blood or bleeding disorders—for anyone, but women of color are statistically more likely to be faced with these challenges.”

According to Norris, studies have shown significant disparities in diagnosis, treatment and care for more common chronic diseases. “To eliminate diagnosis and treatment disparities in chronic diseases such as blood and bleeding disorders, access and affordability need to improve for all aspects of healthcare, including prevention,” she says.



FIGHTING FOR COVERAGE

Once they finally get the correct diagnosis, many women struggle to afford the necessary treatments and prescriptions for bleeding disorders. Montgomery says the IV medication she desperately needs costs \$350,000 a year. She has learned to maneuver through



the obstacle course of providers and insurers to make sure she gets what she needs. “I have had to learn how to work with both my insurance company, my husband’s insurance company, the pharmacy that provides the medication and a benefits coordinator to make certain that I get my medication.”

During the times when she couldn’t afford what she needed, she would go to charitable organizations, such as Patient Services, Inc. “When I had to involve them, I had to get them and get everybody else on a conference call at one time and say, ‘Hey, I’m the patient. These are my insurance companies, and this is the benefits coordinator at the pharmacy. This is what I know.’”

Montgomery says it has been stressful but worth it. “I’m going to do whatever is necessary within the legal lines of the law to get the necessary medication I need to live and enjoy my family,” she says with conviction.

Unfortunately, says Montgomery, many Black women are so used to being told no that they don’t have the skills to get access to the care they need. Montgomery has since become a voice for women who are struggling to find equitable and affordable care. She serves as a co-chair of the Medical University of South Carolina Diversity and Inclusion Patient and Family Advisory Task Force.

According to Norris, “These health disparities can only be removed by starting with increasing equitable prevention efforts, and from there working to reduce costs and reform the healthcare system at large.” To achieve this reality, “NHF is ready and willing to work together with community members and partners, beginning with increased education around diagnosis and management, and advocacy for increased medication affordability.”

Montgomery has also helped her daughter, Connor Graham, 24, navigate the waters of doctors, medications and care for her own bleeding disorder. Recently, Graham graduated from

an advanced degree program in South Carolina and moved to Boston for a job as a communications specialist. She has been diagnosed with factor V Leiden and as a carrier for factor VII deficiency. “Mine started manifesting during puberty, with heavy and long periods,” she says. Later, she also started having nosebleeds and microbleeds in her joints after physical activities. “Because I am a carrier for factor VII deficiency, and I don’t have the disorder like my mother, I am not eligible for specific medications and treatments,” Graham says.

She has learned to manage by being mindful and taking preventive steps, especially for joint bleeds. Graham golfs for recreation when she can, and uses compression, icing and elevation to reduce the chances of knee bleeds from all the walking. She also takes birth control pills to help reduce the length and severity of her periods. “I take a progesterone-only pill right now, due to my factor V Leiden diagnosis,” she says. “I feel fortunate to have my mother as an advocate who understands the challenges we face. Most women like me don’t have that,” Graham says.



MEETING MULTIPLE NEEDS

According to Singleton, the challenges that Black women face in managing the diagnosis and treatment of their bleeding disorders are a part of the continuum of health equity issues that they face in all aspects of access to quality healthcare. “The average person, especially individuals of color who come from low-income families and individuals with low academic levels, is struggling to have access to services and the medications they need,” she says.

Norris agrees. “The difficulty in diagnosis, treatment and care for chronic conditions and women of color is, of course, a microcosm of the larger issues plaguing the American healthcare system,” she says. Simply put, if a health outcome is seen to a greater or lesser extent between populations, there is disparity. “From implicit bias to unhidden biases, much work must be done to rid the healthcare system of its troubling and lingering roots in systemic racism,” Norris says.

“We must take a close look at Black women of all ages who have a need for support in their complex healthcare needs,” adds Montgomery. Before retiring, she worked in the healthcare industry. “It is important to understand what the needs are beyond just expensive medicine—including mental, physical, emotional and definitely spiritual needs—when it comes to managing our care.”

Last year, Montgomery and her daughter created a webinar for the Medical University of South Carolina called “Hear with Your Eyes and See with Your Heart: Compassionate Healthcare,” aimed at doctors, nurses and social workers. “I feel like my own healthcare outcomes would have been different if they had listened to me all along,” she says.

As for Black women who are living with blood or bleeding disorders, Montgomery advises them to be fearless in reaching out for help. “It’s important for women to know that their diagnosis does not have to define or limit their possibilities in life,” she says.



FAMILY TIES

Connie Montgomery (opposite page) was photographed by Kevin Titus at her home in Pawleys Island, South Carolina, and her daughter Connor Graham was photographed by Nicole Loeb at her home in Boston.



I FEEL FORTUNATE TO HAVE MY MOTHER AS AN ADVOCATE WHO UNDERSTANDS THE CHALLENGES WE FACE. MOST WOMEN LIKE ME DON'T HAVE THAT.

