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Four blood and bleeding disorders advocates share their stories.

# HEM AWARE

The Bleeding Disorders Magazine



NATIONAL BLEEDING DISORDERS FOUNDATION | [hemaware.org](http://hemaware.org)

THE FOUNDATION  
REBRANDS

## A New Name for a New Era

p.20

~~DAYS~~  
~~WEEKS~~  
~~MONTHS~~

**YEARS  
WITHOUT  
PROPHYLAXIS  
IS POSSIBLE**

Results are based on 112 people whose data were collected for at least 6 months before receiving ROCTAVIAN and compared with their data over a 3-year follow-up period.\*

**90** out of **112** people responded to ROCTAVIAN and were able to stop and stay off continuous prophylaxis.†

\*The 3-year follow-up period began 5 weeks or more after administration and consists of a median follow-up of 3 years with a range of 1.7 to 3.7 years. Median is the middle number in a list of numbers arranged from smallest to largest. Range shows the lowest and highest numbers in a list.

†ROCTAVIAN was studied in 112 people whose data were collected for at least 6 months before their infusion (rollover population) and 22 people who immediately received their infusion (directly enrolled population). ROCTAVIAN worked for 80% (90/112) of people in the rollover population and 68% (15/22) of people in the directly enrolled population throughout the 3-year follow-up period. Andrew was part of the directly enrolled population.

‡Prophylaxis is defined as the ongoing use of Factor VIII or another treatment to prevent bleeds.

## What is ROCTAVIAN?

ROCTAVIAN is a one-time gene therapy used for the treatment of adults with severe hemophilia A who do not have antibodies to the virus, AAV5 which is determined by a blood test.

## Important Safety Information

Do not take ROCTAVIAN if you have an active infection or if you have a long-term infection that is not controlled by the medicines you take, have scarring of the liver (significant liver fibrosis or cirrhosis), are allergic to mannitol (an inactive ingredient in ROCTAVIAN).

## What is the most important information I should know about ROCTAVIAN?

ROCTAVIAN may cause serious side effects during the infusion and afterward:

- During and in the hours following the infusion, tell your doctor or nurse immediately about any symptoms you experience, including hives or other rashes, itching, sneezing, coughing, difficulty breathing, runny nose, watery eyes, tingling throat, nausea (feeling sick), diarrhea, low blood pressure, rapid heartbeat, light-headedness (near-fainting), fever, chills, or shivering. Talk to your doctor about what to do if you experience any side effects after you leave the infusion
- Before and regularly following administration of ROCTAVIAN, your doctor will perform blood tests to check your liver health. Make sure you obtain these blood tests during the specified time your doctor instructs you to. Based on your liver test results, you may need to take corticosteroids or another medicine for a period of time (several months or longer) to help decrease liver enzyme levels, which may cause side effects while you receive them. Talk to your doctor about these side effects and what you need to do to improve and maintain your liver's health

Andrew,  
ROCTAVIAN clinical  
study participant



**ROCTAVIAN™**  
(valoctocogene roxaparvovec-rvox)  
Suspension for intravenous infusion

**Get started with  
eligibility testing today**



**Visit [ROCTAVIAN.com](https://roctavian.com)**

- Patients with active Factor VIII inhibitors should not take ROCTAVIAN. Following administration your doctor will monitor you for inhibitors and you will have regular factor level testing. Talk to your doctor if you start bleeding following ROCTAVIAN, in order for your doctor to assess the need for additional tests or treatments
- Depending on your risk factors, an improvement in Factor VIII levels may mean an increased possibility of unwanted blood clots (so called “thromboses,” in either veins or arteries). You and your doctor should discuss your risk factors before and after treatment and how to recognize symptoms of unwanted clots and what to do if you think you may have one
- ROCTAVIAN can insert itself into the DNA of human body cells. The effect that insertion may have on those cells is unknown, but such events may contribute to a theoretical risk of cancer. There have been no reported cases of cancer caused by treatment with ROCTAVIAN. Your doctor may perform regular monitoring if you have pre-existing risk factors for developing liver cancer. In the event of cancer, your doctor may send a sample to BioMarin Pharmaceutical Inc. for further testing

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](https://www.fda.gov/medwatch) or call 1-800-FDA-1088.

**Please see the brief summary of the Patient Information on the next page and Prescribing Information at [ROCTAVIAN.com](https://roctavian.com).**

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# Brief Summary of Patient Information

## What is ROCTAVIAN?

ROCTAVIAN is a one-time gene therapy used for the treatment of adults with severe hemophilia A who do not have antibodies to the virus, AAV5 which is determined by a blood test. ROCTAVIAN uses a modified virus, called a vector, to deliver a working copy of the Factor VIII gene to liver cells to enable your body to produce clotting factor on its own, which helps the blood to clot and prevents or reduces the occurrence of bleeding. The modified virus does not contain viral DNA and does not cause disease in humans.

## Do not take ROCTAVIAN if you:

- Have an active infection or if you have a long-term infection that is not controlled by the medicines you take
- Have scarring of the liver (significant liver fibrosis or cirrhosis)
- Are allergic to mannitol (an inactive ingredient in ROCTAVIAN)

## What is the most important information I should know about ROCTAVIAN?

ROCTAVIAN may cause serious side effects during the infusion and afterward:

- During and in the hours following the infusion, tell your doctor or nurse immediately about any symptoms you experience, including hives or other rashes, itching, sneezing, coughing, difficulty breathing, runny nose, watery eyes, tingling throat, nausea (feeling sick), diarrhea, low blood pressure, rapid heartbeat, light-headedness (near-fainting), fever, chills, or shivering. Talk to your doctor about what to do if you experience any side effects after you leave the infusion
- Before and regularly following administration of ROCTAVIAN, your doctor will perform blood tests to check your liver health. Make sure you obtain these blood tests during the specified time your doctor instructs you to. Based on your liver test results, you may need to take corticosteroids or another medicine for a period of time (several months or longer) to help decrease liver enzyme levels, which may cause side effects while you receive them. Talk to your doctor about these side effects and what you need to do to improve and maintain your liver's health
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- ROCTAVIAN can insert itself into the DNA of human body cells. The effect that insertion may have on those cells is unknown, but such events may contribute to a theoretical risk of cancer. There have been no reported cases of cancer caused by treatment with ROCTAVIAN. Your doctor may perform regular monitoring if you have pre-existing risk factors for developing liver cancer. In the event of cancer, your doctor may send a sample to BioMarin Pharmaceutical Inc. for further testing

## What should I tell my doctor before I get ROCTAVIAN?

Talk to your doctor about the following:

- **Your medical conditions including:**
  - Any general risk factors for unwanted blood clots and for cardiovascular disease

- If your immune system's ability to fight infections is reduced
- If you have inhibitors or a history of inhibitors to Factor VIII

- **All medicines you take or new medicines you plan to take**, including prescription and nonprescription drugs, vitamins, herbal supplements, and vaccines

- If you have a female partner that plans to become pregnant within 6 months of treatment

## What should I avoid after taking ROCTAVIAN?

- Avoid alcohol use for the first year. Talk to your doctor about how much alcohol may be acceptable after the first year
- You and any female partner must prevent becoming pregnant for 6 months. Discuss with your doctor which methods of contraception are suitable
- Do not donate semen for at least 6 months after treatment
- Do not donate blood, organs, tissues, or cells

## What are the possible side effects of ROCTAVIAN?

### • The most common side effects of ROCTAVIAN are:

- Nausea, fatigue, headache, infusion-related reactions, vomiting, and abdominal pain
- Changes to laboratory results from blood tests that measure your liver health and other ways your body is responding to ROCTAVIAN

## What other information should I know before getting ROCTAVIAN?

- **Receiving gene therapy again in the future:** ROCTAVIAN is a one-time treatment. Currently, treatment with ROCTAVIAN means you cannot receive another gene therapy for hemophilia
- **Hemophilia treatment registry:** After treatment with ROCTAVIAN, you will be asked to enroll in a 15-year registry to help study the long-term safety of the treatment and how well it continues to work
- **Understanding the risks and benefits of ROCTAVIAN:** While the majority of patients experience a benefit from ROCTAVIAN, the treatment response and duration may vary. Some patients do not experience a benefit from ROCTAVIAN. It is not possible to predict if and how much a patient may benefit. After administration, your doctor will monitor your lab tests and talk to you about whether you can stop prophylaxis, whether you should start prophylaxis again, and whether and how you should treat any surgeries, procedures, injuries, or bleeds

Talk to your doctor about the potential risks and benefits of ROCTAVIAN. Whether a patient experiences a benefit or not, the risks discussed here and with your doctor still apply.

These are not all the possible side effects of ROCTAVIAN. Talk to your doctor for medical advice about side effects. You may report side effects to BioMarin Pharmaceutical Inc. at 1-866-906-6100 or FDA at 1-800-FDA-1088.

## Please see additional safety information in the Prescribing Information and Patient Information at ROCTAVIAN.com.

**You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch) or call 1-800-FDA-1088.**

Manufactured by

**B:OMARIN**

BioMarin Pharmaceutical Inc.  
105 Digital Drive, Novato, CA 94949

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**“People are working hard for others not to go through what we’ve been through.”**

On the Cover

Photo Illustration by  
Angela Koch



### CORRECTION:

In the "Hemophilia & Heart Health" article on Page 26 of the Spring/Summer 2023 issue of *HemAware*, Ashley Gonzales was incorrectly identified as a clinical psychologist.



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All manuscript submissions should be typed double space, between 500 and 1,000 words in length and sent by email to donna.behen@manifest.com. *HemAware* retains the right to edit submissions. The content of the article is the responsibility of the author(s) and must be original work that has not been, nor will be, published elsewhere. A copyright agreement attesting to this and transferring copyright to NBDF is required.

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PHOTOGRAPH BY LUCY HEWETT

# In Focus

PRESIDENT AND CEO LEONARD A. VALENTINO, M.D.



**We remain  
dedicated to  
supporting our  
network of over  
50 chapters  
across the country  
and channeling  
funds into blood  
and bleeding  
disorders  
research.**

## A New Chapter Begins

This year's Annual Bleeding Disorders Conference in National Harbor, Maryland, was a milestone meeting for the foundation. In addition to celebrating our 75th anniversary, we also formally announced our new name: the National Bleeding Disorders Foundation.

Over the past 75 years, our foundation has served people facing other blood and bleeding disorders such as von Willebrand disease, rare factor deficiencies, and platelet disorders, but our name and image did not reflect that inclusivity, until now.

Along with our new name, we've introduced a new logo — featuring a circle of blood drops in different colors to represent inclusivity — and a new tagline that better

reflects our powerful combination of research, education, and advocacy: innovate, educate, advocate. You can read more about the rebranding on Page 20.

One thing that did not, and will not, change is our focus. We remain dedicated to supporting our network of over 50 chapters across the country and channeling funds into blood and bleeding disorders research. And we will continue to educate and support families living with these disorders — along with the clinicians who care for them — as we work tirelessly to protect access to health care on the state and local levels.

No matter what type of blood or bleeding disorder you have, you will find a home in the National Bleeding Disorders Foundation.

**Online** | [hemaware.org](https://hemaware.org)



**National Bleeding Disorders Foundation:** [hemophilia.org](https://hemophilia.org)

**Facebook:** @NationalBleedingDisordersFoundation

**X, formerly known as Twitter:** @NBD\_Foundation and

@NBDFespanol

**Instagram:** @nbd\_foundation

**TikTok:** @natlbdfoundation

**YouTube:** [youtube.com/@NBDVideo](https://youtube.com/@NBDVideo)

**Advocacy:** [hemophilia.org/advocacy](https://hemophilia.org/advocacy)

**Steps for Living:** [stepsforliving.hemophilia.org](https://stepsforliving.hemophilia.org)

**Victory For Women:** [victoryforwomen.org](https://victoryforwomen.org)

**Better You Know:** [betyouknow.org](https://betyouknow.org)

# OUR VISION: A WORLD WHERE NO LIFE IS LIMITED BY GENETIC DISEASE

At Spark® Therapeutics, we are committed to discovering, developing and delivering gene therapies.

