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NBDF's new president and CEO joined the organization in February.

p.30 | ADHERENCE MATTERS
Not following treatment regimens remains a major problem for patients and providers.

p.34 | STEPS FOR LIVING MAKEOVER
NBDF's premier informational website is being updated with new and comprehensive content.

HEMAWARE

The Bleeding Disorders Magazine

NATIONAL BLEEDING DISORDERS FOUNDATION | hemaware.org

WHEN HEALTH INSURERS DENY DRUG COVERAGE, PATIENTS AND FAMILIES HAVE TO SCRAMBLE FOR SOLUTIONS

THE IMPACT OF FORMULARY RESTRICTIONS

p.24

SPRING / SUMMER 2024

HEMGENIX®
etranacogene dezaparvovec-drlb

HEMGENIX HAS BEEN ADMINISTERED ACROSS THE U.S.

Meet Andrea, who's part of a team administering HEMGENIX

HEMGENIX is a one-time infusion offering elevated and sustained factor IX levels for years. HEMGENIX was approved by the FDA in 2022, but it's been studied for many years.

We talked with Andrea, APNP, at a center in Wisconsin about treating people with hemophilia B over the last year with HEMGENIX. Andrea also currently sits on the Region V-West Regional Council and Regional Gene Therapy Working Group.

Please note that Andrea is sharing her patients' experiences and others' experiences may vary.

When you first heard about the approval of HEMGENIX as the first gene therapy for hemophilia B, what did you think?

Initially there are a lot of thoughts that go through your mind. It's a new treatment, it's novel. We've never done this before outside of clinical trials.

We dug into the clinical data to make sure that it was something we felt was going to be safe and work well for our patients. I think the data showing the factor levels after 3 years is very exciting. But looking at the consumption of factor greatly decreasing after treatment with HEMGENIX really told us more. So that made HEMGENIX a very viable option for us to put in our toolbox.

What was it about the people you treated that made them good candidates for HEMGENIX?

Those we treated were born with hemophilia B, were very afraid of needles, still experienced bleeds, and even as young men they had identified target joints. Also, they were very motivated and excited to know that there was a treatment option that could potentially improve their quality of life.

They had not been fully compliant with their factor infusions before—and I have heard from others that a person who's not compliant is not the best fit due to the required post-monitoring—but that is not something that would make us withhold treatment. It just meant we had to have more conversations around the decision to move forward.

> **"Their factor IX levels started coming up after the first week, and they have been able to stop prophylaxis after week two."**

What is your process leading up to Infusion Day?

It is about a 4-month process from the time our patients decide they want to move forward with HEMGENIX until Infusion Day.

For the patients we've treated at the center, we completed an initial consent form and brought them in right away to do their AAV5 neutralizing antibody testing. Even though this wouldn't have excluded

them, we wanted to make sure they didn't have abnormally high levels. We set up their labs and liver diagnostics in the interim, and chose to do a psychosocial assessment. We did a lot of education with each person, requiring them to teach back to us and make sure they understood the process.

While the eligibility tests are underway, how is the coverage process?

I think the HEMGENIX process is very well designed, and we have a lot of help along the way from CSL Behring and are able to reach out to them at any time. Our patients have found the HEMGENIX Connect℠ program helpful with the benefits investigation and providing education on CSL Behring's travel assistance program.

While we are waiting for the eligibility labs and diagnostics to get done, I start writing the letters of medical necessity and gathering historical notes, so that as soon as we have all the test results, we can send the packet off to the insurance company.

> **"They will tell you, hands down, it was the best decision that they could have made."**

What have your Infusion Days been like so far?

Infusion Days have been very exciting! Our policy for anyone undergoing gene therapy is to visit us the day before for one final check-in. They stay in town overnight, and then arrive the next morning, ready for their infusions.

I think most people will say there may be a few nerves when you're doing a new therapy, but I don't think we could have asked for better days for the infusions. We have not had any infusion reactions and when the patients have finished there have been lots of hands in the air and cheers. We have our patients stay in town overnight for a follow-up visit the next day just to make sure things are going well. We then provide them with calendars with their weekly lab monitoring for the next 3 months and choose to send them home with steroids in the event they need them.

How have your patients responded to treatment with HEMGENIX?

The people I've treated have been early responders—their factor IX levels started coming up after the first week, and they have been able to stop prophylaxis after week two. They have had some liver enzyme elevations and took steroids to bring those levels back down.

Patients almost a year out are still experiencing fantastic results. They've remained off prophylaxis. They've had some injuries that, in the past, would have caused a bleed, but none required any factor IX infusions.

This truly has made an impact on their lives. If you ask them, it's more than anyone ever imagined it could be for them. They will tell you, hands down, it was the best decision that they could have made.

What have your patients told you was the biggest difference they have felt during their post-administration visits?

They always say they can't believe they've gone this long without a factor IX infusion. Patients have had some injuries in that time frame, and they were in a little bit of shock over the fact that those injuries didn't bleed like they did prior to HEMGENIX.

If someone with hemophilia B is considering HEMGENIX, what advice would you give them?

I would advise talking with every person with hemophilia B about their quality of life. What their goals are for themselves and their bleeding disorder, and making sure that they know now that there is this option out there that could help them meet those goals. There are great resources from CSL Behring, and HEMGENIX Connect is available to support them along the way.

This interview has been edited for length and clarity.

Please see Important Safety Information on the next page and the brief summary of prescribing information for HEMGENIX on page 14.

HEMGENIX®
etranacogene dezaparvovec-drlb

CSL Behring

IMPORTANT SAFETY INFORMATION

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- 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 at HEMGENIX.com.

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

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

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CSL Behring

www.CSLBehring.com **www.HEMGENIX.com** **USA-HGX-0700-APR24**

Inside

Features

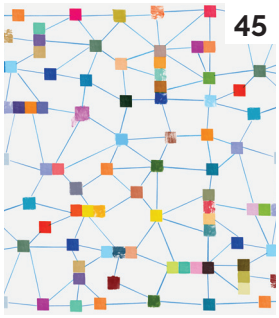
For the first time since its launch in 2011, the Steps for Living website is getting a complete makeover.

On the Cover

The Romans family, of Gretna, Nebraska

Photography by **Colin Conces**

Inside



In Every Issue

Living Well

Community in Action

Explore Head-to-Head Pharmacokinetic (PK) Study Data

See half-life, clearance, and other PK data from the crossover study comparing Kovaltry® and Advate®.

Visit Kovaltry-us.com to find out more.

Kovaltry®

Antihemophilic Factor (Recombinant)

In Focus

PRESIDENT AND CEO PHIL GATTONE, M.ED.

"Our strength lies not in our individual endeavors but in our collective unity."

We Are Stronger Together

Since joining the National Bleeding Disorders Foundation (NBDF) in February, I have been inspired by the unwavering dedication displayed by our incredible staff, over 50 chapters, board members, advocates, and community partners. Each of you plays a vital role in the fabric of our community, and your never-ending commitment continues to motivate us all.

"Never forget that NBDF is here with you, and we'll stay with you every step of the way." These words encapsulate the essence of our commitment to those we serve. Whether you're facing a diagnosis, navigating treatment options, or seeking support and advocacy, NBDF is here for you. We are more than an organization. We are a family, bound by compassion, empathy, and a shared determination to make a worldwide difference in the blood and bleeding disorders community. At NBDF, we understand the daily journey, uncertainties, and setbacks that can happen while living and thriving with a bleeding disorder. But it is precisely during these times that our unity shines brightest.