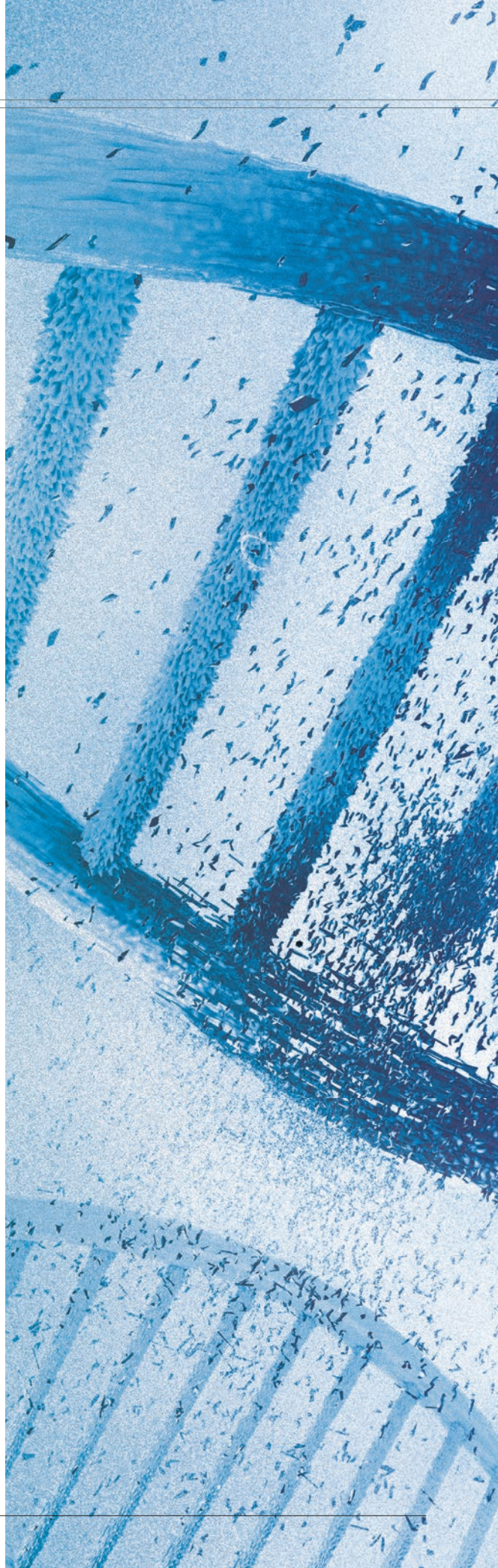


—CONNOR GRAHAM

Gene Therapy: Where Are We Now And What's On The Horizon

THE NATIONAL HEMOPHILIA FOUNDATION'S 16TH WORKSHOP ON NOVEL TECHNOLOGIES AND GENE TRANSFER FOR HEMOPHILIA, HELD IN NOVEMBER, ADDRESSED THESE QUESTIONS AND MORE.

WRITTEN BY MARCI L. HARDY, PHD



FUNDAMENTALS OF BLEEDING DISORDERS

Blood contains proteins (clotting factor) that help stop bleeding after an injury or surgery. Hemophilia, a rare blood disorder, involves low amounts of either factor VIII or factor IX clotting factor, which causes a person's blood to not clot correctly—leading to nosebleeds, blood in the urine or stool, bleeding issues after an injury or surgery, or unexplained bleeding, pain, swelling or tightness in the joints. Extremely low levels of clotting factor indicate a more severe diagnosis and possible serious bleeding-related health problems.

The current preferred treatment for hemophilia is to replace the missing factor or to administer medicines that circumvent the need for the missing factor. People can learn to administer the treatment (clotting factor concentrates) by injecting them into a vein (infusing) to treat periodic bleeding episodes. Their healthcare provider may also prescribe regular prophylactic infusions to try to prevent bleeding episodes.

+ MANY UNANSWERED QUESTIONS

While much has been learned about gene therapy, a lot more research remains to be done.

Since 1996,

leading immunologists, clinicians and researchers discuss the latest findings and obstacles in the search for possible gene therapies. They have concluded that while much has been learned, much more needs to be done.

SCIENCE DOES NOT STOP FOR A PANDEMIC

In November, co-chairs Glenn Pierce, MD, PhD, and David Lillicap, MD, along with the National Hemophilia Foundation's (NHF) Medical Programs and Information Department, convened the 16th Workshop on Novel Technologies and Gene Transfer for Hemophilia in Washington, DC. The hybrid community of online and masked, socially distanced attendees from around the globe presented their latest bleeding disorder gene therapy research and challenged one another through thoughtful discussions.

THE ORIGINS OF GENE THERAPY

Once researchers discovered that DNA was the source of genetic inheritance and diseases, they began exploring the possibility of replacing a damaged gene. Today, more than 40 years of advancements have turned the hypothetical into probable for several medical conditions. As genetic sequencing became more affordable and available, scientists identified genetic markers for numerous conditions. However, the technology needed to safely deliver nucleic acid cargo inside cells has lagged behind the technology development used to identify the disease-associated genes.