We believe investigational gene therapy has the potential to be transformative in the treatment of hemophilia and we understand the importance of developing gene therapies that meet the needs of the hemophilia community. Our priority is the safety and well-being of clinical trial participants.



Learn about gene therapy research for hemophilia.

**Spark**<sup>™</sup>  
THERAPEUTICS



Interested in enrolling in a Spark-sponsored hemophilia gene therapy clinical trial?



Want to know more about gene therapy clinical trials?

Discover more about gene therapy research





# EXPLORE THE SCIENCE OF GENE THERAPY RESEARCH

Be informed and feel empowered when you learn about the field of gene therapy and its potential application for hemophilia.



Discover what gene therapy is meant to do.



Explore frequently asked questions about hemophilia gene therapy clinical trials.



See a demo about the science of gene therapy.

Visit  
[HemophiliaForward.com](https://HemophiliaForward.com)



# THANK YOU DR. VALENTINO



Dr. Len Valentino, NBDF CEO, will be stepping away in the coming months. HemAware thanks him for his continued support and service of blood and bleeding disorders.

Send your own well-wishes to [lenv@hemophilia.org](mailto:lenv@hemophilia.org)



Formerly NHF  
NATIONAL  
**BLEEDING DISORDERS**  
FOUNDATION

Innovate | Educate | Advocate

---

# Healthy Start

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STATS, FACTS, AND NEWS YOU CAN USE

## *On the Move*

We asked community members, "How do you help keep your kids physically active?" Here's what some of them shared.



**My son has been obsessed with riding his scooter lately.**

—KARA



VAIDAS\_BUCYS/STOCKSY

*We recently joined Planet Fitness — the bike keeps our joints moving.*

—DAVE

*My kids enjoy golf and swimming.*

—EMILY

*Skijoring — the dogs can come too!*

—ELIZA



## CELEBRATING DR. LEONARD A. VALENTINO'S LEGACY

At the end of 2023, National Bleeding Disorders Foundation President and CEO Leonard A. Valentino, M.D., will retire, ending his nearly four-year tenure. The position capped a career devoted to helping people with blood and bleeding disorders.

Valentino worked as a hematologist for decades, then held positions in the clinical and laboratory research space and the biopharmaceutical industry.

"Len spent time trying to understand how to bring new therapies to people globally," says NBDF Chief Operating Officer Dawn Rotellini. "The Board of Directors hired him to move the foundation into the research space and lead us in our evolution."

Under Valentino's guidance, NBDF conducted an exhaustive series of virtual focus groups and listening sessions, seeking input from community stakeholders. The process amplified the needs of people with bleeding disorders and identified gaps in diagnosis, treatment, and care.

These efforts led to the creation of multiple working groups within the foundation; a health equity, diversity, and inclusion department; a State of the Science Summit; and a summit focused on the care and needs of people who menstruate. It also prompted the foundation to rebrand with a name that's inclusive of the entire bleeding disorders community.

Notably, Valentino's work enabled NBDF to build its National Research Blueprint, which will guide the organization in the future.

"His ability to take a vision and put it into a pathway for us to follow is his legacy," Rotellini says.



# The HELP Copays Act Would Make Medications More Affordable



Clotting factor and other lifesaving medications that treat blood and bleeding disorders are often incredibly expensive, prompting many people to seek manufacturer or charitable copay assistance to afford their therapies. But a growing number of health insurance plans have copay accumulator adjustment programs, which means that financial assistance doesn't count toward a person's deductible or out-of-pocket maximum.

The proposed HELP Copays Act, introduced in the U.S. House of Representatives and the Senate, would resolve this problem, ensuring that all payments made by or on behalf of patients count toward their cost-sharing obligations. "We estimate that about 60% of people with bleeding disorders are on a self-insured plan provided by their employer," says Bill Robie, director of state government relations for the National Bleeding Disorders Foundation. "That's

where we see the majority of these (problematic) policies. They're federally regulated, which is why we need a federal bill."

"There's a lot of activity on health care this year, and the focus is on reform," Robie says. "The All Copays Count Coalition (a patient advocacy group of more than 80 nonprofit organizations) is trying to get our bill attached to a bigger pharmacy benefit manager reform bill that's moving in the House or the Senate."

To support the HELP Copays Act, call or email your legislators. Share your story if you can.

—By Lisa Fields



**SUPPORT NBDF ON GIVING SHOESDAY**

Now in its

# Fourth

year, Giving Shoesday is the National Bleeding Disorders Foundation's

# Global

day of giving on the Tuesday after Thanksgiving, when people who make minimum donations to

# NBDF

can receive limited-edition NBDF shoes.



**LEARN MORE:**

[hemophilia.org/givingtuesday](https://hemophilia.org/givingtuesday)



FIRST AND ONLY FDA-APPROVED GENE THERAPY FOR HEMOPHILIA B

## STEP INTO A WORLD OF ELEVATED FACTOR IX LEVELS THAT LAST FOR YEARS



Patient portrayal; HEMGENIX not intended for women.

### A ONE-TIME INFUSION DELIVERS GREATER BLEED PROTECTION\*

# 37%

AVERAGE FACTOR IX ACTIVITY SUSTAINED AT 2 YEARS



GREATER BLEED PROTECTION VS. ROUTINE FACTOR IX PROPHY\*

# 94%

OF PEOPLE DISCONTINUED FACTOR IX PROPHY AND REMAINED PROPHY-FREE†

\*In the clinical trial, annualized bleed rate (ABR) for all bleeds decreased from an average of 4.1 for patients on prophylaxis (prophy) during the lead-in period to 1.9 (54% reduction) in months 7-18 after treatment.

†51 out of 54 people remained free of continuous routine factor IX prophylaxis (prophy).

Step into a new world today at [HEMGENIX.com](https://www.HEMGENIX.com)



### IMPORTANT SAFETY INFORMATION

#### What is HEMGENIX?

HEMGENIX®, etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

#### What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

#### What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

#### What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

#### What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

#### Please see full prescribing information for HEMGENIX.

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

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

HEMGENIX is manufactured by uniQure Inc. and distributed by CSL Behring LLC.

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**CSL Behring**



# Making Sense of Health Insurance Terms

If you're a young adult who has recently transitioned to your own health insurance plan, here's a rundown of the terms you need to know



FOR MORE TERMS: [healthcare.gov/glossary](https://www.healthcare.gov/glossary)



**CLAIM**

An explanation of medical services that you or your provider sends to your insurance company to obtain payment.



**COINSURANCE**

The portion of health care services that you are responsible for paying after you have reached your deductible.



**COPAYMENT**

The amount you pay at the time of your appointment.



**DEDUCTIBLE**

The amount you pay out of pocket before your insurance company will begin payments.



**IN-NETWORK PROVIDER**

A health care provider who has a contract with your insurance company to give you medical care.



**NONCOVERED SERVICES**

Health care services that your insurance company won't pay for.



**POLICY**

A contract between you and your insurance company that covers your health care costs.



**PREMIUM**

What you pay every month for health insurance.



**REFERRAL**

An authorization from your primary care provider that your insurance company may require you to obtain before you can receive certain services or see a specialist.

JANE\_KELLY/GETTY IMAGES

**BRIEF SUMMARY OF PRESCRIBING INFORMATION**

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

**HEMGENIX® (etranacogene dezaparvovec-drlb) suspension, for intravenous infusion**  
Initial U.S. Approval: 2022

**INDICATIONS AND USAGE**

HEMGENIX is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or
- Have repeated, serious spontaneous bleeding episodes.

**CONTRAINDICATIONS**

None.

**WARNINGS AND PRECAUTIONS**

- Infusion reactions: Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or interrupt administration. Re-start administration at a slower infusion once resolved.
- Hepatotoxicity: Closely monitor transaminase levels once per week for 3 months after HEMGENIX administration to mitigate the risk of potential hepatotoxicity. Continue to monitor transaminases in all patients who developed liver enzyme elevations until liver enzymes return to baseline. Consider corticosteroid treatment should elevations occur.

- Hepatocellular carcinogenicity: For patients with preexisting risk factors (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age), perform regular (e.g., annual) liver ultrasound and alpha-fetoprotein testing following administration.
- Monitoring Laboratory tests: Monitor for Factor IX activity and Factor IX inhibitors.

**ADVERSE REACTIONS**

The most common adverse reactions (incidence ≥5%) were elevated ALT, headache, blood creatine kinase elevations, flu-like symptoms, infusion-related reactions, fatigue, malaise and elevated AST.

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring at 1-866-915-6958 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](https://www.fda.gov/medwatch).

**USE IN SPECIFIC POPULATIONS**

No dose adjustment is required in geriatric, hepatic, or renal impaired patients.

Based on November 2022 version

**VITAL ORGAN**

The liver plays a crucial role in regulating blood clotting.

# The Liver Disease That's on the Rise

Here's why it matters for people with bleeding disorders

Have you heard of metabolic dysfunction-associated steatotic liver disease, or MASLD?

The term refers to a rising disease in which fat accumulates in the liver, causing it to enlarge and become impaired. While some fat is normally present in the liver, if fat makes up more than 5% to 10% of the liver's weight, that meets the criteria for MASLD. (This disease used to be called nonalcoholic fatty liver disease.)

It's important for everyone to have a healthy liver, but that's especially true for people with hemophilia and other bleeding disorders, because in addition to serving other functions such as digesting food, removing toxins, and storing energy, the liver plays a crucial role in the body's ability to regulate blood clotting.

**RISING RATES**

A 2023 study conducted by researchers at the Charles R. Drew University of Medicine and Science in Los Angeles revealed that MASLD in Americans increased 131% during the past three decades, rising from 16% in 1988 to 37% in 2018.

A Taiwanese study in *Clinical and Applied Thrombosis/Hemostasis* found a high prevalence of MASLD in people with hemophilia. This is important, researchers say, because "liver health is essential for persons with hemophilia (PWH) in order to maintain access to new therapies, such as gene therapy."

In addition, untreated MASLD can lead to cirrhosis, liver cancer, and even liver failure.

**WHAT CAUSES MASLD?**

Bruce Luxon, M.D., Ph.D., chair of the Department of Medicine at Georgetown University Medical Center in Washington, D.C., and a member of the National Bleeding Disorders Foundation's Medical and Scientific Advisory Council, says the main risk factors for developing complicated MASLD are:

- High body mass index (BMI)
- High blood pressure

- Elevated serum glucose
- High triglycerides and low LDL cholesterol
- Smoking
- Having or being at risk for cardiometabolic disorders, a cluster of diseases that includes cardiovascular disease, metabolic syndrome, and diabetes

**THE CONNECTION TO HEMOPHILIA**

Experts aren't exactly sure what is driving the increase in MASLD in people with hemophilia, but one hypothesis, according to the Taiwanese study, is being overweight or obese. While rates of obesity have increased in the general population due to the pandemic, "people who have hemophilia often have limited joint mobility, so their ability to exercise and maintain reasonable weight is more difficult," Luxon says.

He urges people with hemophilia who are at risk for MASLD to stay positive. "Medical science is trying to find a pharmacologic treatment that would help minimize liver damage. It'll be a slow process as we figure out how to use these pharmacologic agents, but I think we will have that in a couple of years," Luxon says.

—By Beth Levine

## Protect Your Liver

Georgetown University Medical Center's Bruce Luxon, M.D., Ph.D., offers advice on how you can reduce your risk of developing MASLD:

- If you have a cardiometabolic disorder (cardiovascular disease, metabolic syndrome, or diabetes), make sure it's being treated and well managed by your provider.
- Maintain a healthy BMI. If you have trouble exercising, ask your doctor or physical therapist to help you develop a safe routine.
- Eat a healthy diet. Your menu should be primarily plant-based, and you should avoid processed and fatty foods.
- Reduce alcohol intake.
- Quit smoking.



# 4 Sneaky Money Traps

Try to avoid costly culprits such as payment plans and auto-renewing subscriptions

Gen Z's spending power is growing, thanks to a historic job market and strong starting salaries. But young adults can face financial problems, and quickly, if they don't realize how much they're spending.