As we continue to embrace transformative shifts, such as our new bleeding.org web domain name, and celebrate our unwavering commitment to all people at NBDF's Bleeding Disorders Conference on Sept. 12-14 in Atlanta, let us continue to enfold the power of unity and celebrate together as a community.

Remember, our strength lies not in our individual endeavors but in our collective unity. Because when we stand shoulder to shoulder, united in our purpose to innovate, educate, and advocate, we will truly ensure that every voice is heard, needs are met, and rights are protected.

Online | hemaware.org

National Bleeding Disorders Foundation: bleeding.org
Facebook: @NationalBleedingDisordersFoundation
X: @nbd_foundation and @NBDFespanol
Instagram: @nbd_foundation
TikTok: @natlbdfoundation
YouTube: youtube.com/@NBDFvideo
Advocacy: bleeding.org/advocacy
Steps for Living: stepsforliving.hemophilia.org
Victory For Women: victoryforwomen.org
Better You Know: betteryouknow.org

HEMAWARE
hemaware.org

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PHOTOGRAPHY BY NATALIA WEEDY

Learn the science of gene therapy for hemophilia

Discover HEMHORIZON

Pfizer

Pfizer gene therapy for hemophilia A is currently not approved.

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™ THERAPEUTICS

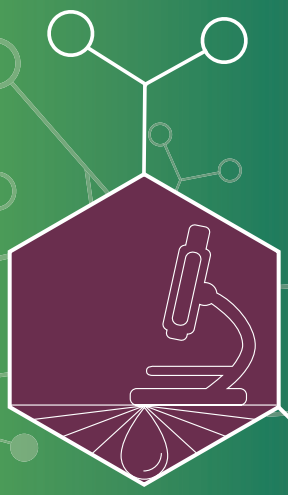
Interested in enrolling in a Spark-sponsored hemophilia gene therapy clinical trial?

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.

HEMOPHILIA FORWARD®

Explore frequently asked questions about hemophilia gene therapy clinical trials.

See a demo about the science of gene therapy.

Visit HemophiliaForward.com

Spark™ THERAPEUTICS

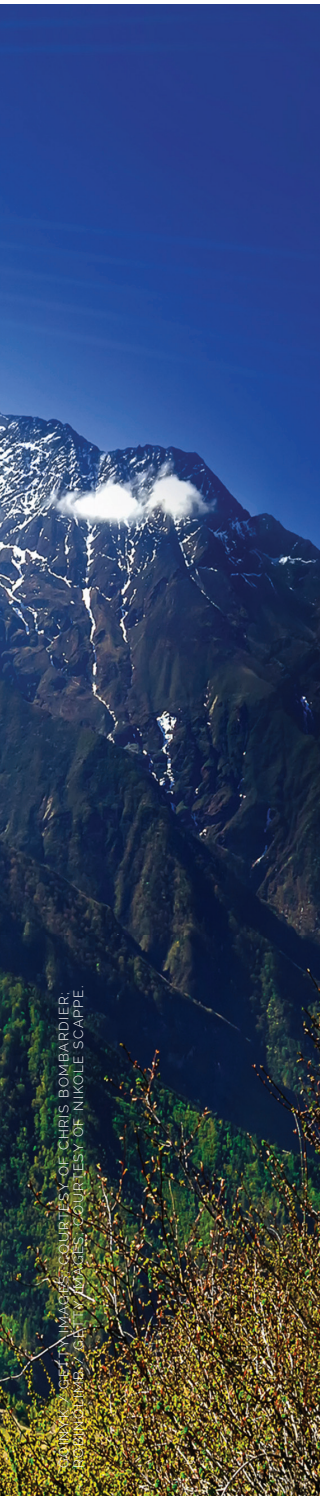
Healthy Start

STATS, FACTS, AND NEWS YOU CAN USE

Living with hemophilia has made me stronger and more determined to live life to the fullest."

—CHRIS BOMBARDIER

Bombardier is the first person with hemophilia to successfully climb the Seven Summits, including Mount Everest. Read more about him at **hemaware.org/bombardier**.



PHOTOS: GETTY IMAGES; COURTESY OF CHRIS BOMBARDIER; FROM LEFT: GETTY IMAGES; COURTESY OF NIKOLE SCAPPE

Election Year Toolkit

"This is a critical election year, and your vote in state and national elections can have a strong impact on our community's access to health care," says Bill Robie, director of state government relations for NBDF. Here's how you can get involved:

REGISTER TO VOTE

Every state except North Dakota requires people to register if they want to become voters. Depending on your state, the registration deadline could be as much as a month before an election. Find your state's voter registration deadlines at *usvotefoundation.org*.

QUESTION THE CANDIDATES

"If somebody comes knocking on your door asking for your vote, this is an opportunity for you to educate them about bleeding disorders," Robie says. "You can start by asking them, 'What do you know about rare diseases like hemophilia and other bleeding disorders, and some of the challenges that patients face?'"

FIND OUT WHEN YOU CAN VOTE

As of March 2024, 46 states, the District of Columbia, Guam, Puerto Rico, and the Virgin Islands offer early in-person voting to all voters (this includes states with all-mail elections). Four states — Alabama, Delaware, Mississippi, and New Hampshire — do not offer early in-person voting, although they may provide options for eligible absentee voters. For details about the rules in your state, visit *vote.org/early-voting-calendar*.

MEET AN NBDF STAFFER

Nikole Scappe, Manager of Education

WHAT DO YOU DO TO STAY HEALTHY AS YOU MANAGE YOUR BLEEDING DISORDER?

While living and thriving with von Willebrand disease (VWD), I prioritize both my physical and mental well-being by engaging in strength training three times a week and incorporating some form of movement on other days. Ensuring that I take my bleeding disorder medication regularly helps me feel my best and prevents any unexpected bleeding episodes.

WHAT'S BEEN YOUR BIGGEST CHALLENGE IN MANAGING YOUR BLEEDING DISORDER, AND HOW HAVE YOU ADDRESSED IT?

Accessing the appropriate factor medication has been my biggest challenge. I encountered resistance when I was recommended a medication that didn't suit my needs. Taking matters into my own hands, I became my own advocate. I meticulously documented my frequent bleeding episodes and captured them with photographs. Presenting this detailed information at my hemophilia treatment center (HTC) was pivotal in advocating for my case and ultimately enabled me to access the factor medication that was most effective for me.

WHAT ADVICE DO YOU HAVE FOR SOMEONE WHO IS NEWLY DIAGNOSED WITH VWD?

Actively ask questions and seek connections within the bleeding disorders community, including your HTC, local chapter, and other affected individuals and their support networks. It's also crucial to engage with your local chapter and meet other affected community members.

FIND MORE ADVICE FROM NIKOLE:
hemaware.org/scappe

HEMGENIX®
etranacogene dezaparvovec-drlb

A FUTURE FREE OF PROPHY MAY NOW BE POSSIBLE— WHAT ARE YOU WAITING FOR?

SCHEDULE ELIGIBILITY TESTING TODAY! TALK TO YOUR DOCTOR TO GET STARTED.

David
Factor IX level of 37% at 2 years
Patient portrayal; HEMGENIX not intended for women.

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CSL Behring

A ONE-TIME INFUSION OF HEMGENIX OFFERS ELEVATED FACTOR IX LEVELS FOR YEARS

37%

AVERAGE FACTOR IX ACTIVITY **ELEVATED AND SUSTAINED FOR YEARS***

GREATER BLEED PROTECTION VS. ROUTINE FACTOR IX PROPHY†

94%

OF PEOPLE DISCONTINUED FACTOR IX PROPHY **AND REMAINED PROPHY-FREE‡**

*Elevated factor IX levels have been observed annually.

†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.