Naturally occurring viruses (such as cold viruses) can deliver genetic material into cells, "tricking" them into making more copies. Scientists have taken advantage of this and devised adaptations to deliver gene therapy using "vectors" based on viruses. However, some viral vectors have produced serious adverse events, including cancer.

Gene therapy for hemophilia involves replacing the mutated F8 or F9 gene with a functioning copy of the gene so that the instructions for making clotting factor are not broken. Several techniques have been explored to determine which ones show the most promise with the fewest side effects, and scientists are completing research to evaluate the safety and efficacy of the potential treatments. Researchers have also begun exploring gene therapy for von Willebrand disease (VWD). While most gene therapy researchers hope to completely cure bleeding disorders, many recognize that even improving the body's ability to make clotting factor may lessen disease severity and give people a better quality of life.

The most important aspects of gene therapy are efficacy and safety, followed by effectiveness and cost. The distinction is between how the treatment works in a controlled, ideal condition such as the research studies discussed at the workshop (efficacy) versus possible side effects that might occur once the therapy is used in the real world (effectiveness). These fundamentals are paramount to understanding what researchers have learned and where gene therapy is headed.

WORKSHOP HIGHLIGHTS

The studies presented at the workshop that involved bringing a novel therapy from the lab to trials to the clinic were complex. The research is occurring in mice, primates and dogs. The presenters discussed gene therapy targeted at liver cells (hepatocytes) and bone marrow (intraosseous).

Although multiple vehicles (vectors) have been evaluated for targeting the liver for gene therapy in people with hemophilia, most of the discussion was about adeno-associated viruses (AAV) and lentiviral vectors.

1 ADVANCEMENTS IN AAV RESEARCH

AAVs are small viruses that infect some primates, including humans. AAVs produce a mild immune response rather than cause disease, and different AAVs have affinity for different tissues. As of 2019, AAVs have been used in more than 250 gene therapy clinical trials across a variety of diseases, including hemophilia.

In laboratory and animal models, researchers identified the main mechanism leading to the formation of genome particles generated by the host cell environment. The scientists concluded that their results provide new clues for how to improve both vector efficiency and safety.

Another researcher shared that the differences between rodents and humans have become apparent in AAV clinical trials. Some findings in mouse models were replicated in humans, but others have poorly translated. Researchers still need to better understand how human liver cells (hepatocytes) can replace the mouse functional tissue of an organ (parenchyma). They also discussed limitations and strategies to improve preclinical AAV gene therapy mouse models. In addition, the researchers discussed their ongoing efforts to improve the ability of AAV vectors to target and infect specific cells.

Also, studies looking at AAV-transduced liver tissue under a microscope found that after a single infusion of gene therapy (AAV5-hFVIII-SQ), clinically significant factor VIII levels and reduced annualized bleeding occurred. Analyzing the therapy's effect on the livers demonstrated no structural changes and mild fat buildup in the livers of four of five participants.

2 ADVANCEMENTS IN LENTIVIRAL VECTORS

Researchers also explained that a human immunodeficiency virus 1 (HIV-1)-derived vehicle is the most used lentiviral vector in gene therapy. However, the production of lentiviral vectors has been challenging and low-yielding, and thus not cost-effective. One trial using lentiviral gene therapy with a corticosteroid immune-suppression regimen in nonhuman primates demonstrated efficient and well-tolerated liver gene transfer, with an improved therapeutic index for factor VIII.

Another study delivered lentiviral vectors into bone marrow, targeting factor VIII. Gene therapy delivery to the shin or hip bone

chers in gene therapy have been convening every one to three years to discuss genetic cures for bleeding disorders. Each time they have come together, more work needs to be done.

of four dogs with hemophilia A resulted in factor VIII in the platelets of the treated dogs but no factor VIII expression in their plasma. All the treated dogs had reduced annual bleeding in the two years after gene therapy.

Researchers also evaluated gene transfer therapy into humanized mice's stem cells that give rise to other blood cells. Injecting the lentiviral vectors into the bone marrow of humanized mice led to factor VIII platelet expression. The results indicate safety and efficacy in canine and humanized mice, adding evidence for translating the model to clinical trials.

3 GENE THERAPY RESEARCH FOR VWD

While gene therapy research for hemophilia is in advanced stages, other bleeding disorders have not received the same attention. Von Willebrand disease research, in particular, has faced hurdles.

The complementary DNA (cDNA) that encodes von Willebrand factor (VWF) exceeds the compatible physical size for AAV delivery. Also, VWF is best expressed in the thin layer of cells that line the interior surface of all blood vessels (endothelial cells) rather than in liver cells (hepatocytes). Researchers are working on mechanisms to overcome these issues. Their use of a specific vector (dual-hybrid AAV9) to overcome size limitations resulted in long-term expression of VWF in lab studies of human endothelial cells and in VWF-deficient mice. However, they noted that the current VWF levels are too low to be clinically relevant and that additional engineering is needed to improve expression levels of VWF.