Natalie Stanger, a member of the American Association of Daily Money Managers, recommends carefully monitoring your monthly income and expenses. Start with a budgeting app such as Mint, Honeydue, or You Need a Budget, or an online resource from your bank. But remember: "These apps are a tool," she says. "You have to do the work to review and modify your cash flow."

As you look through your finances, watch out for these four money traps.

## BUY NOW, PAY LATER PLANS

BNPL plans are alluring because they break up larger purchases into smaller payments, and some do so with no interest for, say, the first year. Nearly

two-thirds of Gen Z buyers — more than any other generation — have used a BNPL plan, according to a March 2023 survey by LendingTree.

The risk is that these installment plans may cause you to lose sight of the total purchase cost and take on unnecessary debt.

Stanger recommends using BNPL strictly for items you need but may not have the cash on hand to buy in one transaction, such as appliances, and only if you can pay them off within the interest-free period.

"That way you have stretched out your money and it hasn't cost you anything in interest," she says. If you extend payments beyond the introductory period, transfer the balance to a lower-interest credit card.

## MONTHLY SUBSCRIPTIONS

Americans spend \$219 per month on subscriptions, according to C+R

Research. From music streaming to curated clothes delivery, auto-renewing services quickly accumulate.

"Prioritize which subscriptions are best for you. You don't need every service," says Brian DuVal, a 22-year-old with severe hemophilia A. After considering his options for streaming services, for example, DuVal decided on YouTube Premium and opted out of others.

Stanger suggests periodically reviewing your financial statements to know what you're paying for and canceling what you no longer use.

## FOOD DELIVERY CHARGES

The pandemic normalized food delivery services, but the convenience comes at a cost — more than 40% of an average meal price includes fees that go to the service provider and delivery person.

"These conveniences add up to a lot of money in a month's time," Stanger explains.

Next time you're craving a carne asada burrito, walk or drive to your favorite spot to skip the delivery fees. Better yet, buy the ingredients from a store and make the meal at home. If you want food delivery, save it for a rare occasion.

## HEALTH INSURANCE

Managing health insurance costs starts with selecting the best plan for you, which might not be the least expensive one, DuVal says. Plans with lower premiums might end up costing you more for the treatments you need.

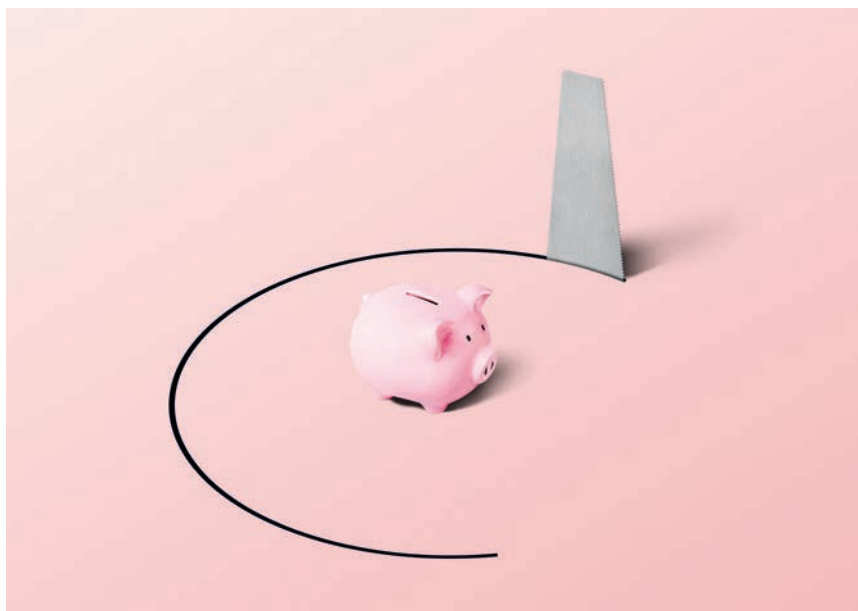
DuVal recommends meeting with someone at your hemophilia treatment center or an insurance navigator to ensure your plan will provide adequate coverage for your care.

"People with bleeding disorders can face an additional financial burden," he says. "We rely on expensive medication, and receiving access to medication should always be the first priority."

—By Celeste Sepessy

## WATCH OUT

You could be cutting into your finances without realizing it.



## FINANCIAL PLANNING WITH A BLEEDING

**DISORDER:** To secure your financial future, you need a plan. Here's what you should know: [hemaware.org/financialfaqs](http://hemaware.org/financialfaqs)

**BE AWARE**

Common symptoms include fatigue, hair loss, and brain fog.

# A Red Flag for Women of Childbearing Age

Those with bleeding disorders are at risk of iron deficiency, with or without anemia

Why are women and people with the potential to menstruate (WPPM) who have hemophilia, von Willebrand disease, or other bleeding disorders more likely than men to develop iron deficiency?

Heavy, prolonged menstrual periods increase their risk. Pregnancy also contributes to iron deficiency: Mothers transfer iron to their babies in utero, and blood is lost at the time of delivery.

“Women, girls, and people with the potential to menstruate can bleed in every way that other people can,” says Bethany Samuelson Bannow, M.D., a hematologist at Oregon Health & Science University’s

Center for Women’s Health in Portland. “When you add menstruation and pregnancy on top of it, that’s why this population is at higher risk for iron deficiency.”

When iron deficiency arises, a person’s iron stores are lower than they should be, and people may notice that they don’t feel well.

“[It] can manifest in symptoms ranging from fatigue, lightheadedness, and brain fog to hair loss and nail changes,” says Angela Christine Weyand, M.D., a pediatric hematologist at the Hematology Oncology Clinic at C.S. Mott Children’s Hospital in Ann Arbor, Michigan.

Doctors can look for iron deficiency by checking someone’s levels of ferritin, a blood protein that contains iron. People with more severe iron deficiency may also develop anemia, when they have low levels of hemoglobin, a protein in red blood cells.

“Anyone who menstruates should get their ferritin checked at least once a year,” Samuelson Bannow says. “Ask for that ferritin number, not just complete blood count and hemoglobin [for] anemia.”

**NEW TESTING CRITERIA**

For years, many labs have used different parameters when measuring ferritin and hemoglobin levels in women and men, with lower numbers deemed “normal” for women.

“Their ‘normal’ range is not giving us the full picture,” says Samuelson Bannow, who notes that labs settle on their numbers by looking at ferritin and hemoglobin levels in a healthy population, but because iron deficiency is prevalent among WPPM, lower levels may seem to be the norm. “There’s no reason to think that people who menstruate need less red blood cells or less iron stores compared with people who don’t menstruate.”

Some labs have started to update their parameters for women, girls, and people with the potential to menstruate, but this varies by location, Samuelson Bannow says.

Treatments for iron deficiency are available for WPPM with bleeding disorders. Among them are dietary changes, oral supplements, and intravenous iron. “It is important to replace the deficit and correct the underlying issue,” Weyand says.

Hormonal birth control and tranexamic acid may be prescribed to help control heavy or prolonged menstrual bleeding.

“If you don’t slow menstrual losses, the iron deficiency is going to recur, and you’re going to need IV iron again and again,” Samuelson Bannow says.

—By Lisa Fields



LEARN MORE ABOUT WPPM AND BLEEDING DISORDERS:  
victoryforwomen.org

# When a Sibling Has a Bleeding Disorder

The extra attention can breed jealousy in a brother or sister. Here's how to handle it.

When a child has a chronic health condition, it affects the whole family. Though it can cause uncertainty and stress, it also creates the possibility of drawing closer as a unit.

Of course, try telling that to a brother or sister who isn't the focus of a parent's continual attention. Sibling rivalry is a normal occurrence, but it can be amplified when one child needs special care for a blood or bleeding disorder and one does not.

## BE AWARE

Kate Bazinsky's 7-year-old son, Colin, lives with severe hemophilia A and requires daily infusions. During this process, he gets to pick a TV show to watch so that he'll sit still. Aware that her 9-year-old daughter, Nora, can feel left out, Bazinsky allows her to pick the show when the family watches in the evening.

"It's hard for Nora because Colin's condition requires a lot of attention from myself and my husband," she says. "For example, if we planned to do something with Nora and Colin gets hurt and has to go to the hospital, then we have to cancel on Nora. Although she manages really well, I know she sometimes feels angry and resentful about these interruptions."

Bazinsky says they work hard to minimize the impact. In the case of Colin's hospital visits, she has her parents, who live nearby, watch Nora. "Instead of being an afterthought at the hospital," Bazinsky says, "she gets to do fun things with her grandparents."

## TRY THESE TIPS

Susan Earl, a licensed clinical social worker for pediatric hematology patients at the Utah Center for Bleeding & Clotting Disorders at Primary Children's Hospital in Salt Lake City, suggests several ways parents can handle feelings of jealousy from siblings when one child is dealing with a medical condition.

**1. Communicate openly.** Be honest and keep it simple, Earl says. "Start early. Make the diagnosis a part of life. You're going to have to point out that the child with a bleeding disorder may need more help or more attention at times."

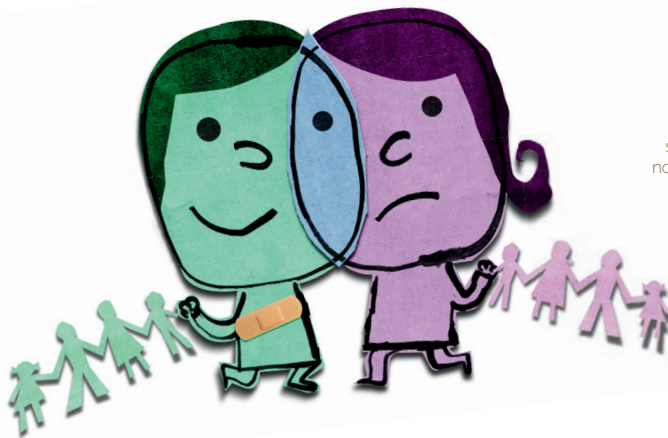
**2. Involve siblings in care.** You can teach the other children who don't have a bleeding disorder how to help their brother or sister, Earl says. "Let them know we can all be involved in it."

**3. Attend a family camp.** A lot of National Bleeding Disorders Foundation chapters offer summer camps for children with bleeding disorders, and some camps allow siblings to go, too. It's a great way to include the siblings who might be feeling left out.

**4. Support your child's interests.** Find that one thing the sibling is passionate about and support it, Earl says. Celebrating a special interest can help foster a sense of individuality in each child.

**5. Teach empathy.** Having a sibling with a disorder is an opportunity to teach compassion and concern. Help children try to understand what others are feeling. This can work both ways — the child with the bleeding disorder may better understand why a brother or sister is jealous sometimes.

—By Amanda Kippert



## FAMILY TIES

Make an effort to include siblings so no one feels left out.

**“If we planned to do something with Nora and Colin gets hurt and has to go to the hospital, then we have to cancel on Nora.”**

**HOW'S YOUR PARENT PARTNERSHIP?** While you're making sure your children feel included, don't forget to nurture the relationship with your partner. Read more at [hemaware.org/life/nurturing-your-parent-partnership](http://hemaware.org/life/nurturing-your-parent-partnership)



**GET GOING**

Start saving now if you haven't already.

# It's Not Too Late to Plan for Retirement

Here's what people with bleeding disorders need to keep in mind

Until recently, planning for retirement was a privilege most people with hemophilia didn't get to have. That's changed, thankfully, now that treatment advances have improved people's quality of life and extended their lives.

"We used to think we were not going to live past our 40s and 50s. Now, we think we're going to live long enough to see grandchildren grow up," says Beth Merz, a social worker at the Johns Hopkins Hemophilia Treatment Center in Baltimore. "If your timeline gets extended due to improved treatments, you'll need to live differently in all

areas — financial life included. It has far-reaching implications."

Experts recommend planning for retirement early in your career. But what if you are in your 40s or 50s and haven't done much about it yet?