‡Two patients were not able to stop routine prophylaxis. During months 7–18 an additional patient received prophylaxis during days 396–534 (approximately 20 weeks).

Learn about the next step on HEMGENIX.com

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.

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Please see Brief Summary of prescribing information on the following page, and the full prescribing information at HEMGENIX.com.

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

You 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.

www.CSLBehring.com www.HEMGENIX.com USA-HGX-0701-APR24

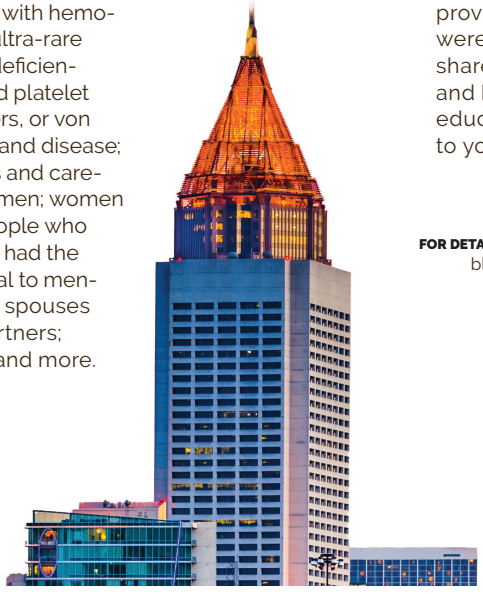
3 *Reasons to Attend BDC This Year*

The National Bleeding Disorders Foundation's (NBDF) 76th Annual Bleeding Disorders Conference (BDC) will be held Sept. 12-14 in Atlanta. It will provide three days of educational sessions and invaluable opportunities to connect with the inheritable blood and bleeding disorders community at workshops and networking events and in the grand exhibit hall. BDC 2024 offers more than 120 hours of programming and several track options for consumers, chapter leaders, and professionals.

Whether you're a veteran attendee or thinking of coming for the first time, here are three more reasons to register for this year's conference:

THERE'S A LITTLE SOMETHING FOR EVERYONE.
Consumer tracks are in English and en Español. Sessions feature topics for various patient, family, and support network audiences, with focus areas such as people with hemophilia, ultra-rare factor deficiencies and platelet disorders, or von Willebrand disease; parents and caregivers; men; women and people who have or had the potential to menstruate; spouses and partners; teens; and more.

DISCOVER ATLANTA.
The conference will be in downtown Atlanta at the Georgia World Congress Center, close to many of the historical sites, museums, and green spaces that Atlanta has to offer.



NEW THIS YEAR: CONSUMER PROGRAMMING.
For the first time, NBDF reached out to the community in its call for sessions and received 24 applications from patients, caregivers, and health care providers who were eager to share their stories and bring new educational topics to you.

FOR DETAILS AND TO REGISTER:
bleeding.org/events

SEAN PAVONE / GETTY IMAGES

INTRODUCING NBDF'S NEW PRESIDENT AND CEO, PHIL GATTONE

PHOTOGRAPHY BY NATALIA WEEDY

In February, the National Bleeding Disorders Foundation (NBDF) warmly welcomed Philip Gattone, M.Ed., as its new president and CEO. Gattone is a seasoned leader dedicated to amplifying the impact of nonprofit organizations through his distinctive blend of leadership and strategic insight. Prior to NBDF, Gattone was the president and CEO of the Epilepsy Foundation. He orchestrated unprecedented growth and expanded the organization's reach and influence, with annual revenues surging from $10 million in 2012 to over $25 million in 2020.

"My father was a great pianist and conductor, and when I think about NBDF and our community, I think of us in the same analogy as an orchestra," Gattone says. "Everyone has talents that they can offer, and when we come together playing from the same page, we can really make a beautiful sound. Our collaborative work together is not only powerful but a true testament to what an outstanding and passionate team we have here at NBDF.

"My hope and belief is that we can take the strengths of advocates, families, lived experience experts, medical professionals, and even friends who care deeply about this mission, and make a world-changing impact together for the inheritable blood and bleeding disorders community."

READ MORE ABOUT PHIL GATTONE:
bleeding.org/nbdf-new-ceo

Pfizer gene therapies are currently not FDA approved.

WHERE COULD HEMOPHILIA TREATMENT GO NEXT?

Learn more about what may be on the horizon and how shared decision making can help as you think about your treatment goals.

Gene therapy research continues to evolve. While more information becomes available, the below may help you better understand gene therapy and shared decision making within an evolving treatment landscape.

The Potential of Gene Therapy

Gene therapy aims to target the cause of a genetic disease. It can potentially help treat certain diseases by introducing a healthy (working or functioning) gene.

- In hemophilia A or B, the introduced gene is intended to inform the body to produce the factor VIII or factor IX protein involved in blood clotting
- Gene therapy could potentially provide a long-term treatment benefit to people living with a genetic disease with a single dose, as a one-time treatment option
- Gene therapy may help some people with hemophilia manage their disease without the need for frequent infusions or injections

It's important to note gene therapy may not be appropriate for all people with hemophilia.

Scan this code to learn more about the potential of gene therapy.

The Science of Gene Therapy

Gene Therapy Transfer or Addition is the transfer or addition of genetic material into the cell. A carrier shell delivers a healthy (working or functioning) gene, which is introduced into the cells of a person to treat a specific medical condition.

- When carriers contain the healthy gene, they are called vectors. Vectors are designed to deliver healthy genes directly into target organs, such as the liver
- In hemophilia, the healthy gene aims to provide the body with information to produce clotting factor protein VIII or IX
- People treated with gene therapy transfer or addition may still pass on genetic mutations and conditions to their children

Gene Regulation is the process of turning genes on or off.

- Gene regulation is accomplished by a variety of mechanisms by targeting RNA, the product of DNA transcription

Gene/Genome Editing: The goal of gene editing is to remove or correct pieces of DNA within a person's gene rather than replace the gene in the cell with a healthy gene, as gene transfer or addition would do.

- Gene editing can be used to correct, introduce, or remove pieces of DNA in different types of cells

Scan the code to learn more about the science of gene therapy.

Still have questions? Check out *FAQs: Gene Therapy*, available in this issue.

Shared Decision Making: *Discussing Gene Therapy With Your Care Team*

Shared decision making is a partnership between people with hemophilia and their health care providers. Choices about treatment options such as gene therapy may be made with the help of shared decision making in order to meet one's treatment goals.

See if you may be ready to talk about a change by reviewing *Quiz: Are You Ready to Discuss Different Treatment Options?* available in this issue.

Being prepared for a discussion with your health care provider is an important step of the shared decision making process.

If you'd like to discuss treatment options like gene therapy, you may want to bring a list of questions, such as:

- Is it a good fit for me?
- What are the risks and benefits?
- What is the process like?

Find more examples of questions to ask in *Discussing Treatment Options With Your Care Team*, available in this issue.

If you're considering a different hemophilia treatment like gene therapy, talk to your care team to find out what options may be a good fit for you.

Information available at HEMHORIZON.com may help you learn more. Scan the code to get started.

Pfizer gene therapies are currently not FDA approved.

PFIZER PATIENT AFFAIRS LIAISONS

SERVING THE HEMOPHILIA COMMUNITY

The role of a Pfizer Patient Affairs Liaison (PAL) is to support people living with hemophilia and their loved ones. Each PAL covers a specific geographic area. Across the United States, they help provide compassion, commitment, and connection to the hemophilia community. You do not need to be on a Pfizer product to connect with a PAL.