PULLING IT ALL TOGETHER

Many lessons were learned during the workshop. However, many unknowns remain, including safety concerns regarding the liver, whose job is to regulate most chemical levels in the blood by producing proteins for blood plasma and cholesterol to help carry fats through the body. The liver also produces bile, which helps carry away waste and break down fats in the small

THE GENETICS OF HEMOPHILIA

Because hemophilia is an inherited disorder, understanding genetics is important to understanding the potential impact of gene therapy. Genes provide instructions for making proteins. A mutation in the gene that gives the directions for clotting factor is typically passed from one generation to the next and may result in the blood not clotting properly. You inherit one chromosome from each parent. Females have XX chromosomes, and males have XY. The gene for factors VIII and IX are both on the X chromosome, so males will have symptomatic disease if their lone X chromosome has the hemophilia mutation.

intestine during digestion. Therefore, the researchers' focus on maintaining liver health is to ensure the liver does not become severely damaged and can properly continue breaking down harmful substances and excreting the byproducts for removal from the body.

Several researchers at the workshop agreed that, rather than being a cure, gene therapy will likely reduce the severity of disease, requiring less factor therapy and improving quality of life. However, some people may have an immune response to the virus used for gene therapy, which would diminish the therapy's effectiveness. The researchers also discussed how concerns about an unwanted immune response to the vector led to the incorporation of immunosuppression therapy into some clinical studies. In addition, the dose required to elicit the desired response is variable. The researchers agreed that work remains to safely translate the animal research to human applications.

A father with the mutation passes it to his daughters when he gives his lone X chromosome to them. He would not pass the mutation to any sons, as the Y chromosome he passes does not have the mutation. A female may have one X chromosome with the mutation and one without, so her unaffected X chromosome may signal enough clotting factor that she might not realize she is a carrier. A mother who has one affected X chromosome has a 50% chance of passing it to her offspring. It is rare for a mother to have two affected X chromosomes, but if she does, she will pass an affected X chromosome to 100% of her offspring.

For many workshop attendees, the most impactful presentation was a panel made up of two physicians, an NHF staff member and a graduate student, all of whom are familiar with healthcare and either live with or are a caregiver for a person with a bleeding disorder. The four panelists shared hopes and concerns for future treatments. Their questions reminded the room that some people may eventually be able to improve their quality of life significantly and either stop using or reduce their reliance on clotting factor products with the use of gene therapy—but that many people will not qualify for these treatments because of health concerns, the way their body responds, or simply a lack of access and the treatment's high cost.

This somber note included a plea for continued research into treatment pathways beyond gene therapy to help those with bleeding disorders live their best lives possible.

“Most of life’s work is in the getting there.”

— Val Bias



Sanofi honors *Val Bias* for a lifetime of courageous leadership and dedication to people living with bleeding disorders. From summer camps to the halls of Congress, Val championed the patient voice with a tireless drive to build bridges to a better future for the community he loved. His legacy lives on in those who were privileged to know Val and share his commitment and vision.

sanofi



In Memoriam: Val D. Bias

The former National Hemophilia Foundation CEO spent decades inspiring the inheritable blood disorders community

NHF was deeply saddened by the sudden passing of former CEO Val D. Bias in December 2021. Val was a uniquely skilled community builder who left an indelible mark on the inheritable blood disorders community in the US and internationally. Here and on the following pages, community members share their tributes.



“
Val was a partner and a friend. Throughout his leadership and support for hemophilia advocacy, he always wore his trademark smile.

”
—CESAR GARRIDO

COMMUNITY IN ACTION

NHF UPDATE



“Val had a great laugh, so big and full of life. I miss you so much my friend and blood brother.”

—JONATHAN HILL



“
It was an honor to know Val and experience his work.
”

—ELIZABETH VARGA



“Val was a longtime and effective advocate for those living with hemophilia and HIV. He was also a community leader, gentleman and a friend. His lifetime of work will not be forgotten.”

—BRIAN O'MAHONY

1970

First patients ever receive gene therapy

1997

First rFIX products approved by FDA

1999

First gene therapy trial in hem B

2018

Late-stage trials for gene therapy in hem B underway

EVERY STEP HAS BEEN EVOLVING THE SCIENCE OF GENE THERAPY IN HEMOPHILIA B

We're working to make gene therapy a reality for you.

HemEvolution

Explore the advancing science at [HemEvolution.com](https://www.HemEvolution.com)



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“I’ve been honored to meet many outstanding people in my life, and Val was one of the greatest. He sacrificed his time, energy and health for people with hemophilia. The world is a better place thanks to him.”

—TONY MAYNARD



“So very sad to hear of the passing of my friend and fellow hemophilia advocate, Val Bias. He was a true force to be reckoned with, a survivor, and I take great pleasure in looking back on my time with him over the years. So lucky to have called him friend.”

—ALEX BORSTEIN

“
*Val was a leader,
friend and a
mentor. The
world has one less
hero in it.*

”

—PATRICK JAMES LYNCH



.....
WANT TO SHARE YOUR MEMORIES OF VAL?
EMAIL THEM TO: communications@hemophilia.org

BIAS & BORSTEIN: PATRICK McMULLAN

To the National Hemophilia Foundation, its chapters, and community members:

Our Hemophilia Team at Pfizer stands by your side as we continue to feel the loss of Val Bias. We share his memory and remain uplifted by his legacy through our continuous commitment to support positive health outcomes for those affected by hemophilia.