It's never too late to start, says Carolyn McClanahan, M.D., CFP, a financial planner and physician in Jacksonville, Florida, who specializes in financial planning for people with chronic illnesses and other health issues.

"When you have a chronic condition such as a bleeding disorder, it's about much more than just saving for

retirement," McClanahan says. "You also need to make sure that you have good health insurance now; that if something bad happens, you have good disability insurance to pick up if you can't work; and that when you do retire, good supplemental insurance will cover your medications."

"The earlier you plan, the more money you can accumulate. But in your 40s and 50s, if you don't start saving something, you're going to run into a situation where you may never be able to quit working," she adds.

—By Andrea Atkins



Look at your spending and savings



Choose your Medicare plan wisely



Have an emergency fund

## Steps You Can Take

If retirement (or even semi-retirement) is looming large, here's what financial planner Carolyn McClanahan recommends:

### LOOK AT YOUR SPENDING AND YOUR SAVINGS.

"The biggest determinant of being able to retire is really how much you spend. If you spend \$80,000 a year, that's a lot easier to support than if you spend \$300,000 per year," McClanahan says, adding that a certified financial planner can help you assess whether you're saving enough to support your spending. (You can even hire someone on an hourly basis to get started, she says.)

### DIVIDE YOUR SPENDING INTO NEEDS AND WANTS.

You need a home and health insurance, but you may want to have dinner out three times a week. You may have to eat out less or spend less on discretionary purchases. "Pare back the things that are not bringing much value to your life," McClanahan says.

### ESTIMATE HOW MUCH STEADY MONEY YOU'LL HAVE COMING IN.

Assess your Social Security and pension options to see how much cash you'll generate on retirement.

### DECIDE WHERE YOU'LL PUT YOUR SAVINGS.

"Contribute to an IRA or put money into a broker-age account," McClanahan says, noting that if you don't take advantage of benefits such as employee matching grants and health savings plans, you're leaving money on the table.

### CHOOSE YOUR MEDICARE PLAN WISELY.

McClanahan recommends staying away from Medicare Advantage plans, as they "don't always cover what you need."

### HAVE AN EMERGENCY FUND.

For someone with a chronic disease, she recommends putting aside a year's worth of living expenses. "People who have illnesses often struggle between spending lavishly while healthy and having money for the future," McClanahan says. "The good news is that it's rare these days that anyone is going to die of hemophilia. You have to be prepared for living longer."



The Medical College of Wisconsin is seeking volunteers with severe Hemophilia A with a history of inhibitors for a research study being performed at Froedtert Hospital in Milwaukee, Wisconsin. This trial is registered at [clinicaltrials.gov](https://clinicaltrials.gov) (NCT03818763)

In the study, blood cells will be collected from the patient, combined with a gene to direct the patient's platelets (blood cells that help blood to clot) to make factor VIII, then returned to the patient. These platelets will possibly make Factor VIII themselves. This project is designed to test and find out more about the side effects (problems and symptoms) of a gene therapy for severe Hemophilia A.

### WHO IS ELIGIBLE?

- Age 18 years and older, with severe hemophilia A, and a history of inhibitors to FVIII
- Receiving factor VIII treatment (any prophylaxis and on demand treatment for bleeding)

### WHO IS NOT ELIGIBLE?

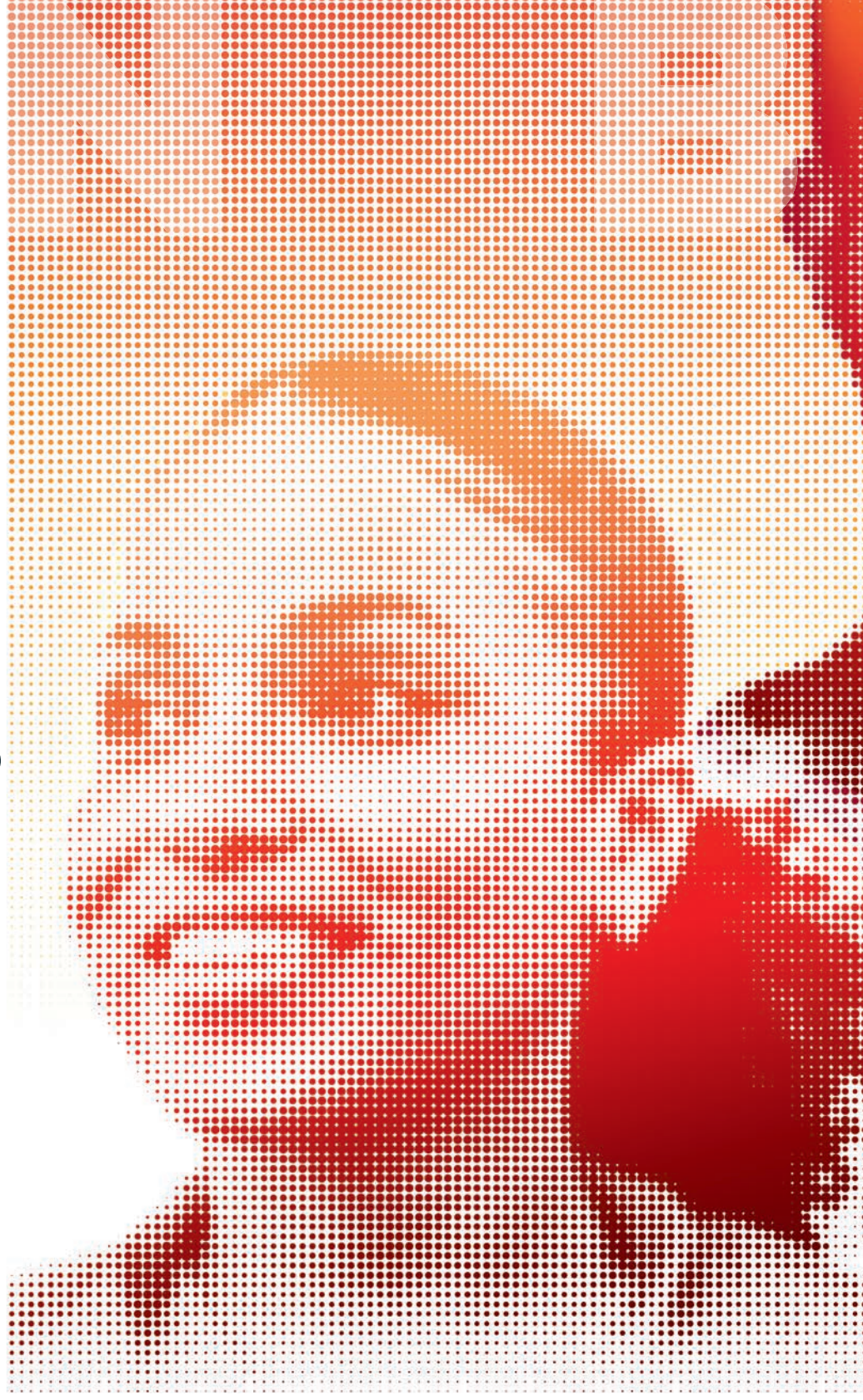
- Younger than 18 years, or if over 18, must not have recently participated in another gene therapy trial

### HOW CAN I LEARN MORE ABOUT THE TRIAL?

- Information is available at [clinicaltrials.gov](https://clinicaltrials.gov) and search using NCT03818763
- You may contact Ms. Arielle Baim, Program Manager, at 414-805-8745 or email: [abaim@mcw.edu](mailto:abaim@mcw.edu)

# A NEW NAME FOR A NEW ERA

**NHF is now the National Bleeding Disorders Foundation**





“

*The goal of rebranding is to become more inclusive of the disorders that we are already providing services for.*

”

Dr. Leonard Valentino



This year, as the foundation celebrates its 75th anniversary, an exciting new chapter begins. The organization formerly known as the National Hemophilia Foundation, which has served generations of people with blood and bleeding disorders and their families, is now known as the National Bleeding Disorders Foundation. The organization's website and educational materials also bear a new look and logo.

“When I came to the NHF, my perspective as a hematologist was that we take care of all blood and bleeding disorders — not just hemophilia but also von Willebrand disease, platelet disorders, and rare coagulation factor deficiencies,” says foundation CEO Leonard Valentino, M.D. “So, we wanted to make sure that people who live with, say, factor XIII deficiency could see themselves in the organization.”

“The community was consistently telling us that our name was not inclusive of disorders that we were currently serving,” adds Chief Operating Officer Dawn Rotellini. “We heard from many community members who hadn’t always realized that we served their bleeding disorder.”

In fact, in one case, it took more than six years from the time of diagnosis for one individual to learn that the foundation could help navigate treatment for their factor VII deficiency. “They were all alone and had no idea of the resources that were available,” Rotellini says.

“The goal of rebranding is to become more inclusive of the disorders that we are already providing services for so that we can reach more people with those disorders, and then potentially also expand our reach into other disease communities that we haven’t supported as well,” Valentino says.

#### RETURNING TO OUR ROOTS

The NHF was founded as the Hemophilia Association in 1948 by Robert and Betty Jane Henry, whose son, Lee, had hemophilia. They were moved to act by a sense of isolation and their frustration over the lack of research into the treatment of bleeding conditions.

“When it was incorporated, the organization was actually focused on blood disorders and related conditions,” Valentino says. “But in the 1980s, the group (known then as the National Hemophilia Foundation) became laser focused on hemophilia because of the HIV crisis and the rise of hepatitis C. Advocacy around the disease became paramount.”

From the late 1970s through the mid-1980s, about half of the people with hemophilia in the U.S. became infected with HIV through contaminated blood products, and thousands died. The hepatitis C virus was also transmitted through contaminated factor products. Before testing for the virus began in 1992, an estimated 44% of people with hemophilia had contracted hepatitis C.

The impact of these diseases on the bleeding disorders community pervaded the next few decades. “Now, it’s clear that while we need to maintain that focus, we should return to focusing on other blood and bleeding disorders,” Valentino says.



### NAMES MATTER

The rebranding process evolved from strategic planning meetings that began a few years ago. Participants were asked to envision how the foundation should look in the immediate future and in 30 years.

“A few chapters had changed the name to say ‘bleeding disorders’ several years ago, and then we started to see more do it,” Rotellini says. “We decided this was the right time.” As of press time, 17 chapters had switched.

The decision coincided with the beginning of the COVID-19 pandemic. So, over the course of several months, the organization held multiple virtual listening sessions, each with 10 to 15 stakeholders, including people with bleeding disorders, health care providers, chapter representatives, and industry partners.

Then, after the feedback was posted online, 900 additional comments were received. “What came through loud and clear was that people wanted us to be more inclusive,” Rotellini says.

Not everyone was sure of the new focus. Some people with hemophilia worried that they might be left behind.

“Whenever there is a name change, there are people who feel as though maybe they are not going to be as supported,” Valentino says. “Those are the voices we want to hear and understand.”

Adds Rotellini, “We will still be dedicated to serving the communities that we’ve been serving, and that includes hemophilia. That is not going away.”

### BRINGING IN THE PROS

The foundation chose Merz Branding of Media, Pennsylvania, to facilitate the rebranding. The company, which has managed the process for other health organizations, talked to key foundation leadership and staff, the Board of Directors, and medical professionals.

Merz also sought input through one-on-one interviews and Zoom focus groups with donors, policymakers, experts, and people living with bleeding disorders. “We turned the telescope inside and out,” says Mary Kate Lo Conte, a firm partner.

With its findings, Merz began presenting a range of names and logos. “We reviewed

feedback we received, and it crystallized into the name and logo we shared with stakeholders and then fine-tuned, which resulted in the final brand identity,” Lo Conte says.

The new logo also reflects the foundation’s big-tent approach. “It has a strong color palette, and there is a visual element to it — basically a circle of blood drops,” Lo Conte says. “The drops are in different colors to represent inclusivity.” The circular design reflects the “power up” button on digital devices. The meaning: “The organization is only getting stronger and better,” she says.