How PALs can help

- Guide people living with hemophilia and their caregivers towards resources that may help them
- Provide introductions and connections to advocacy organizations
- Offer information on Pfizer programs that may be available

Interested in learning more about gene therapy? *Connect with your PAL to gain access to information such as:*

- Answers to frequently asked questions
- A glossary of gene therapy terms
- A video on *The Science of Gene Therapy*
- A 1-page brochure with information on gene therapy
- A meeting to review *Gene Therapy: An Introductory Presentation For People With Hemophilia*

Together For Rare™ is the online home of Pfizer PALs. *Visit TogetherForRare.com to:*

- Opt in to connect with your PAL
- Schedule a call right away with our online scheduler tool
- Browse our hemophilia resource library
- Apply for Pfizer's Soozie Courter Hemophilia Scholarship. *Restrictions apply, visit TogetherForRare.com/scholarships for more information*

PALs **cannot** provide medical advice, share personal or contact information to promote Pfizer products, or proactively reach out to individual patients or caregivers.

Scan the code with your smartphone camera or visit TogetherForRare.com to get started.

WORLD VIEW Devin Smith's top international travel tip: Always be prepared.

COURTESY OF DEVIN SMITH

Travel Tips from a Pro

A globe-trotting college student shares advice for traveling with a bleeding disorder

Having a bleeding disorder shouldn't keep you from seeing the world. Just ask Devin Smith, a college student in Iowa with von Willebrand disease who completed a study abroad program.

"During my four months overseas, I was able to travel all around Ireland, as well as to Scotland, Germany, Czechia, Austria, Slovakia, and Italy," Smith says. "I've also traveled with my family to Bulgaria and Switzerland."

His No. 1 piece of advice is to be well prepared. Here are his top travel tips.

WORK WITH YOUR HTC NURSES

Smith recommends meeting with your hemophilia treatment center (HTC) nurses before you travel. They can help you confirm the appropriate medications and supplies to pack, review your current treatment plan to carry with you, and talk through where the nearest HTC might be at your travel destination, says Tami Bullock, RN-BC, BSN, a nurse coordinator at the Iowa Hemophilia and Thrombosis Center in Iowa City.

"An HTC nurse can also review your plan for receiving infusions if you need assistance and help you to know if you have emergency care insurance coverage while traveling," Bullock says.

REQUEST A TRAVEL LETTER

This document, signed by your health care provider, outlines your diagnosis and everything a medical team would need to know to treat you when you're away from home. It should include 24-hour contact information for your regular HTC should questions arise.

"The travel letter has been extremely helpful for when I go through airport security, too," Smith says. "I just ask them to read the letter, and it explains everything."

"The travel letter has been extremely helpful for when I go through airport security."

Bullock says the travel letter serves as authorization for security agents to allow medications and supplies, including liquids and needle products, to be in your carry-on luggage. "Have your prescription label on each box, vial, or bottle of medication," she says.

SAFELY TRANSPORT YOUR SUPPLIES

Check the storage requirements for your factor products and other medications. Many must be refrigerated, which means you'll need to transport them in a cooler with ice. "Use caution with hotel refrigerators, which sometimes have areas where items can freeze," Bullock says.

"Don't leave factor exposed to extreme temperatures, hot or cold," she adds. "Don't leave it in a hot car or on the beach."

If traveling by air, carry your cooler on the plane with you instead of checking it in your luggage to avoid the risk of it being lost in transit. If you'll be gone for more than a week or two, consider shipping your supplies to your destination.

HAVE A TRUSTED TRAVEL BUDDY

Wear an up-to-date medical ID tag throughout your travels. Also, when Smith goes with others, he makes sure that at least one person in his group is aware of his bleeding disorder.

"That way," he says, "they can help advocate for me if I'm unable to advocate for myself."

—By Shelley Flannery

KNOW BEFORE YOU GO: For more tips on traveling with a bleeding disorder, visit stepsforliving.hemophilia.org/step-up/travel

Talking to Teens and Young Adults About Safer Sex

Learn why and how you should have these tough conversations

TIME TO TALK Early childhood is the time to start having age-appropriate conversations.

With abortion access restricted in many states and sexually transmitted infections (STIs) on the rise nationwide, having a conversation with your teen or young adult about safer sex has never been more important.

The statistics are startling: The U.S. has one of the highest rates of teen pregnancy in the developed world. While HIV/AIDS was once the main STI concern for hemophilia treatment centers, today chlamydia, gonorrhea, and HPV are more common, says Diane Bartlett, LCSW, program manager and social worker at St. Luke's Hemophilia Center in Boise, Idaho.

"The two age groups with the highest risk for STIs are adolescents to young adults and those over age 65," Bartlett says. Half of all new cases of STIs occur in people ages 15 to 24, according to the Centers for Disease Control and Prevention.

Don't expect schools to cover sex education, says Joseph Stanco, DNP, FNP-BC, CPI, a family nurse practitioner with Northwell Health Hemostasis and Thrombosis Center in New Hyde Park, New York. Only 30 states and the District of Columbia require sex education in middle school or high school. Even then, the information your child receives may be limited. "It's really up to parents," Stanco says. "And studies over the last decades have shown parents have a positive influence on their child's sexual behavior."

"Children will start talking about sex around 9 to 10 years of age."

HERE'S WHEN AND HOW TO HAVE THESE NECESSARY CONVERSATIONS:

1. Talk early and often. Beginning in early childhood and continuing throughout your child's life, use age-appropriate and medically accurate language to discuss anatomy, consent, and safe touch. "Children will start talking about sex around 9 to 10 years of age, with their friends and on the playground, so parents want to have some of these conversations prior to that," Bartlett says.

2. Discuss consent. Talk to your child about what consent means and what that looks like in a healthy relationship, Bartlett says. Role-playing can help teens and young adults recognize unsafe situations and learn to speak up and set boundaries.

3. Use sexual content in TV shows, movies, and social media as a discussion springboard. "This way, it doesn't call out your child personally. It's a more neutral approach," Bartlett says.

4. Avoid shaming or blaming. Use neutral and nonjudgmental language. Don't label normal sexual health, expression, and behaviors as gross or "dirty," Bartlett says.

5. Identify another trusted adult. Teens and young adults may feel more comfortable talking about sex with someone other than their parents.

6. Tap into HTC resources. Sexual health education, including access to STI prevention tools, screening, and treatment, is part of the care that all HTCs provide for patients throughout their lives. HTC chapters often hold events focused on sexual health that parents and children can attend together.

—By Rita Colorito

Help for LGBTQ+ Kids

Hemophilia treatment centers are especially beneficial for children who fear coming out to their parents as LGBTQ+. "We always encourage children to speak to their parents and involve them in their care, but there are times when it may not be in their best interest," says HTC family nurse practitioner Joseph Stanco, DNP, FNP-BC, CPI. "We create a space so this is comfortable and confidential."

ILLUSTRATION BY MIGUEL DAVILLA

Navigating Labor, Delivery, and Postpartum

Creating a birth plan with your medical team is essential

Lindsay Callahan was pregnant with her second child when bloodwork showed that she had Factor V Leiden, a genetic clotting disorder. At nine weeks, she started hemorrhaging and was transferred to a high-risk practice. When she started losing amniotic fluid at 39 weeks, her labor was induced.

Although Callahan and her baby had a safe and healthy delivery, in retrospect she wishes she had asked more questions. "If there was a birth plan, I didn't know about it. Now that I am more educated, I would ask why I wasn't given treatment and monitored more frequently?" says Callahan, 41, of Stillwater, New York.

While Callahan has a clotting disorder and not a bleeding disorder, a study in *Haemophilia* in 2023 revealed that in more than half of the cases of delivery or postpartum hemorrhaging, a woman's bleeding disorder wasn't diagnosed prior to pregnancy.