Through the creation of the “Steps for Living” program in collaboration with NHF, we remain honored to have had the opportunity to recognize Val as our partner in developing patient and caregiver education for all life stages. As the founding sponsor of the North American Camping Conference for Hemophilia Organizations (NACCHO), which focuses on the work of bleeding disorder summer camps, we’re proud to see how this specialized conference continues to evolve, helping to ensure that the needs of camp associations throughout the country are met. Val was a tremendous advocate for camps, realizing early on their potential to bring life-changing experiences to patients and their families. We are humbled to have helped Val realize his vision, both for NACCHO and camping organizations at large. We are grateful to have had the opportunity to recognize Val as a collaborative partner and trusted voice in these initiatives, plus additional educational and advocacy activities through the years. It brings us joy to realize that many of the foundational initiatives that we worked on closely together still carry positive impact in the community today. Our hearts continue to pause for his family, those dear to him, and you.

Our commitment to supporting the hemophilia community remains forever inspired, thanks to you, Val.



“ *Advocacy for me is not what has been accomplished but **who has been inspired by the effort.** There is no case where I did anything without ‘us.’*

Advocacy is about the many and never the few or the one.

*If I have motivated anyone, **helped young people to believe in themselves and their potential,** then that is all the accomplishment I want. ”*

Val Bias
1958-2021

*Celebrating a lifetime of fierce, visionary service
to the bleeding disorders community.*



Transformation Through Collaboration

A partnership with the Haemophilia Foundation of Nigeria has led to meaningful progress on many fronts

The World Federation of Hemophilia (WFH) collaborates with its national member organizations to make lasting, sustainable care possible in countries around the world. One example of this collaboration is in Nigeria, where the Haemophilia Foundation of Nigeria (HFN) is working with the WFH to drastically improve the lives of people with bleeding disorders there.

DIAGNOSIS AND TREATMENT AT LAST

In 2005, WFH board member Megan Adediran relaunched the HFN, which had been dormant for nearly 30 years. At that time in Nigeria, diagnosis and treatment for people with bleeding disorders were nonexistent. The HFN's collaboration with the WFH began with the WFH Cornerstone Initiative. The clear goals of this program—and the support provided—led to tangible change. With this infrastructure in place, more product donations were making their way to the country through the WFH Humanitarian Aid Program to be administered by trained healthcare providers. For the first time, people with bleeding disorders in Nigeria could count on effective treatment for their conditions.

Akintunde Richard, a young man from Abeokuta, benefited from treatment product donations and WFH training. Before his diagnosis, he experienced neck stiffness, high blood pressure and intense headaches—which led to him being scheduled for brain surgery before

CHANGE MAKER

Executive Director Megan Adediran's persistence has enabled the HFN to go far.

a physician properly diagnosed him. “I was able to reach out to Megan Adediran, and she and the doctor immediately took action to help,” says his grateful mother, Akintunde Collins.

SUCCESSFUL TWINNING PROGRAMS

The WFH Twinning Program partnership between the National Hospital Abuja and the Rush University hemophilia treatment center (HTC) in Chicago helped raise awareness of bleeding disorders among healthcare professionals and provided training opportunities for laboratory technicians, nurses and physicians. A second pairing a few years later, between the University of Nigeria Teaching Hospital in Enugu and the Children's Hospital of Philadelphia HTC, led to the identification of 30 people with bleeding disorders, the creation of educational resources for nurses and the training of more than 25 healthcare workers.

“Our twinning with the National Hemophilia Foundation in 2012 was our breakthrough, and the knowledge we gained from that relationship took our

organization from level 1 to 5 on the ladder of advocacy, fundraising and organizational development,” Adediran says.

In the ensuing years, the HFN invested in local chapters and built the national infrastructure to advocate for sustainable treatment and care. It reached a major milestone recently when the Nigerian Ministry of Health began including bleeding disorders in national policy. This policy aims to ensure early detection and medical care, the creation of a national structure to disseminate information and the promotion of comprehensive care to improve the management of inherited bleeding disorders.

Adediran's boldness and persistence have helped the HFN get this far, but no one can lead such profound transformation alone. For nearly 60 years, the WFH has been working to create sustainable change for the global bleeding disorders community. The progress is not always immediate, but it is transformational.



For nearly 60 years, the WFH has been working to create sustainable change.



LEARN MORE: Visit wfh.org

**FINISHED!**

Maggie and Jeanne Carruth pose for a celebratory selfie.

Running for Teddy

Mother-daughter team Jeanne and Maggie Carruth ran the New York City Half Marathon for the National Hemophilia Foundation, far surpassing their fundraising goal

Jeanne Carruth remembers the day last year when she saw a post on Facebook about running the 2022 United Airlines New York City Half Marathon to raise money for the National Hemophilia Foundation (NHF).