The response has been positive. “We’ve gotten really good feedback,” Lo Conte says. “Within the organization, people are saying things like, ‘Every time I see it, I

like it more,’ which is fabulous to hear.” The new name could pose a challenge when the organization is lobbying Congress. However, Rotellini believes the foundation is well established on the Hill and elsewhere and can weather any potential confusion.

“We plan to use the phrase ‘formerly known as National Hemophilia Foundation’ for about a year,” she says. “I’m not too concerned about it, but communication will be key.”

### NEW OPPORTUNITIES TO SERVE

The new look, name, and focus are just the beginning, says Rotellini, who sees the rebranding effort as a work in progress. As it is rolled out, the foundation plans to conduct a landscape analysis.

“We need to know what is out there and what blood and bleeding conditions are not currently being served in any way,” Rotellini says. “If there is a condition that has no organization, but someone is doing patient advocacy, we will look to partner with them. It’s in communities that have no educational resources, materials, or training where we will potentially see expansion down the line. However, there’s no intention to take over what another organization already does very well in their community.”

“Rebranding represents an opportunity for adding voices,” Valentino says. “You are always stronger when you speak with one voice. And you’re stronger when you have more voices that are speaking the same way.”

“

*Rebranding represents an opportunity for adding voices.*

”

Dr. Leonard Valentino

#### +

### INCLUSIVITY

The goal of rebranding is to reach more people with blood and bleeding disorders and expand into other disease communities.

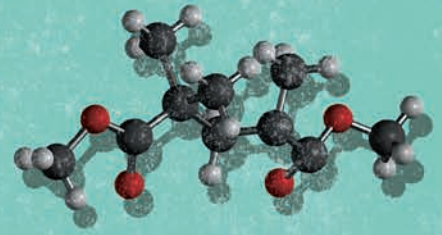
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Technology is making way for a more connect

WRITTEN BY STEPHANIE CONNER ILLUSTRATIONS BY PATRIC SANDRI

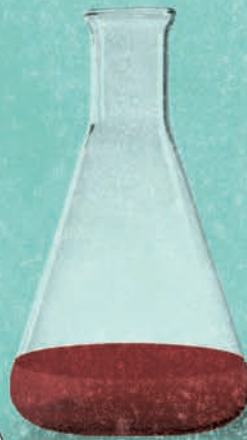


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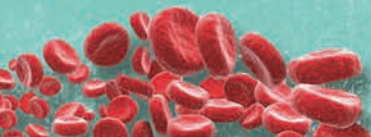
DIGITAL HEALTH

r

e



ted future, but it must have a personal touch



# Roshni Kulkarni, M.D., recalls a time when a patient was attacked by a wild turkey and wanted advice about whether he needed to take his factor.

## LOOKING AHEAD: HOW AI CAN IMPROVE HEALTH CARE

Data is necessary to make health care decisions, but digital health is producing more than humans can handle. Physicians, for example, can receive tremendous amounts of data on a patient — the latest lab numbers, self-reported vitals, and more.

That's where advances in artificial intelligence (AI) are exciting.

"It's impossible for a person to look at thousands and thousands of data points and make sense out of it," says Roshni Kulkarni, M.D., a pediatric hematologist/oncologist. "But I think in the future it may be possible to have some kind of a technology look at and collate the data and give it in a form that people can understand."

Alex Krist, M.D., MPH, a family physician, sees opportunities to use AI to benefit patients as well, helping them process information from their doctor.

"I think AI has potential for that. We're just so new into that realm," he says. "The focus has been creating systems for clinicians, not creating systems for people to use, and we need to change that."

"He was 60 or 70 miles from me, so we did a telemedicine visit. And I could see he had a 6-inch laceration on his leg," she says. "Fortunately, he had just taken his factor the day before, and the injury site looked pretty dry. I brought in my infectious disease specialist, who said the patient needed a tetanus shot."

The ability to use a video screen and internet connection to deliver health care — called telehealth — can be useful in situations such as these, says Kulkarni, who is professor emerita in pediatric hematology/oncology and director emerita for the Center for Bleeding and Clotting Disorders at Michigan State University.

Telehealth is just one example of digital health, a broader movement in health care seeking to use digital tools to enhance the patient and provider experience. And from Kulkarni's perspective, there are many reasons to be optimistic about how it can help people with blood disorders access comprehensive care more conveniently.

But questions remain: How do we overcome some of the challenges inherent in the digital experience? And how can patients and providers best use digital tools to enhance the quality of health care without diminishing the personal touch?

### WHAT IS DIGITAL HEALTH?

One thing complicating conversations around digital health is how vast the landscape is. Digital health is "more than an app on your phone," says Kevin Perea, author of *The Digital Health Revolution* (2019) and founder of TranscendIT Health,

### ESTABLISHING CONNECTION

The goal of digital health is to help people access comprehensive care more conveniently without diminishing the personal touch.



a consulting firm that specializes in digital health. “It’s really a means of connecting you to resources, whether they’re real or virtual.”

Digital tools can include electronic health records, patient portals, mobile applications, telemedicine platforms, data analytics systems, remote monitoring, wearable technologies such as smartwatches, and even artificial intelligence and chatbots.

“As a family physician, I think of digital health as tools that can help clinicians and patients better manage information, be connected, and act on information,” says Alex Krist, M.D., MPH, professor in the Department of Family Medicine and Population Health at Virginia Commonwealth University.

### DIGITAL HEALTH IN ACTION

During the pandemic, many medical practices took advantage of telehealth technologies. For people who didn’t need to be seen in a clinic, it was safer and, in some cases, just as effective to have a conversation via videoconference.

For those with blood and bleeding disorders, regular check-ins with a doctor are important and can lead to better medication adherence, Kulkarni says. But for some people, in-person appointments can be a burden, so the ability to use telehealth can be advantageous.

One of the biggest benefits of telehealth is saving time and money, Kulkarni says. In 2016, she and a colleague published a letter to the editor in the *New England Journal of Medicine* describing their cost analyses. To travel to her clinic for specialty care, people racked up bills averaging around \$1,300 for hotel, gas, food, and time off work. A visit with a specialist using telehealth cost around \$40.

But telehealth isn’t the only application of digital health. For individuals participating in clinical trial research, digital platforms can provide the opportunity to answer survey questions or provide other data from home rather than driving to a clinical care site. This means more people — from various backgrounds and geographic areas — can benefit from new and experimental therapies.

People with chronic conditions also can use digital tools to monitor their health — reporting blood sugar or blood pressure levels or physical activity — and to communicate with their physicians.

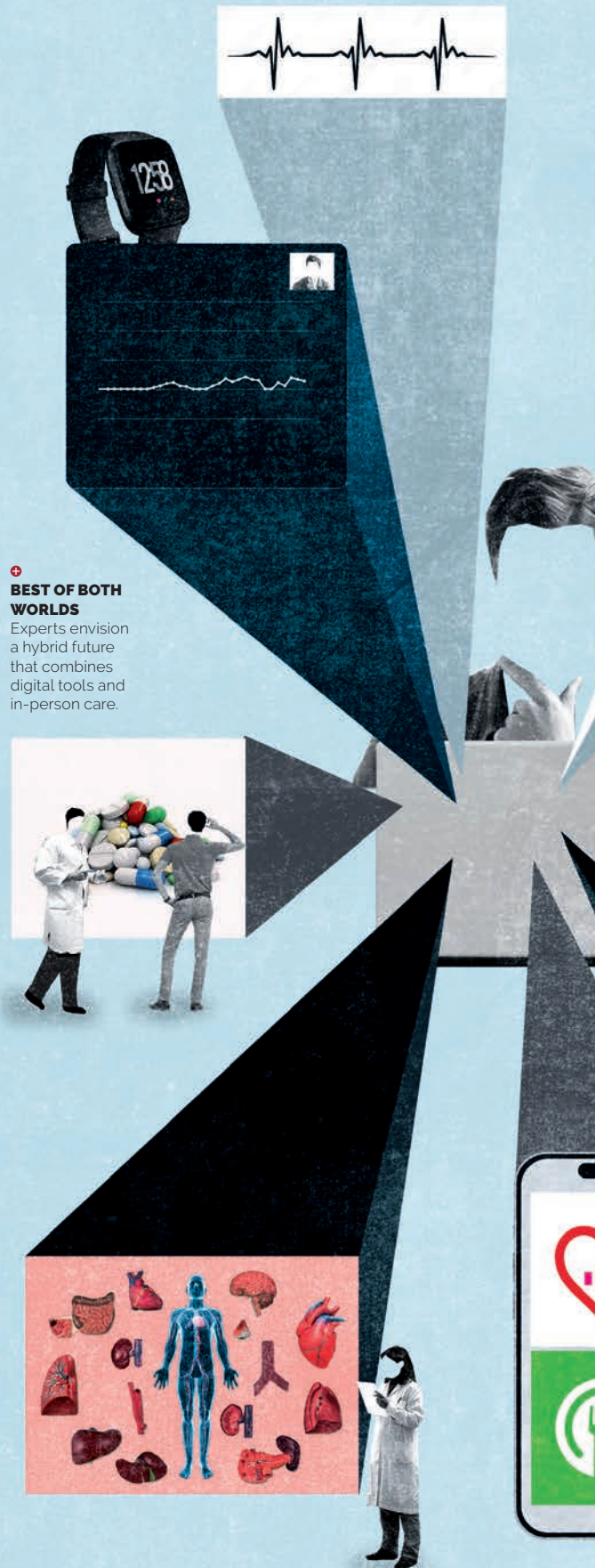
Being able to receive simple messages and educational materials — pictures of the exercises your physical therapist showed you during your appointment or written instructions for a medication — through your provider’s patient portal is another example of digital health tools at work.

### THE CHALLENGES


With billions of dollars invested in digital health each year and digital technologies becoming more powerful and omnipresent, there are reasons to be hopeful, but there are also challenges.

For providers, Kulkarni says, “one disadvantage is you’re not touching and feeling the patient.”

Provider burnout is another concern that’s been compounded by digital health. A September 2022 study in *Mayo*



**BEST OF BOTH WORLDS**  
Experts envision a hybrid future that combines digital tools and in-person care.



## Q&A WITH LEONARD A. VALENTINO, M.D., DIGITAL HEALTH ADVOCATE

The National Bleeding Disorders Foundation (NBDF) believes digital health can provide numerous benefits for people with blood disorders. Leonard A. Valentino, M.D., president and CEO of NBDF, shares his thoughts.

### WHY IS DIGITAL HEALTH AN IMPORTANT PART OF NBDF'S MISSION?

Improving access to care and achieving health equity are critical to our mission of enhancing the health and well-being of people living with inherited bleeding and blood disorders. Digital health strategies are important tools we can use.

### IN WHAT WAYS IS NBDF SUPPORTING THE ADVANCEMENT OF DIGITAL HEALTH AND ADVOCATING FOR THE BLEEDING DISORDERS COMMUNITY?

Our Digital Health Priority Action Team was created to identify opportunities for NBDF to advance health equity using digital strategies. Plus, we are partnering with organizations like the American Telemedicine Association on advocacy and public policy issues to increase access to digital technology and overcome the digital divide (millions of Americans lack access to the internet or to mobile devices). We also hope to partner with hemophilia treatment centers interested in telemedicine and other digital strategies to improve access to care.

*Clinic Proceedings* showed that nearly 63% of physicians were reporting emotional exhaustion and other symptoms of burnout. Burnout is commonly associated with “system inefficiencies, administrative burdens, and increased regulation and technology requirements,” according to the American Medical Association. Corresponding with people via patient portals or taking on additional patients via telehealth can contribute to the overwhelm.

“And it’s not just physicians. It’s all health care providers — nurses, social workers, dietitians, everyone,” notes Kulkarni, who is also the former director of the Division of Blood Disorders for the U.S. Centers for Disease Control and Prevention.

Plus, while a lot of digital health tools are available, it’s uncertain that they are accomplishing their goals.

“It’s understanding what people need, and it’s also improving health outcomes, improving the care delivery process, and improving the health of the health care team,” Krist says. “We have to have data showing that doing these digital health things has all of those different improvements, and we’re not studying it enough to know that.”