When you have an inheritable blood or bleeding disorder, you have an elevated risk of bleeding complications during labor, delivery, and postpartum, says Jerome J. Federspiel, M.D., a maternal-fetal medicine physician at Duke University.

Federspiel and other experts recommend these four tips:

PREPARE Advanced planning can help reduce any risks for mom and baby.

SEVENTYFOUR IMAGES / ALAMY STOCK PHOTO

READ NBDF'S GUIDELINES FOR PREGNANCY AND PERINATAL MANAGEMENT:
bleeding.org/masac-pregnancy-guidelines
WATCH NBDF'S COLLABORATIVE LEARNING EXCHANGE TRAINING FOR CLINICIANS ON THIS TOPIC:
bleeding.org/clinical-challenges-wgppm

1 MAKE A PLAN WITH YOUR TEAM

Different bleeding disorders require different care plans tailored to your needs. "When a patient presents, even prematurely, the team on call already has a well-thought-out plan for your care. That kind of advance planning makes a huge difference," Federspiel says.

"I had very detailed birth plans in place before giving birth to both of my children, and had several meetings with adult and pediatric hematology teams," says Elaine Lai, 47, of San Francisco, who has mild factor VIII deficiency.

2 GO TO A TERTIARY CENTER

The safest care is often delivered in a place that combines expertise in both high-risk pregnancy and hematology. Your team should consist of an obstetrician, an anesthesiologist, adult and pediatric hematologists, a pharmacist, and a maternal-fetal medicine specialist. "I respect the relationship that the woman has developed with her regular obstetrician, but I want them to understand that the delivery and immediate postpartum care are very important and might be lifesaving for the mom as well as the infant," says hematologist Maissaa Janbain, M.D., director of the Louisiana Center for Bleeding and Clotting Disorders.

3 GET YOUR FACTOR LEVELS CHECKED

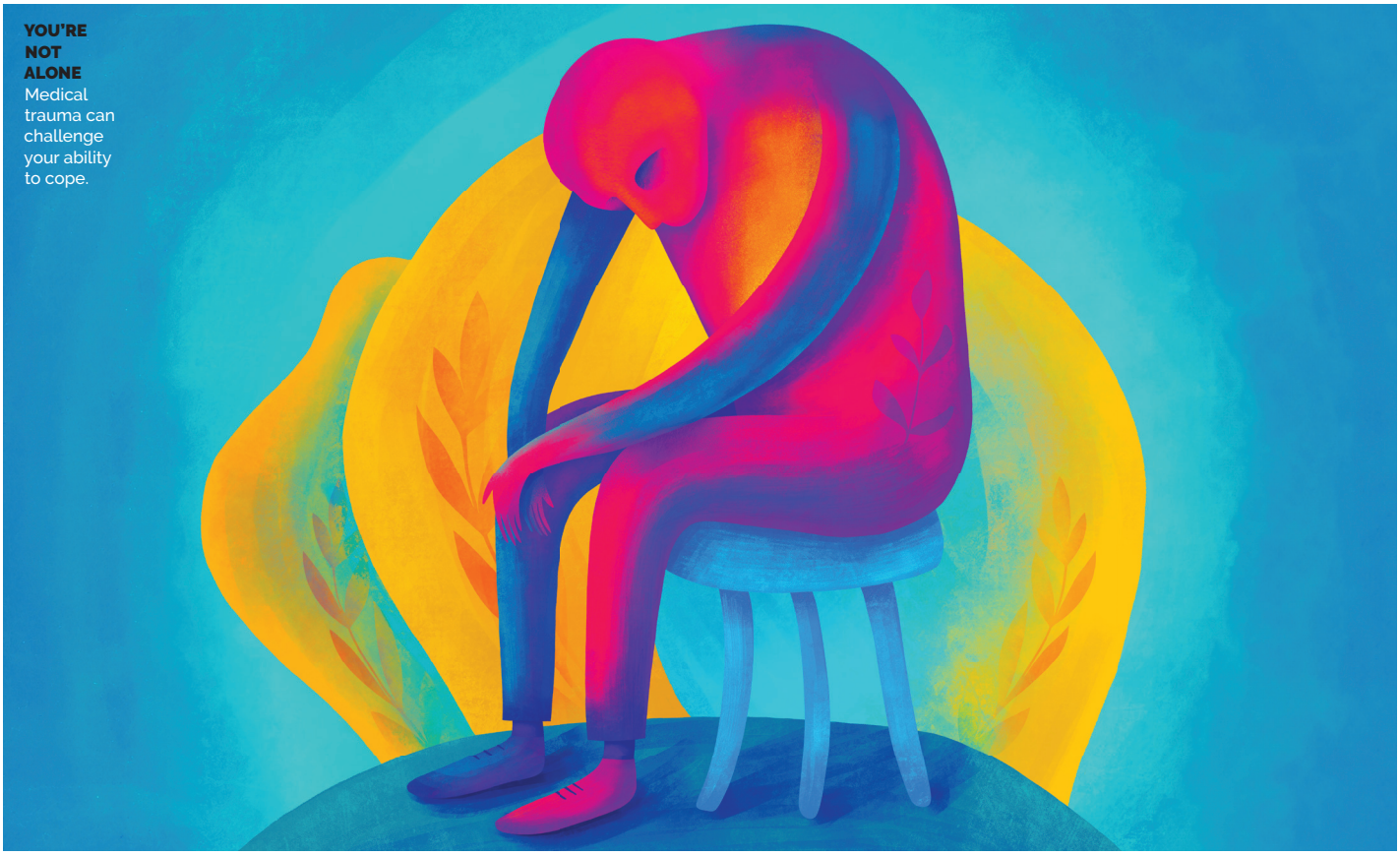
Knowing your baseline factor level will help the team know what type of replacement therapy you will need during labor, delivery, and (especially) in the postpartum period. That therapy in turn will make it more likely that you can safely be given an epidural.

Janbain says close monitoring of factor levels after delivery is important to prevent bleeding and other complications. "My birth plan specified that my factor levels would be checked at 24 and 36 hours after giving birth," Lai says.

4 EXPECT POSTPARTUM FOLLOW-UP

Women with bleeding disorders are more likely to experience postpartum bleeding problems than those without such conditions. Because of that, your team should follow you for at least a few weeks postpartum.

—By Beth Levine



YOU'RE NOT ALONE Medical trauma can challenge your ability to cope.

PTSD Isn't Just for Soldiers

This condition can also affect people battling a chronic disease. Here's how to identify the symptoms and get the help you need.

Justin Levesque had no physical symptoms of hemophilia, but he still felt unwell. "I was just getting nauseous all the time and feeling sick and tired whenever I went out in public, and I wasn't functioning socially," says Levesque, 37, an artist based in Portland, Maine. "I basically felt terrible."

He describes it as a "mental storm," but it goes by another name in medical literature: post-traumatic stress disorder, or PTSD, a condition that affects about 12% of adults with hemophilia, most of whom are men — and that is only now being fully understood.

In a study of 178 people with hemophilia, 101 reported having experienced a traumatic event related to their disease, according to study author Amanda Stahl, LICSW, a social worker at the Boston Hemophilia Center at Brigham and Women's Hospital. Of those who experienced trauma, 82% described it as chronic, meaning it was not a single incident.

"When you go through a medical trauma," Stahl says, whether it's a bleed, developing an inhibitor, or an unpleasant childhood memory of needles, "it can really challenge your ability to cope. It can impact your identity and inform how you view the world. Once you experience repeated traumatic stressors, your body can become programmed to react to ordinary stress the same way it would as if you were experiencing something more traumatic."