Ever since her daughter Maggie's first-born child, Teddy, was diagnosed with severe hemophilia A shortly after he was born in January 2021, she and her husband have been educating themselves about the disease and looking for ways to support Maggie and her family.

"Maggie was a D-I (Division I) runner, and I also run. So I sent it to her and said, 'Hey, I think we should do this and raise money,' not really thinking too hard

about it. And then 10 seconds later, she sent me an email that she was signing up, and so that was that. We were running," Jeanne says.

Maggie says she was initially daunted by the fundraising aspect (each runner is required to raise at least \$1,500), but she needn't have worried. Combined, the two ended up raising more than \$17,000 for NHF.

Jeanne and Maggie both live in Jackson, Wyoming, and they say their close-knit community there was instrumental in helping them raise as much as they did. "My brother is a professional hockey player, and he donated a jersey and a goalie stick for a raffle, and

my dad owns a hockey team in Jackson, and they sponsored a 50/50 raffle, and those brought in the biggest donations," Maggie says. "And then the rest came from family and friends."

"It really was amazing how many people donated, even friends of my siblings and teammates of my son," Jeanne says. "It was a very, very humbling experience."

The fundraiser also served as a way to educate people about hemophilia. "Many people in our community had little to no knowledge of bleeding disorders, so the range of understanding was huge," Jeanne says. "So this was a great opportunity to not only raise money but also raise awareness."

The day before the race in March, NHF hosted a welcome brunch for the runners and their guests. While Maggie was unable to attend because she couldn't arrive in New York until later that evening, Jeanne says the brunch was an important part of the weekend for her. "Being able to meet other members of the community and hear their stories, it really opened up a lot of resources for me and for Maggie, too," Jeanne says.

The next morning, Maggie and Jeanne were both excited to run. "We told everyone at the hotel and basically anyone who would listen why we were running the half-marathon," Maggie says. The two started the race together, but Maggie quickly picked up her pace and ended up finishing about 40 minutes ahead of her mom.

"She was there at the finish line for me, and I saw her smiling face and of course I started crying," Jeanne recalls.

Looking ahead, Maggie says she's sure that she and her family will continue to participate in fundraising events for NHF. "Hopefully, as he gets older, Teddy will run, if that's something he wants to do, and be involved in other events, too. Active families is something we're all really passionate about."

—By Donna Behen



IF YOU'RE INTERESTED IN RUNNING WITH NHF, EMAIL: nhfgo@hemophilia.org

Stronger Together

Throughout the year, NHF chapters provide vital programs that connect people within the bleeding disorders community and raise much-needed awareness of critical issues. Here are details of a few successful chapter initiatives.



TRAINING MATTERS

The new dental program is open to dentists worldwide.

WEST VIRGINIA CHAPTER OF THE NATIONAL HEMOPHILIA FOUNDATION: NEW DENTAL PROGRAM

The West Virginia Chapter of the National Hemophilia Foundation has launched a much-needed dental program. "We've heard many stories of members being refused treatment by dentists when they hear of their condition," chapter Director Chelsea Hilty says. "But even for dentists who want to treat them, specific training can make a difference."

The chapter's new training program can be used both by dentists local to the chapter and by anyone else around the world who wants to learn. Dentists are already signing up, and the reaction has been gratifying.

"We have members who stopped even trying to go to the dentist because of the bad experience of being turned away," Hilty says. "They are excited that now they can be paired with dentists who want to help them and will be trained."

Learn more about the West Virginia Chapter of the National Hemophilia Foundation: wvnhf.org



WANT YOUR EVENT FEATURED IN CHAPTER ROUNDUP?

We are not able to highlight all programs due to an overwhelming response. However, we appreciate your submissions and look forward to highlighting more in future issues. If you would like to feature your chapter's event in a future issue, email donna.behen@manifest.com.



ASOCIACIÓN PUERTORRIQUEÑA DE HEMOFILIA Y CONDICIONES DE SANGRADO: SPOTLIGHT ON ADVOCACY

The Asociación Puertorriqueña de Hemofilia y Condiciones de Sangrado, which became an official NHF chapter in 2020, has started in-person educational programs. It also plans to hire an advocacy and public policy coordinator for six months with the intention of establishing a strong advocacy program called *Alza tu Voz* (Raise Your Voice), says Anthony Llanes Rodríguez, the chapter's executive director. The chapter is considering adding another part-time employee as well.

"We're also in the process of creating directories of senators and legislators to serve as an advocacy contact list," Llanes Rodríguez says. "But we don't want to just be a chapter to serve people treating their bleeding disorders, but to help people in Puerto Rico who may not know they have the disease to recognize symptoms so they can get diagnosed and tested."

Llanes Rodríguez says he is talking with the Puerto Rico Department of Health about a massive media campaign to get the word out about symptoms of bleeding disorders.