### KEEPING THE HUMAN CONNECTION

For patients and providers alike, maintaining human connections remains critical as digital health transforms. Perea says digital companies learned an important lesson in the early 2010s: You can never develop a solution that is in lieu of a person’s doctor.

“Somewhere along the line,” he says, “the telehealth companies figured out that we shouldn’t be competing against the doctor. We should be helping doctors better connect and service their patients.”

Although people used telehealth options extensively during the pandemic, that trend is reversing course, Perea adds, because people have a need to connect. “More people are coming in to see their doctor,” he says, “which I think speaks volumes about how people prefer to consume health care.”

But digital technology can help providers keep in touch with their patients when they’re not face to face. “I think it’ll always be a delicate balance,” Perea says. “You get access to the best resource you can, in the shortest amount of time. Sometimes, that’s going to be in person. Sometimes, that’s going to be virtual.”

Kulkarni envisions a hybrid future: “Sometimes physical, sometimes virtual, but the whole idea is to provide care to the patient.”

The key, Krist says, is finding smart ways to use digital health tools. “When digital health is done right, it aggregates information and it allows people to connect as people,” he says. “Chronic care, like family medicine, should be grounded in a trusting, longitudinal relationship. Effective digital health tools need to build those relationships.”



**HOW TO GET THE RIGHT TREATMENT FOR YOU:** Whether it’s virtually or in person, health care is better when patients and providers work together. Read more about shared decision-making for bleeding disorders: [hemaware.org/shared-decision-making](https://hemaware.org/shared-decision-making)

People in the bleeding disorders community  
to turn their personal challenges and exper

VOICES

FOR

CHANGE





come to advocacy in different ways, but what unites them is the desire to turn experiences into positive change. Here, four people share their stories.

**+ A NEW FOCUS**  
When chronic medical problems prevented Joey Smiles from working, he turned his attention to advocacy. Smiles was photographed by Matt Stanley at the Pennsylvania State Capitol Complex in Harrisburg.

## JOEY SMILES A PERFECT MATCH

**W**hen Joey Smiles, 44, was growing up in the 1980s and '90s, treatment for hemophilia was “on-demand,” as he puts it. He was using factor only after an injury or after feeling symptoms of a spontaneous bleed. Major bleeds required multiple infusions every 12 to 24 hours to resolve.

Smiles, who lives near Scranton, Pennsylvania, eventually became a biochemist, working in research and development of vaccines at a pharmaceutical company. He felt like he had his hemophilia under control at that point. Unfortunately, Smiles went on to experience two major, life-threatening bleeding incidents and continues to have chronic medical problems. He now takes a prophylactic treatment regimen with a prolonged half-life clotting factor. “I’m having no bleeds now,” he says.

His chronic medical problems make it impossible for him to return to the laboratory or even allow him to work a normal schedule. Rather than become depressed or frustrated, Smiles took a different route. “My struggles with hemophilia have allowed me to develop a superpower — adaptability. The reason I’ve been able to lead a semi-normal life is directly related to the miracles of modern medicine,” Smiles says. “So, I wanted to give back. Being no longer able to make my contribution from my lab bench, I knew I needed to adapt, and that’s what ultimately led me to pursuing advocacy.”

Smiles has been advocating on behalf of people with hemophilia and those with other bleeding disorders on the state and federal level with the Eastern Pennsylvania Bleeding Disorders Foundation.

“It was a perfect match because I have experiences from some of the darkest times in hemophilia’s history,” he says. “I think that brings a lot of perspective when you’re telling lawmakers and the public about the whole history of hemophilia.”

Thanks to advances in science and medication, Smiles says the problems he’s encountered are never going to happen to others, as long as people continue to have access to medication. “Medicine for blood disorders is extremely expensive. It’s bad enough to just have hemophilia, but not having your lifesaving medication available makes an already bad situation so much worse,” Smiles says. “To us, it matters a great deal what’s going on with health care, and what bills are being introduced on the state and national level because it could affect us directly.”

Smiles, like many people with bleeding disorders, understands that not everyone is comfortable revealing that they have a bleeding condition, but he urges people to be open about it. “You have a unique voice,” he says, “and telling your story helps all of us in the bleeding disorders community.”

WRITTEN BY CHRISTINA FRANK  
PHOTOGRAPHY BY MATT STANLEY, CARL COSTAS,  
ANGEL ENRIQUE VALENTIN CONCEPCION, AND BRENDAN DAVIS

**“You meet people that you can relate to, and you realize that you’re not alone. If you’ve been misunderstood, you’ll be understood.”**

Fabiola Danastorg Cartagena



### **FABIOLA DANASTORG CARTAGENA** FINALLY UNDERSTOOD

**f**abiola Danastorg Cartagena, 30, didn’t receive a diagnosis of type 1 von Willebrand disease until she was in her early 20s. Until that point, she had been bleeding excessively during her menstrual periods for more than a decade. “I used to have to wear diapers the flow was so heavy,” she says.

The bleeding took a toll on her life, leading to absences from school and work. She also had to deal with having her concerns dismissed. She says that in general people don’t consider period problems a legitimate excuse for missing work or school. “It got to a point where I thought it was all in my head,” she says.

Danastorg Cartagena had been to several gynecologists in search of a diagnosis, but it wasn’t until she was hospitalized at one point that a hematologist confirmed she had von Willebrand disease. She now has a Mirena IUD, known to be effective at treating women with bleeding disorders. She says the intrauterine device has diminished her blood loss by 85%.

For the past four years, Danastorg Cartagena has been involved with the Puerto Rico Association of Hemophilia and Bleeding Disorders. She started as a volunteer and is now secretary of her chapter’s board. She also helped create a women’s committee and participates in support groups that specifically address issues that affect women. “In one meeting, all we talked about was Mirena,” she says.

Danastorg Cartagena encourages women with bleeding disorders to attend meetings and support groups and to get involved with their local chapters. “You meet people that you can relate to, and you realize that you’re not alone. If you’ve been misunderstood, you’ll be understood. It’s a good opportunity to speak up and leave being ashamed at the door,” she says. “You also get to see how important fundraising is and that people are working hard for others not to go through what we’ve been through.”





**+**  
**WOMEN POWER**  
Fabiola Danastorg Cartagena, center, helped create a women's committee at her chapter. She was photographed by Angel Enrique Valentin Concepcion surrounded by her close friends in Parque Luis Muñoz Rivera in San Juan: from left, Zulyanette Rosario Rivera, Lillys Chaparro Morales, Vanessa Diaz Mattei, and Maribel Canales Rosario.

**"I am interested in giving the patient perspective as well as the system perspective."**

Bobby Wiseman



**BOBBY WISEMAN**

## A LIFE-CHANGING CONNECTION

**b**obby Wiseman believes that advocacy comes in many forms, from the personal to the political. As such, the 52-year-old has served in many capacities as an advocate and has been involved in numerous bleeding disorders organizations over the years.

In 2018, Wiseman, who has severe hemophilia B, participated in a gene therapy clinical trial that he says has changed his life and helped advance the science. His hematologist had been keeping an eye out for an appropriate trial. When Wiseman met with the point of contact for a company that was doing a gene therapy trial while attending a hemophilia symposium, he put the two in touch.

“I hopped on the phone, called my doctor, and the two of them connected. The hospital got the paperwork in place and I was selected for the trial. “It was communication, relationship building, and advocacy that made that happen,” he says.

The gene therapy, called Hemgenix, was cleared by the Food and Drug Administration in November. It has been remarkably effective for Wiseman, who hasn’t had to have factor due to a spontaneous bleed in five years; since starting gene therapy, his factor levels typically range between 52% and 62%.

Wiseman also used the opportunity to make recommendations on how to improve the overall trial selection process to include more diverse study participants and reduce bias. “I am interested in giving the patient perspective as well as the system perspective,” he says.

These days, Wiseman, who lives in Tucson, Arizona, devotes his time to Potential of Ability, a nonprofit he founded to help teens and young adults with the various intersectionality of the social determinants of health, and Rising Beyond, where he provides strategic planning and program enhancement. He has one child, 12 godchildren, nieces and nephews, and he became a first-time grandfather in January 2023.

**+**  
**DIVERSITY MATTERS**

Bobby Wiseman has advocated for improving the clinical trial selection process so there’s a more diverse pool of study participants. He was photographed by Carl Costas at home in Tucson, Arizona.

**“We had several years without any problems with our insurance. Then our plan changed. Trying to understand the plan itself and what the cost of the medications would be was so challenging that it got me interested in the behind-the-scenes policymaking and rules.”**

Briana Reinking



**BRIANA REINKING**  
LOBBYING FOR INSURANCE REFORM

**b**riana Reinking, former chair and current advocacy committee member of the National Bleeding Disorders Foundation’s Colorado chapter, became involved in advocacy after her son was diagnosed with hemophilia. A few years later, her second child was also diagnosed.

Reinking, 45, says her primary focus has been on health insurance issues. “We had several years without any problems with our insurance,” she says. “Then our plan changed. Trying to understand the plan itself and what the cost of the medications would be was so challenging that it got me interested in the behind-the-scenes policymaking and rules.”

The chapter works with the Chronic Care Collaborative, a coalition of advocacy groups, which Reinking says has allowed them to have a more prominent voice in the Colorado legislature. In 2022, they were able to help pass a bill prohibiting step therapy.





**HITTING HOME**  
Briana Reinking says involving kids in meetings with legislators helps them better understand the challenges that come with insurance obstacles. She and her sons were photographed by Brendan Davis at home in Centennial, Colorado.

Step therapy is the term used when an insurance company requires people to try the least expensive drug for their condition first; if the drug doesn't work, they may be approved to try a different and often more expensive medication.

"It doesn't allow the patient and the doctor to make the decision about the best medication," Reinking says. "It puts it in the hands of the insurance company and may force someone to try a less effective drug prior to trying one that works. For people with bleeding disorders, taking a less effective drug can cause bleeding episodes that can lead to long term damage to joints and muscles."

The Colorado chapter and the collaborative also helped support legislation to prohibit copay accumulators. "To have those two bills passed has been a really good outcome for the community on a couple of pretty challenging issues," Reinking says. "We've put in a lot of work over many years to build those relationships at the Capitol."

She also stresses how important it is to put a human face on the insurance obstacles that families grapple with.

"Families are often unable to obtain the medication they need, or they have extremely large expenses for medication that they're unable to pay for," she says. "Sometimes they have to make the choice between paying for the medication and paying other bills. Having legislators see how this is actually impacting people in their community is so important."

Reinking recalls bringing her son with her to a meeting with a legislative aide in Washington. "I was explaining the challenges we'd had, but I really was not connecting with him," she says. "Then my son showed the aide pictures of himself playing soccer and learning to rock climb, and the aide kind of lit up and connected with my son. He understood that we're really just trying to get him medication so he can lead as close to a normal life as possible and just be a kid. Legislators are really just people, and they want to hear your story."



# 2023 Awards of Excellence

Congratulations go to these people and organizations, which won the 2023 Bleeding Disorders Conference for dedication to



### PHILANTHROPIST OF THE YEAR

Joseph Alioto, M.D., Chicago, Illinois

Over the past few years, Alioto and his family have given and raised over \$200,000 for research into bleeding disorders treatments and continue to do more each year.



### RYAN WHITE YOUTH AWARD

Owen Crabbe, Buford, Georgia

At the young age of 10, Owen, who lives with a bleeding disorder, has an impressive list of athletic and academic accomplishments.

He competes in soccer, cycling, and swimming while also maintaining straight A's.



### RESEARCHER OF THE YEAR

Donna DiMichele, M.D., Washington, D.C.

For more than 25 years, DiMichele has been a strong advocate for the development of physician-researchers. Her own research has

focused on pediatric hematology, pediatric rare diseases, and biomedical ethics.



### MARY M. GOOLEY HUMANITARIAN OF THE YEAR

Chris Bombardier, MSGH,  
Salem, Massachusetts

Bombardier is the executive director of Save One Life. A highly accomplished mountaineer, he is the first person with hemophilia to climb the Seven Summits, including Mount Everest.