That's what happened to Levesque, who had an inhibitor that was resolved once he started taking a new medication for his hemophilia. The good news was that the drug ended his bleeding episodes and he spent less time on daily and weekly disease treatments. But it also gave him more time to think.

PUTTING OUT NEW FIRES

Without the daily fears of hemophilia, Levesque says, he was beset by memories and thoughts that he hadn't had time to contemplate before. "All of a sudden, I wasn't putting out fires every day or thinking about the problem right in front of me," he says. "And the rest of the train sort of catches up to the front here, and it's a crash, basically."

Stahl says that a diagnosis of PTSD includes exposure to a traumatic event, followed by the following symptoms:

- Intrusive memories (recurring, invasive thoughts of frightening medical scenarios, nightmares about a certain event)
- Avoidance (staying away from social settings, a health care provider's office, or even injections)
- Negative thinking or mood (feeling sad or in pain)
- Alterations in arousal or reactivity symptoms (hypervigilance, irritable or aggressive behavior, problems with concentration)

Stahl's study revealed that one-third of the participants could identify a traumatic event and went on to experience at least one of these symptoms.

Christi Humphrey, LCSW, a trauma-informed social worker at Hemophilia of Georgia, says people with hemophilia often have traumatic memories of childhood medical experiences that are "alive and well when the person has contact with the medical field. A strategy can develop where they escape the feelings by avoiding infusions or doctor's appointments, or avoid talking about what happened."

Levesque says he realized how traumatized he was by memories of being held down for painful injections as a child. Today, with the help of a trauma-informed therapist and medication, he feels better. "The difference is like night and day," he says.

HELP IS AVAILABLE

It's important for people who are having PTSD symptoms to understand that it's not unusual and that they're not alone, Humphrey says. "Your brain is designed to protect you, and it will go to great lengths to ensure your survival, including causing feelings designed to avoid fearful experiences," she says.

"Finding solutions starts with acknowledging the distressing thoughts, memories, and feelings. Seeking help from trained providers can start the process of change and open doors to new emotional experiences."

Some patients, like Levesque, find relief in talk therapy. Others try different types of therapy. If you're experiencing these symptoms, contact your hemophilia treatment center for a referral to a mental health professional. *—By Andrea Atkins*

EMDR Therapy May Be Beneficial

Many people with PTSD are finding relief with eye movement desensitization and reprocessing (EMDR), says Hemophilia of Georgia's Christi Humphrey, a licensed clinical social worker who is trained in the therapy.

EMDR is a technique in which a therapist guides a person through a series of standardized eye movements designed to stimulate brain activity. The goal is to help people identify ways to feel safe before exploring difficult memories, then learn to reprocess those memories so they don't cause symptoms.

"Every night in rapid eye movement (REM) sleep, the brain naturally processes situations and ideas from the day," Humphrey says. "EMDR mimics REM sleep to help with more traumatic memories and situations that are not as easily processed by the brain." The therapy "takes the traumatic memory and helps the brain process it in new ways so the memory can become more emotionally tolerable."

To be effective, the first step in the process requires developing a bond with a therapist, Humphrey says. While EMDR therapy does not work overnight, she says, it can be very effective at treating PTSD pain and trauma.

BRAIN REBOOT EMDR helps the brain process traumatic memories in new ways.

MARUSYA WROBEL / STOCKSY; CATHERINE MACBRIDE / STOCKSY

COVERAGE DENIED

UNEXPECTED CHANGE
Rodney Dalrymple was surprised to learn that the medication he had been using for years would no longer be covered.

ILLUSTRATION/ STAFF ARTIST

THE GROWING PROBLEM OF FORMULARY RESTRICTIONS

BY LISA FIELDS

For many years, Rodney Dalrymple, of Booneville, Mississippi, has managed his severe hemophilia A with a factor product that he infuses daily. In November 2023, he was shocked to hear that his health insurance company's pharmacy benefit manager (PBM) wouldn't cover his medication anymore. The only product that it would pay for, Dalrymple learned, turned out to be a product that he doesn't feel comfortable taking.

Dalrymple's hematologist appealed to the PBM on his behalf, and even had a peer-to-peer meeting to discuss the benefits of Dalrymple staying on his current medication, but the requests were denied. Dalrymple told the PBM that he'd be willing to switch to another factor VIII drug, but he hasn't been offered an alternative. As his factor supply dwindles, Dalrymple is uncertain about his prospects and growing anxious about his treatments.

"I've been skipping days to try to stretch and manage my factor, but I'm running out," he says. "My ankles have been bothering me, and I'm worried that I could start having more bleeds. Or in the situation of an accident, I wouldn't have any factor on hand. It puts me in a really bad situation. I don't know what I'm going to do."

Dalrymple's situation isn't isolated. In recent years, a growing number of PBMs — third-party companies that function as intermediaries between insurance providers and pharmaceutical manufacturers — have removed many medications from their lists of prescription drugs that they will cover, known as formularies. These formulary restrictions can limit patients' access to specific brands of medications — or in this case, particular brand-name clotting factors that people need to help them manage their bleeding disorders.

"It can be very disturbing to our practice, and to our relationship with the patient," says Maissaa Janbain, M.D., director of the Louisiana Center for Bleeding and Clotting Disorders and associate professor of hematology and oncology at Tulane University School of Medicine. "This is a problem that is going to come up more and more, now that there are so many products that are completely different."

> "THIS IS A PROBLEM THAT IS GOING TO COME UP MORE AND MORE, NOW THAT THERE ARE SO MANY PRODUCTS THAT ARE COMPLETELY DIFFERENT."
>
> MAISSAA JANBAIN, M.D.

A COST-CUTTING MEASURE

When insurance companies' PBMs adjust their formularies, it's often to remove very expensive medications and replace them with cheaper generic alternatives. Because the majority of biopharmaceutical products used to treat or manage bleeding disorders are quite costly and have no generic versions, some have been restricted from formularies.

"We understand the importance of managing costs and keeping the bottom line down, but that can't come at the expense of a patient's health, well-being, or their life," says Nathan Schaefer, senior vice president of public policy and access for the National Bleeding Disorders Foundation (NBDF). "They're limiting access to the medication that a physician and the patient have determined is most appropriate."

In 2023, CVS Caremark, one of the country's largest PBMs, removed all standard half-life products for people with hemophilia B from its 2024 formulary. Before that, BlueCross BlueShield of Tennessee removed at least 17 treatments for bleeding disorders from its 2023 formulary.

Formulary restrictions have been increasing for more than a decade, according to research published in March in the journal *Health Affairs*. In 2011, Medicare Part D plans excluded 20.4% of medications from their formularies. In 2020, the number of Medicare formulary exclusions rose to 30.4%. The study found significantly more restrictions for brand-name medications, which are pricier than generic drugs, but generics were also restricted.

PBMs don't often share details about the internal processes that lead to decisions on formulary restrictions.

"They tell you, 'We run it by our experts.' Who are your experts?" Janbain says.

WORRISOME DEVELOPMENT
Emily and Brian Romans with their sons (from left) Macklin and Kolson. When Kolson started crawling, he developed internal bleeds in his knees.

PHOTOGRAPHY BY COLIN CONCES

WHAT YOU CAN DO

Pharmacy benefit managers may alter their formularies at any time, and your medication could no longer be covered. If you can't get factor or other essential medications anymore, take these steps:

INVOLVE YOUR HEMATOLOGIST. Contact your hemophilia treatment center as soon as you learn about formulary restrictions, so that it can advocate for you.

"Patients always need their health care provider to back them up," says hematologist Maissaa Janbain, M.D. "Working as a team with the HTC and the health care provider is very important."