Learn more about the Asociación Puertorriqueña de Hemofilia y Condiciones de Sangrado: hemofiliapr.org

NEW ENGLAND HEMOPHILIA ASSOCIATION: MENTAL HEALTH TASK FORCE

The New England Hemophilia Association (NEHA) has created a Mental Health Task Force. The first phase called for forming multiple working groups to address areas including educational programming, communications and the development of an internal crisis plan. NEHA also recently hired a new mental health professional coordinator, thanks to a grant from the National Hemophilia Foundation.

Earlier this year, the task force expanded to add a working group focusing on denial of treatment issues for substance use disorder and mental health issues, says Nancy Messina, the association's board president. "Even pediatric patients are finding it tough," she says. Adds Executive Director Rich Pezzillo: "We're looking into what issues it could hit on, from regulatory to advocacy. We're diving into it on a national level and in our six-state area to figure out why it's happening and what solutions are possible."

Learn more about the New England Hemophilia Association: nehemophilia.org





CONTROL
For people with type 3 VWD, a new drug may help.

VWD Drug Approved for Routine Prophylaxis

A new treatment regimen for the rarest and most severe form of the disease

In January, the US Food and Drug Administration (FDA) approved Vonvendi® for routine prophylaxis to reduce the frequency of bleeding episodes in adults with severe type 3 von Willebrand disease (VWD) receiving on-demand therapy. Type 3 VWD is the most severe form of the disease.

“VWD is a complex disease where both patients and providers may experience stress and uncertainty due to the unpredictable disease course and limited treatment options,” said Miguel A. Escobar, MD, professor in the Department of Pediatrics and Internal Medicine at the McGovern Medical School at The University of Texas Health Science Center at Houston and an investigator in the Vonvendi prophylaxis study, in a press release issued by Takeda, the drug’s manufacturer. “A prophylactic treatment option may allow for greater disease control and the potential to enhance the standard of care.”

Vonvendi is the only recombinant von Willebrand factor therapy on the market. The new FDA approval represents an expanded indication for the drug, which was first approved in 2015 for the on-demand treatment and control of bleeding episodes in adults with VWD.

VWD is an inherited disorder that affects women and men equally. It is caused by a deficiency or defective function of von Willebrand factor, one of several proteins in the blood that are needed for proper blood clotting. Because of this defective function or deficiency, blood cannot clot effectively in people with VWD. Vonvendi is an infused product designed to replace the body’s missing or dysfunctional von Willebrand factor.

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**ALL IN THE FAMILY**

Rose Bender (shown with her parents, at left, and mom and grandmother, below) says her family inspired her to get more involved with advocacy efforts.

Finding Purpose in Advocacy

Inspired by my parents, I've made it a priority to help others with bleeding disorders

When I was 9 months old, I bled so severely that I needed a whole blood transfusion, cryoprecipitate and fresh frozen plasma just to stay alive. This was in the late '90s, and at the time, nobody thought that women could get hemophilia, so I was diagnosed with von

Willebrand disease. A few years later, my parents learned that I actually had hemophilia A, which, already a rare disease, is even more rare in females.

When I was growing up, people said kids with hemophilia needed to be protected. We weren't supposed to be

active because we could bleed and hurt ourselves. My parents were very much of the bubble-wrap mindset. Before I started kindergarten, my mom sent my dad and me to the playground to do "climby" lessons. She wanted to make sure I knew how to use the equipment without hurting myself. It's not that I'd never been on a playground before, but I'd never been on one without them hovering over me.

I'm the first in my family to have hemophilia, so my parents made it their mission to learn everything they could about the condition. They also became very involved in advocacy. My father served as the chair of the Board of Directors of the National Hemophilia Foundation (NHF), and my mother volunteered for many committees,

COMMUNITY IN ACTION

MY JOURNEY



including co-founding the New York City Hemophilia Chapter and serving as its first president. They are amazing role models.

I always knew that I had something different about me, and meeting other people who were like me offered a sense of community. Groups like the NHF helped me to do that, and I knew I wanted to help others feel that as well. So advocacy work became one of my life's purposes. I did a lot of fundraising for the NYC Hemophilia Walk, and I spoke at New York City Hemophilia Chapter events. One of my favorite activities was being a counselor at Camp Little Oak and the New England Hemophilia Association (NEHA) Family Camp.

After graduating from college in 2019, I moved to Seattle. Unfortunately, with COVID-19, I haven't had many opportunities to volunteer in person, but I was

lucky enough to be a virtual counselor at NEHA Family Camp. That was tons of fun getting to see all the campers and their families singing the same songs that I sang around the campfire when I was there in person.

I also recently joined the NHF's Medical and Scientific Advisory Council (MASAC). The group includes doctors, scientists and other medical professionals, and its objective is to develop treatment guidelines that healthcare providers of all kinds can follow. I serve as a female patient representative to help raise concerns that are unique to women.

This new venture is a great opportunity to help improve the lives of patients through these recommendations. I'm thrilled to represent the interests of women with hemophilia and to make sure that our voices are heard.

—By Rose Bender, as told to Leslie Pepper



Reconnect at #NHF2022

August 25-27
Houston, TX + Online

hemophilia.org/bdc



IMPORTANT FACTS ABOUT ELOCTATE® [ANTHEMOPHILIC FACTOR (RECOMBINANT), FC FUSION PROTEIN]

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

WHAT IS ELOCTATE?