### SOCIAL WORKER OF THE YEAR

Lauren Dunn, MSW,  
Richmond, Virginia

Dunn has been a key figure in helping to pass the Skilled Nursing Facilities Access Act, creating a statewide assessment for bleeding disorders.



### GENETIC COUNSELOR OF THE YEAR

Kaylee Dollerschell, MS, CGC,  
Denver, Colorado

Dollerschell provides genetic counseling services at the University of Colorado Hemophilia & Thrombosis Center, serving as the first genetic counselor at this hemophilia treatment center.



### PHYSICAL THERAPIST OF THE YEAR

Lora Joyner, MS, PT, PCS,  
Pitt County, North Carolina

Joyner's impact on colleagues within the medical community, in the bleeding disorders community, and with patients and families is evident to those she has worked with throughout the years.



### NURSE OF THE YEAR

Jennifer Donkin, DNP, PNP,  
Los Angeles, California

Donkin has helped international patients, received numerous certifications, conducted original research, co-authored many papers, regularly volunteers with her local chapter, and is an outstanding nurse in every way.





# Honorees

ere recognized at  
ur community



### ADVOCATE OF THE YEAR

Julie Fredricksen Jones, Dallas, Texas

Jones has been the executive director of Texas Central Bleeding Disorders since November 2018 and has volunteered with the group since 2010 in various roles.



### PHYSICIAN OF THE YEAR

Doris Quon, M.D., Ph.D., Los Angeles, California

Quon is a tireless advocate for her patients at the Luskin Orthopaedic Institute's Orthopedic Hemophilia Treatment Center in Los Angeles. For

more than 20 years, she has gained knowledge in product coverage and dosing to care for orthopedic patients before, during, and after surgery and during rehabilitation.



### CHAPTER VOLUNTEER OF THE YEAR

Sean Pentz, San Diego, California

Pentz has shown dedication to the Hemophilia Association of San Diego County's mission and has served as a valuable resource for his local commu-

nity. Pentz, who lives with hemophilia, is a longtime advocate who has loaned his voice and experience to raise awareness, advocate for policy and legislation, and educate others.



### LORAS GOEDKEN OUTSTANDING LEADERSHIP AWARD

Joe Mickeliunas, Omaha, Nebraska

President of the Nebraska Chapter's Advisory Board, Mickeliunas has the power to change the world! As a

teacher by profession, he uses his educational skills to raise awareness of bleeding disorders.



### LIFETIME ACHIEVEMENT AWARD

Judith Baker, DrPH, MHSA

Camarillo, California

SINCE 1990, BAKER HAS MADE AN IMPACT ON THE BLEEDING DISORDERS COMMUNITY AS REGIONAL COORDINATOR FOR THE WESTERN STATES REGIONAL HEMOPHILIA NETWORK; ADMINISTRATOR OF GRANTS FROM THE HEALTH RESOURCES AND SERVICES ADMINISTRATION AND THE CENTERS FOR DISEASE CONTROL AND PREVENTION; A RESEARCHER; AN ADVOCATE FOR THE NBDF'S NURSING, PHYSICAL THERAPY, SOCIAL WORK, AND BEHAVIORAL HEALTH WORKING GROUPS; AND A FRIEND TO MANY IN THE COMMUNITY.

### ZIGGY DOUGLAS AWARD FOR INNOVATION IN YOUTH PROGRAMMING

Colorado Chapter of the National Bleeding Disorders Foundation, Denver, Colorado

Through educational programs and youth-led conferences, the Colorado chapter encourages connection and growth for young community members.

### VAL BIAS AND TODD SMITH INNOVATION IN CAMP AWARD

Camp Hot-to-Clot

Western Pennsylvania Bleeding Disorders Foundation, Pittsburgh, Pennsylvania

This award recognizes Camp Hot-to-Clot for its innovative camp activities and educational experiences.

### JOHN INDENCE AWARD

Western Pennsylvania Bleeding Disorders Foundation, Pittsburgh, Pennsylvania

This chapter put its own creative spin on the Bleeding Disorders Awareness Month theme, "Start the Conversation," and used social media, community members, and other resources to highlight pertinent topics such as advocacy, mental health, and access to care.

# 2024 World Congress Is Coming to Madrid

It will be the first fully in-person conference since the pandemic



**PLAN NOW**  
The annual event will be held April 21-24.

The World Federation of Hemophilia (WFH) is happy to announce a return to a fully in-person meeting next year at the WFH 2024 World Congress, which will be the year's most comprehensive international event on bleeding disorders.

The Congress will be held April 21-24 in Madrid. The city's vibrant energy, rich culture, and accessible location in Europe make it the perfect meeting place for the global bleeding disorders community. Participants can expect to be treated to a warm welcome by La Federación Española de Hemofilia (Fedhemo), the Spanish WFH national member organization and the Congress host.

If you have a bleeding disorder or are a family member, health care professional, researcher, policymaker, advocate, or industry member, the WFH 2024 World Congress is for you! The program will cover the latest developments and innovations in our field, from health care issues to the challenges ahead for the management and treatment of hemophilia, von Willebrand disease, rare factor deficiencies, and inherited platelet disorders. This aspect of the Congress — bringing the best and brightest in our field together in one place — is critical to fostering new ideas and approaches.

By going to the WFH 2024 World Congress, you will be helping the WFH continue to deliver the programs and events that are so critical to our community, especially in countries that have limited resources. The Congress will bring together people from all specialties and interests, including patients, hematologists, pediatricians, orthopedic surgeons, physiatrists, physiotherapists, dentists, nurses, social workers, psychologists, geneticists, laboratory technicians, researchers, scientists, policymakers, regulators, advocates, and industry members.

## A TIME TO CONNECT

Everyone who takes part will have a special opportunity to network, collaborate, share knowledge, and problem-solve with like-minded colleagues from around the world. There will be many chances for participants to reconnect with old friends they might not have seen for years — and make new ones.

The Congress program will cover the latest medical and multidisciplinary developments. There will also be five professional track sessions in the fields of dentistry, laboratory sciences, musculoskeletal care, psychosocial care, and nursing. The progress of WFH programs will also be covered, including the WFH Path to Access to Care and Treatment (PACT) Program, the WFH Humanitarian Aid Program, the World Bleeding Disorders Registry (WBDR), the Gene Therapy Registry (GTR), and many other WFH initiatives. And, of course, a rich social calendar will help participants get the most out of their time in Madrid.

The Congress will take place in the IFEMA Madrid Convention Centre, which is strategically located in the northeast of the city. The venue is easily accessible by taxi, and the Madrid airport is just five minutes away. The venue is also accessible for people with reduced mobility.



LEARN MORE: [wfh.org](http://wfh.org)

# Serving Hope and Joy

Maggie Carruth and Lucas Taylor's Rally for Teddy pickleball tournament raised thousands for the foundation

Teddy Taylor's parents were both top-tier college athletes, so organizing a sporting event to benefit the National Bleeding Disorders Foundation was a natural fit. Teddy, 2½, has severe hemophilia A, which was diagnosed right after he was born.

Maggie Carruth and Lucas Taylor had been thinking about doing a fundraiser when Taylor's friend, Ben Van Hout, reached out and suggested a pickleball tournament. Van Hout and Taylor had been on the tennis team together at Northern Arizona University. Van Hout

is now the director of operations for Dynamic Universal Pickleball Rating (DUPR), a rating system for pickleball players worldwide.

"It was a perfect fundraising idea because the sport is growing so much, and it's a fun thing to do," Taylor says.

The Rally for Teddy pickleball tournament was held July 8 in Jackson Hole, Wyoming, and raised \$12,000 for the foundation, exceeding its goal by \$2,000.

The organizers publicized the event via social media, flyers, and word-of-mouth.

Players registered for the tournament through the DUPR app. There were five events: men's doubles, women's doubles, mixed doubles, singles, and a 60-plus event. "In total, we had over 100 event sign-ups and about 70 individual people compete," Taylor says. Donations and raffle tickets also contributed to the fundraising total.

"I still have quite a few connections through the tennis world," Taylor says. "One of my friends works for a company called Head, and they sponsored the event. They sent a ton of paddles and a ton of pickleballs, so we handed those out to event winners and for raffle prizes." A number of local businesses also supported the event by donating restaurant vouchers and hockey season tickets. "We had great support from the local community," he says.

Taylor and Carruth plan to put on the event again next year, and Taylor is hopeful that people with hemophilia may be able to join. "Pickleball is definitely a sport that anyone can play. I know people with hemophilia are typically blocked out of certain sports, but the end goal is for them to be able to participate in as many sports as possible," he says.

Carruth stresses how much her family appreciates NBDF, and how eager they are to continue supporting the foundation. "We are really passionate about raising awareness around hemophilia and all bleeding disorders, and grateful for all of the work that other parents have done in the past to get the treatment where it is now. Without all of that advocacy, there might not be as much funding and research as there is now."

—By Christina Frank

#### DINKING DELIGHT

The event capitalized on a popular, fun activity.



COURTESY OF THE TAYLOR FAMILY



FOR INFORMATION ABOUT PLANNING  
A FUNDRAISER FOR THE FOUNDATION:  
[hemophilia.org/give/fundraise](http://hemophilia.org/give/fundraise)

# Every Step Counts

From May to November, Unite for Bleeding Disorders walks take place in more than 40 cities across the country to raise funds and awareness for local chapters and help them increase education, support services, and advocacy efforts in their community. Here's a recap from four chapters that held their walks earlier this year.

## GATEWAY HEMOPHILIA ASSOCIATION

On July 16, Gateway Hemophilia Association held their 14th annual walk with over 250 participants. A total of 25 teams raised \$73,000. "Every dollar raised is a step in the right direction," says Executive Director Bridget Tyrey.

**Learn more about the Gateway Hemophilia Association chapter:** [gatewayhemophilia.org](http://gatewayhemophilia.org)



### GOOD CLEAN FUN

The Bubble Bus was a hit with younger participants.



## PACIFIC NORTHWEST BLEEDING DISORDERS

Pacific Northwest Bleeding Disorders' walk on June 3 featured 23 fundraising teams made up of 165 participants. "Our goal was set at \$68,000 and we raised \$73,000," says Fundraising and Events Manager Michelle Fernandez.

**Learn more about the Pacific Northwest Bleeding Disorders chapter:** [pnwbd.org](http://pnwbd.org)



## HEMOPHILIA OF INDIANA

Hemophilia of Indiana's walk on May 20 was "small but mighty," says Development and Event Director Kristy McConnell. "We had fewer walkers than in the past, but we increased our number of teams and exceeded our goal of \$40,000 and ended up raising \$43,294."

**Learn more about the Hemophilia of Indiana chapter:** [hoii.org](http://hoii.org)



## MIDWEST HEMOPHILIA ASSOCIATION

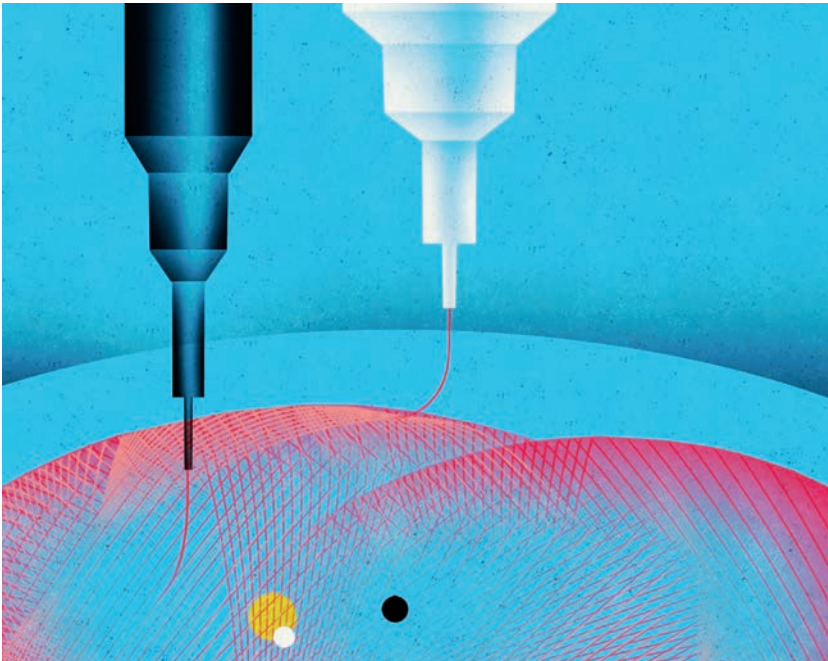
Midwest Hemophilia Association's walk on June 3 featured 73 participants and 15 teams and raised \$25,900. "This year's highlights were having the Just-Us League, a cosplay charity organization in Kansas City, kick off the walk, and the Bubble Guy," says Executive Director Angela Brown.