APPEAL THE DECISION. File a grievance with the PBM and make an appeal to remain on your medication. Share as much personal information as you're willing to reveal.

"Tell them who you are," says specialty nurse Kaitlin Rigsby, R.N. "Let them know that you're a real human with real needs."

SHARE YOUR STORY. If your appeal is denied, contact representatives in your state capital and Washington, D.C.

"Write down your story and send it to your legislators," Rigsby says. "The more that you feel empowered to share your story and how this impacts you, hopefully we can make small changes so that insurance companies and pharmacists are creating formularies that try to have more of a patient-centered communication about decisions."

GET EXPERTS' INPUT WHEN CHOOSING NEW INSURANCE. You may decide to change health insurance plans during open enrollment because of formulary restrictions, or you may need to select a plan when switching jobs. Either way, get input from your social worker, HTC administrative staff, or pharmacist.

"The more you can understand your insurance, which can be really complicated, the better," says social worker Kara Burge, LCSW. "Call your social worker. A lot of times, we do have resources, and we can help break things down."

You should also visit a PBM's website to see its comprehensive formulary list before signing on.

"Open enrollment is usually at the end of each fiscal year," Rigsby says. "A PBM may not release the next year's pharmacy formulary list until Jan. 1, but sometimes they release them early and you can verify that the insurance company or policy covers the medication you need. It's going to make it easier on everyone in the long run."

FOR MORE ON FORMULARY RESTRICTIONS: hemaware.org/formularyFAQs

FRUSTRATING PROCESS After multiple denials by their pharmacy benefit manager, Kolson's parents were finally able to get his medication approved.

"As individualized therapeutic approaches are encouraged, these decisions need to be left to the clinician who knows the patient well and not be imposed as a formulary by the company," she says.

After CVS Caremark removed standard half-life products from its formulary, Schaefer asked the company to explain its decision, but he didn't get specifics.

"They indicated that they have an expert panel that advises what gets onto the formularies and what does not," he says. "I asked them if they could share with me who those panelists are, and they declined to provide that information but indicated that there is hematology expertise represented on their panel."

POTENTIAL LONG-TERM REPERCUSSIONS

Hematologists and bleeding disorders advocacy groups worry that formulary restrictions could negatively affect patients' health. When someone responds well to a medication, they typically remain on it long term. But formulary restrictions may require people to switch drugs unless they can pay out of pocket, which isn't typically feasible.

"If a patient is stable on a particular factor medication, we usually recommend staying with that factor medication for as long as possible," says Kaitlin Rigsby, R.N., a specialty nurse at the Arkansas Center for Bleeding Disorders at Arkansas Children's Hospital in Little Rock. "When you switch factor medications, you can run the risk of developing an inhibitor."

While awaiting a PBM's approval for a nonformulary drug, patients may be nonadherent, causing gaps in their care. Others may not respond well to factor listed on a formulary. Both scenarios can be problematic.

NBDF's Schaefer says that while formulary restrictions may appear to save money in the short term, they can end up leading to much higher costs.

"A person could very well end up in the emergency room," he says. "If they've got permanent joint damage, they could have very expensive interventions that they require for years after the actual episode. We have to consider the patient's health and well-being for the rest of their life."

> **"A PERSON COULD VERY WELL END UP IN THE EMERGENCY ROOM."**
>
> NATHAN SCHAEFER, NBDF

APPEALING CAN BE COMPLICATED

If you want to continue on a medication that's removed from your PBM's formulary, you must pay out of pocket, unless you get an exception from the PBM. Some PBMs require prior authorization from prescribing physicians. Others require step therapy, in which patients first have to try formulary medications before the PBM will consider use of a nonformulary drug.

Janbain says a PBM's requirement that patients must fail on formulary drugs before they'll consider approving a nonformulary drug is particularly concerning.

"Any breakthrough bleed should be a failure," Janbain says. "Whether the payer is going to go with that or not, that's where I have some concerns. Some clinical trials of medications reported one or two bleeds, so what if a PBM will tell you, 'Oh, it's OK to have one or two bleeds'? That's why I'm worried."

In 2018, Ohio's Medicaid PBM stipulated that to qualify for drugs not listed on the formulary, patients would first need to fail on two formulary medications. "Bleeding disorders advocates in Ohio were able to push back, and the Medicaid office changed their policies so that you would only have to fail on one," Schaefer says. "That's still not ideal, but it's an indication of our success when it comes to pushing back on this."

Some manufacturers will cover patients' drug costs while they await exceptions from PBMs, says Kara Burge, LCSW, a social worker at the Arkansas Center for Bleeding Disorders.

"The drug companies make a lot of money off of these patients, so I'm glad they do swoop in to take care of them," Burge says. "On the other hand, the insurance companies know that, so there's no motivation or incentive for them to be more thoughtful about these things."

Schaefer hasn't heard of any successful CVS Caremark appeals since it restricted its 2024 formulary. But sometimes, PBMs do reconsider.

After Kolson Romans, of Gretna, Nebraska, started crawling at 5 months old in late 2023, he developed internal bleeds in his knees due to his severe hemophilia A.

Kolson's hematologist prescribed a medication for prophylaxis. The PBM denied coverage, possibly because Kolson was diagnosed with moderate hemophilia A as a newborn, before his diagnosis changed to severe. His doctor filed a prior authorization, appeals, and a peer-to-peer review, which were all denied. The family contacted the manufacturer for assistance and checked to see if Kolson qualified for Medicaid. "My husband and I were even talking about making a career change to get different insurance," says Emily Romans, Kolson's mom.

Three months after the initial denial, the PBM approved the medication. The news arrived just days after Romans posted about Kolson's situation on social media. (Romans tagged the PBM, and her post inspired a relative to speak with an influential friend at the PBM on Kolson's behalf.)

"We hope it's a life-changing medication for him, but it was a frustrating process," she says. "It feels like it was all about who our family knew and making a big deal about it online."

ADHERENCE *Still* MATTERS

BY CHRISTINA FRANK

Even as therapies for hemophilia have expanded and improved, the issue of not following treatment regimens as directed remains a major point of concern for patients and physicians.

MICHAEL AUSTIN/THE IVY POT

When Sean Pentz was growing up,

his mother made sure he received his infusions of clotting factor three times a week.

Although he learned how to self-infuse at the age of 9, it wasn't until he went off to college that he fully took on the responsibility for his treatment. And as a young adult, he recalls often having "dropped the ball" on his factor infusions.

"The main reason for my nonadherence was immaturity and irresponsibility," says Pentz, now 38, of Escondido, California. "I didn't set enough reminders to infuse, and then I'd just completely forget. Plus, my own shame and stubbornness toward seeking help made it tough to make the changes I needed to be more consistent with prophylaxis," he says. "As a result, I experienced a bad intramuscular bleed in my arm when I was 25 and multiple knee bleeds at 26 after a partial tear of my meniscus."

DANGERS OF NONADHERENCE

The problem of not adhering to your prescribed treatment plan — also called noncompliance or "treatment fatigue" — is certainly not exclusive to people with bleeding disorders. Across all chronic conditions, the treatment adherence rate is estimated to be only 50% on average. Yet generally, adherence rates of around 80% and up are what prescribers consider necessary to get the full benefits of a treatment therapy. In fact, according to the World Health Organization, adherence to one's therapy can have a more direct impact on patient outcomes than the specific treatment itself.

For people with hemophilia, staying on top of their treatments not only reduces the number of breakthrough bleeding events, but prevents added complications such as hemophilic arthropathy.