- ELOCTATE is an injectable medicine that is used to help control and prevent bleeding in people with Hemophilia A (congenital Factor VIII deficiency).
- Your healthcare provider may give you ELOCTATE when you have surgery.

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

- You should not use ELOCTATE if you are allergic to ELOCTATE or any of its other ingredients. Tell your healthcare provider if you have had an allergic reaction to any Factor VIII product prior to using ELOCTATE.
- You can have an allergic reaction to ELOCTATE. Call your healthcare provider or emergency department right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash or hives.
- Your body can also make antibodies called, “inhibitors,” against ELOCTATE. This can stop ELOCTATE from working properly. Your healthcare provider may give you blood tests to check for inhibitors.
- If you have risk factors for developing abnormal blood clots in your body, such as an indwelling venous catheter, treatment with Factor VIII may increase this risk.

THE MOST COMMON SIDE EFFECTS OF ELOCTATE INCLUDE: joint pain, general discomfort, muscle pain, headache, and rash, in previously treated patients, and Factor VIII inhibition, device-related blood clotting, and rash in previously untreated patients. Talk to your healthcare provider for more information and about any side effect that bothers you or does not go away.

WHAT SHOULD I TELL MY HEALTHCARE PROVIDER BEFORE STARTING ELOCTATE?

Tell your healthcare provider about all your health conditions, including if you:

- Have or have had any medical problems.
- Are taking any prescription and non-prescription medicines, including over-the-counter medicines, supplements, or herbal medicines.
- Are pregnant or planning to become pregnant. It is not known if ELOCTATE may harm your unborn baby.
- Are breastfeeding. It is not known if ELOCTATE passes into breast milk and if it can harm your baby.

AFTER STARTING ELOCTATE:

- If your bleeding is not controlled and you experience a lack of clinical response to Factor VIII therapy, call your healthcare provider right away.
- Medicines are sometimes prescribed for purposes other than those listed here. Do not use ELOCTATE for a condition for which it was not prescribed. Do not share ELOCTATE with other people, even if they have the same symptoms that you have.

HOW SHOULD I USE ELOCTATE?

ELOCTATE should be administered as ordered by your healthcare provider. You should be trained on how to do infusions by your healthcare provider. Many people with hemophilia A learn to infuse ELOCTATE by themselves or with the help of a family member. See the booklet called “Instructions for Use” packaged in your ELOCTATE for directions on infusing. If you are unsure of the procedure, please ask your healthcare provider.

QUESTIONS?

The risk information provided here is not comprehensive. To learn more, talk about ELOCTATE with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at www.eloctate.com or 1-855-MyELOCTATE (693-5628). You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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

For Hemophilia A patients,

YOU USE YOUR JOINTS MORE THAN YOU THINK.

That's why you need a Factor VIII treatment you can Count On to protect* you and your joints from bleeds.

1.6

MEDIAN OVERALL
BLEEDS PER YEAR†

0

MEDIAN JOINT
BLEEDS PER YEAR†

#1

PRESCRIBED FACTOR VIII
FOR PROPHYLAXIS IN US‡

*ELOCTATE has been proven to help patients prevent bleeding episodes using a prophylaxis regimen.

†In the A-LONG study, 164 previously treated adult and adolescent males with severe hemophilia A ages 12-65 received ELOCTATE either every 3 to 5 days, once weekly, or on demand.

‡#1 prescribed based on HTC reported data as of September 2020.

INDICATION AND IMPORTANT SAFETY INFORMATION

INDICATION

ELOCTATE[®] [Antihemophilic Factor (Recombinant), Fc Fusion Protein] is an injectable medicine that is used to help control and prevent bleeding in people with Hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ELOCTATE when you have surgery.

IMPORTANT SAFETY INFORMATION

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

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

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

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

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

If you have risk factors for developing abnormal blood clots in your body, such as an indwelling venous catheter, treatment with Factor VIII may increase this risk.

These are not all the possible side effects of ELOCTATE. Talk to your healthcare provider right away about any side effect that bothers you or that does not go away, or if bleeding is not controlled after using ELOCTATE.

**PLEASE SEE BRIEF SUMMARY OF PRESCRIBING
INFORMATION ON THE PREVIOUS PAGE**

**YOU ARE ENCOURAGED TO REPORT NEGATIVE SIDE
EFFECTS OF PRESCRIPTION DRUGS TO THE FDA. VISIT
WWW.FDA.GOV/MEDWATCH, OR CALL 1-800-FDA-1088.**

YOU HAVE QUESTIONS. CoRes HAVE ANSWERS.



Dedicated

CoRes are passionate about helping people in the Hemophilia community.



Understanding

CoRes are advocates with decades of experience who understand the community's needs.



Accessible

CoRes prioritize face-to-face conversations to get to know you. They're just a call, text, or email away.



Scan with your phone to learn more about how to protect you and your joints from bleeds.

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