**Learn more about the Midwest Hemophilia Association:** [midwesthemophilia.org](http://midwesthemophilia.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 chapters in future issues. If you'd like to feature your chapter's event in a future issue of *HemAware*, please email [Donna.Behen@Manifest.com](mailto:Donna.Behen@Manifest.com).



**RESULTS**  
There was a 96.8% reduction in factor VIII usage overall compared with base-line data.

# Gene Therapy for Hemophilia A Gets FDA Approval

The one-time treatment is administered as a single dose via IV infusion

In June, the U.S. Food and Drug Administration approved BioMarin’s ROCTAVIAN™ (valoctocogene roxaparvovec-rvox) gene therapy product for the treatment of adults with severe hemophilia A without antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved diagnostic test. This companion test is designed to help health care providers identify patients who may benefit from receiving ROCTAVIAN. It effectively screens for preexisting anti-AAV5 antibodies, which may render the therapy less effective or ineffective.

FDA approval was based largely on the positive durability, efficacy, and safety results from the global Phase 3 GENER8-1 study, which has been fully enrolled since November 2019, with 134 participants ultimately receiving a one-time dose of therapy. Results showed an overall 82.9% reduction in treated bleeds and a 96.8% reduction in factor VIII usage overall compared with base-line data. Trial participants will continue to be monitored closely.

“Adults with severe hemophilia A face a lifelong burden, with frequent infusions and a high risk of health complications,

**“The first gene therapy for severe hemophilia A has the potential to transform the way we treat adults.”**

including uncontrolled bleeding and irreversible joint damage,” says Steven Pipe, M.D., a professor of pediatrics and pathology at the University of Michigan and an investigator in the Phase 3 study. “The approval of ROCTAVIAN as the first gene therapy for severe hemophilia A has the potential to transform the way we treat adults based on years of bleed control following a single, one-time infusion.”

Peter Marks, M.D., Ph.D., director of FDA’s Center for Biologics Evaluation and Research, says, “Hereditary hemophilia A is a potentially serious bleeding disorder. Severe cases of hemophilia A can cause life-threatening health issues due to increased risk of uncontrolled bleeding. Today’s approval represents an important advance in providing treatment options for patients with this bleeding disorder, and treatment with gene therapy may reduce the need for ongoing routine therapy.”

## Ad Index

BioMarin .....	C2-2 roctavian.com
Spark Therapeutics .....	.6-7 HemophiliaForward.com
CSL Behring .....	.12-13 hemgenix.com
Medical College of Wisconsin .....	.19 abaim@mcw.edu
Sanofi .....	.C3-C4 altuviiio.com

# Riding High

After a sedentary childhood in Vietnam, I'm now passionate about staying physically active

When I was an infant, I developed purple and pink bruises all over my body. My parents dragged me from doctor to doctor in Vietnam, where we lived, but none of the health care workers, nor anyone in my family, had seen this before. Finally, when I was about a year old, I was diagnosed with hemophilia.

There's so much I could say about growing up with hemophilia in a country with inferior health care, but to sum it up in one word, I'd have to say: pain. When I was in my teens, for example, I experienced a bleed in my stomach. The ache was so intolerable, I couldn't stand, sit, or even lie down without feeling excruciating agony, and the only painkiller I had access to was ice. All I could

do was come up with something to distract myself, so I bit my fingers.

The medications we did have in Vietnam, like clotting factor, still contained viruses like hepatitis C and HIV, and they caused terrible side effects. Every time I took the clotting factor, my body would go into shock. Sometimes that meant uncontrollable itching. Other times, I'd get super cold. Even if it was the middle of the summer, I would ask my parents to pour warm water on me to heat up my body. Once, the shock was so strong that I nearly passed out, and after about half an hour, my left leg went numb. To this day, the nerves in my left leg are affected.

My family led a very frugal life, and every extra dollar went to buying treatments for me. But still, most of my childhood was spent sitting in my house, watching the kids play outside and wishing I could run around with them.

Eight years ago, at the age of 26, I moved to Las Vegas, and things changed dramatically. Doctors told me that it's important to stay active when you have hemophilia.

"How is that possible?" I asked. "I have hemophilia."

They said that in this country, we have medications that can help control the bleeding. Knowing that, I was able to find a life I'd never had before. I started off doing easy things like walking and swimming, then I moved to dance competitions, though those were tough on my joints.

Then I discovered mountain biking. I know what you're thinking ... that mountain biking is dangerous. But really, it's less treacherous than road biking because you ride pretty slowly on a mountain bike, and if you do crash, you'll fall onto soft ground, with no chance of being hit by a car.

Although I admit, there are two states in the sport. The first is going up the hill. It's hard work, and I sweat and curse and wonder, Why am I doing this to myself?

But then I reach the top of the hill, and there's a sunrise or a sunset or a beautiful line of trees. And finally, I go down the hill, and I feel the breeze in my face and smell the scent of the grass. And the reward for all that hard work is truly spectacular.

When I'm not mountain biking, I'm usually working on a graphic design project. I design logos and other marketing materials for mom-and-pop businesses around Vegas.

I know people think of hemophilia as a curse. And in some ways, I guess it is. But to me, it was a blessing. Because I learned how to take care of myself from a young age, and to truly appreciate all these little moments.

—By Nghia Nguyen, as told to Leslie Pepper



#### GRATEFUL

Nghia Nguyen now lives an active life thanks to proper treatment.



**Patient Information****Rx Only****ALTUVIIIIO™ (al too'vee oh)****[antihemophilic factor (recombinant), Fc-VWF-XTEN fusion protein-eh]]****for intravenous use after reconstitution only****Single-dose vial**

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

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

Do not attempt to give yourself an injection 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 injecting ALTUVIIIIO so that your treatment will work best for you.

**What is ALTUVIIIIO?**

ALTUVIIIIO is an injectable medicine that is used to control and reduce the number of bleeding episodes in people with Hemophilia A (congenital Factor VIII deficiency).

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

**Who should not use ALTUVIIIIO?**

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

**What should I tell my healthcare provider before using ALTUVIIIIO?**

Talk to your healthcare provider about:

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

**How should I use ALTUVIIIIO?**

You get ALTUVIIIIO as an injection into your vein. Your healthcare provider will instruct you on how to do injections on your own, and may watch you give yourself the first dose of ALTUVIIIIO.

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

**What are the possible side effects of ALTUVIIIIO?**

You can have an allergic reaction to ALTUVIIIIO. 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 ALTUVIIIIO. This can stop ALTUVIIIIO from working properly. Your healthcare provider may give you blood tests to check for inhibitors.

The common side effects of ALTUVIIIIO are headache, joint pain, and back pain.

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

**What are the ALTUVIIIIO dosage strengths?**

ALTUVIIIIO comes in seven different dosage strengths with 3 mL sterile water for injection (SWFI). The actual number of international units (IU) of Factor VIII activity in the vial will be imprinted on the label and on the box. The seven different strengths are as follows:

Strength	Cap Color
250 IU	Yellow
500 IU	Red
750 IU	Garnet
1000 IU	Green

**(continued)**

Strength	Cap Color
2000 IU	Royal Blue
3000 IU	Mist Grey
4000 IU	Orange

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

**How should I store ALTUVIIIIO?**

- Keep ALTUVIIIIO in its original package.
- Protect it from light.
- Do not freeze.
- Store refrigerated 2°C to 8°C (36°F to 46°F) up to 48 months or at room temperature [not to exceed 30°C (86°F)], for a single period up to 6 months. Do not use ALTUVIIIIO after the expiration date printed on the label and carton of each vial.
- When storing at room temperature:
  - Note on the carton the date on which the product is removed from refrigeration.
  - Use the product before the end of this 6-month period or discard it.
  - Do not return the product to the refrigerator.

After mixing with the diluent:

- Do not use ALTUVIIIIO if the mixed solution is not clear and colorless to slightly yellowish.
- Use mixed product as soon as possible.
- You may store mixed ALTUVIIIIO at room temperature, not to exceed 30°C (86°F), for up to **3 hours**. Protect the mixed ALTUVIIIIO from direct sunlight. Discard any mixed ALTUVIIIIO not used within 3 hours.

**What else should I know about ALTUVIIIIO?**

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

This Patient Information has been approved by the US Food and Drug Administration.

Manufactured by:  
Bioverativ Therapeutics Inc.  
Waltham, MA 02451  
A SANOFI COMPANY  
US License Number 2078

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A FIRST-IN-CLASS, ONCE-WEEKLY INFUSION

**ALTUVIIIIO™**  
Antihemophilic Factor (Recombinant),  
Fc-VWF-XTEN Fusion Protein-ehtl

# FACTOR UP with ALTUVIIIIO™

Higher-for-longer Factor VIII levels in the near-normal to normal range (**over 40%**) for most of the week



## HIGHER FACTOR LEVELS FOR LONGER

Above 40% for most of the week (near-normal to normal range).<sup>\*†</sup>

**48**

## HOUR HALF-LIFE IN ADULTS

In a Phase 3 study,<sup>†</sup> ALTUVIIIIO offered adults the longest half-life of any Factor VIII therapy.

**0.7**

## BLEEDS PER YEAR<sup>‡</sup>

Mean annual bleed rate observed in 128 people previously treated with prophylaxis therapy.<sup>‡</sup>

<sup>\*</sup>Average trough levels were 18% for adults 18 years and older, 9% for adolescents aged 12 years to under 18 years, 10% for children aged 6 years to under 12 years, and 7% for children aged 1 year to under 6 years.

<sup>†</sup>159 adults and adolescents with severe hemophilia (aged 12 years and older) were enrolled in the XTEND-1 study; 133 people were in Group 1, and switched to ALTUVIIIIO prophylaxis from prior prophylaxis therapy. Efficacy of prophylaxis was evaluated in 128 of these patients.

<sup>‡</sup>Data based on treated bleeds.

## INDICATION

ALTUVIIIIO™ [antihemophilic factor (recombinant), Fc-VWF-XTEN fusion protein-ehtl] is an injectable medicine that is used to control and reduce the number of bleeding episodes in people with hemophilia A (congenital Factor VIII deficiency).

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

## IMPORTANT SAFETY INFORMATION

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

Do not attempt to give yourself an injection 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 injecting ALTUVIIIIO so that your treatment will work best for you.

### Who should not use ALTUVIIIIO?

You should not use ALTUVIIIIO if you have had an allergic reaction to it in the past.

### What should I tell my healthcare provider before using ALTUVIIIIO?

Tell your healthcare provider if you have had any medical problems, take any medications, including prescription and non-prescription medicines, supplements, or herbal medicines, are breastfeeding, or are pregnant or planning to become pregnant.

### What are the possible side effects of ALTUVIIIIO?

You can have an allergic reaction to ALTUVIIIIO. 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 ALTUVIIIIO. This can stop ALTUVIIIIO from working properly. Your healthcare provider may give you blood tests to check for inhibitors.

The common side effects of ALTUVIIIIO are headache, joint pain, and back pain.

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

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

Please see brief summary of Patient Information on the previous page.

## Connect with your CoRe today

Sanofi Hemophilia Community Relations and Education (CoRe) Managers provide information about ALTUVIIIIO, living with hemophilia, and treatment options.



Sign up to connect  
and learn more today!

sanofi

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