"All these tiny bleeding events can accumulate and lead to long-term damage," says Robert Sidonio Jr., M.D., M.Sc., director of hemostasis and thrombosis clinical operations at the Aflac Cancer and Blood Disorders Center of Children's Healthcare of Atlanta at Emory University. "We're trying to prevent things now so that 20 years down the road, patients will be in optimal health. We have great therapies in this country, and they work really well when you take them, but they don't work so well if you don't take them."

THE TRANSITION TO ADULTHOOD

Teenagers and young adults are especially vulnerable to being lax about sticking with their treatment plans. Studies have found that nonadherence rates among people with hemophilia are highest among 18- to 24-year-olds.

In addition to the heightened sense of not wanting to be deemed "different" that tends to characterize this age group, young adulthood is typically the time when individuals leave home for the first time, start to make their own decisions, and have to take on full responsibility for staying on top of their medical treatment.

"All of a sudden there's nobody seeing you every morning at the breakfast table reminding you to infuse," says Andrew Leavitt, M.D., director of the Adult Hemophilia Treatment Center at the University of California San Francisco.

Some hematologists are concerned that younger patients with hemophilia may not be as compliant with their medications because they've never experienced a bleed. "Maybe they've been on this treatment for 14 years and they haven't bled, and then they have a lot of other things to focus on in high school and feel invincible," says Courtney Thornburg, M.D., M.S., medical director of the Hemophilia and Thrombosis Treatment Center at Rady Children's Hospital-San Diego. "Adherence with the treatment can go by the wayside, and then there can be bleeding complications."

Leavitt says their pediatric centers work very hard at trying to make sure that kids take progressive and age-appropriate ownership and responsibility for their care. "The more you are in charge of your care, the better you will be and the happier you'll be," Leavitt adds.

TIPS FOR STAYING ACCOUNTABLE

Keeping on top of your treatment regimen can help prevent serious complications and improve your quality of life. These tips can make it easier to stay compliant.

Respect routines. "Setting routines in place as young as possible will help you be successful with your adherence to your prophylaxis," says Sean Pentz, who has hemophilia. "By taking command of giving yourself infusions, ordering your medication, communicating with your hematologist, and setting your schedule, you will definitely reduce the amount of pain and suffering from bleeds that are the result of nonadherence."

Set reminders. Use a written log, a calendar on the

refrigerator, or one of the many medication apps to remind you when it's treatment time. "It doesn't matter which one you use, just pick one," says hematologist Robert Sidonio Jr., M.D., M.Sc. "Use the one that you feel is useful. Some of the apps allow communication with staff at your treatment center, which is helpful as well."

Get support from community. Make it a priority to connect with others who share your condition, Sidonio says. "It's nice to get encouragement from the staff at your hemophilia treatment center, but I think peer encouragement is helpful, too. You have people who are going through the exact same thing and sometimes it's nice just to hear other people saying, 'I'm struggling with this too. I missed a dose last week and then I had a bleed,' or 'I'm just kind of tired of doing these infusions,'" he says.

"There are lots of virtual communities, lots of groups out there, and lots of opportunities to have get-togethers and go to family camps. These things help reinforce the reasons why you're doing this. You feel like you're not the only one."

Be honest. Above all, it's important to be truthful with your doctors if you have missed doses of medications. Clinicians understand that people miss doses or get off track. They're there to offer help, not to judge, but they can't offer suggestions when they do not know the reality of the situation.

"The worst thing that can happen is when a patient tells us that they're doing their factor prophylaxis when they aren't," Sidonio says. "We had a young child get hurt and develop an intracranial hemorrhage. We found out they were not doing the prophylaxis because they had issues with IV access. We could have worked with them and prevented that."

Pentz says to set aside any shame you may feel and reach out for help. "There is no reason to let shame hold you back from making a positive change for your care."

FIND A LIST OF TOOLS TO STAY COMPLIANT: Visit hemaware.org/compliancetools

DO BETTER TREATMENTS LEAD TO COMPLACENCY?

For many years, the only option for preventing bleeds in people with severe hemophilia was through a standard regimen of clotting factor replacement given intravenously as often as three times a week. Today, we're in what Sidonio calls the "golden era" of new therapies for hemophilia, including treatments that can be delivered subcutaneously and less often. For some individuals, gene therapy, a one-time treatment, may now be an option. However, even with all these advancements, bleeds are still possible.

Experts say that as treatments for bleeding disorders have expanded and improved, they are seeing a greater issue with nonadherence. For instance, people with severe hemophilia who are taking extended half-life products and now consider themselves "free" from intense treatment and management may become less attentive to their treatment.

"With less frequent as well as subcutaneous treatments you would think that adherence should be better, but there are still challenges with adhering to the new therapies," Thornburg says.

Even people who have had success with gene therapy may become overconfident and overdo activities that may jeopardize their joint health. While gene therapy may slow down or prevent hemophilic arthropathy progression, it cannot undo the damage that's already been done, Leavitt says.

"Patients who undergo gene therapy typically experience marked improvement in their joint symptoms, and we see that they then tax their joints well beyond what they did in the past. We need to be sure patients understand that underlying arthropathy is not expected to improve, so wise choices are still important for the best possible long-term joint health and function," he says.

For Sean Pentz, those bad bleeds in his late 20s forced him to come to terms with his nonadherence and become much more vigilant about his hemophilia treatment.

"With support from family and doctors, I learned from mistakes, developed better habits, and have minimized bleeds for the past 15 years," he says. "Now, I enjoy an active life with my daughters — hiking, biking, and playing sports, free from past limitations."

MAKING

DOBRILA VIGNJEVIC / GETTY IMAGES

HEALTHY START
Many parents discover their child has a bleeding disorder early in life. Steps for Living can be a valuable guide at this stage.

LEAPS

At every life stage, a blood or bleeding disorder can lead to new questions and concerns. The Steps for Living website from NBDF was designed to be the premier informational site for the bleeding disorders community, providing education across the life span. And now, for the first time since its launch in 2011, Steps for Living is getting a complete makeover.

WRITTEN BY STEPHANIE CONNER
ART BY DANIELA HRITCU

INFORMATION AT EVERY STEP

An inheritable blood or bleeding disorder can present new opportunities and challenges in every stage of life. The Steps for Living website was designed to help people with blood and bleeding disorders manage their health and live the best life possible.

WHAT ACTIVITIES ARE SAFE FOR ME?

GETTY IMAGES, TULCARION, MAXOIDOS

STEP	TARGET AGE RANGE	KEY TOPICS
First Step	Birth to 8 years old	Child care centers, babysitters, first years of life
Next Step	9 to 15 years old	School, social situations (including bullying and fitting in), camp, puberty, periods
Step Up	16 to 25 years old	Building independence, gap years, vocational training, colleges and universities, sexual and reproductive health, family planning
Step Out	Adults	Family planning, sexual and reproductive health, aging, work life, menopause, end-of-life planning

NEXT STEPS
As children get older, they progress toward independence. Steps for Living has informative content for them, too.

WHAT IS STEPS FOR LIVING?

Let's start with what's not changing — and that's the steps for living themselves. (See the chart at left.)

"Steps for Living starts when you have a newborn and you learn they have a bleeding disorder. How are you navigating life with a baby with a bleeding disorder? What are the things to look out for?" says Lena Volland, the director of education for the National Bleeding Disorders Foundation (NBDF). "Then, we provide information for the start of preschool on through elementary school, middle school, high school, university, vocational school, gap years, choosing a job, and becoming independent."

The site has long been a valuable tool for health care providers and NBDF's chapters.

"It's a wonderful resource for when a new family or individual is referred to our chapter," says Maureen Grace, MPA, the senior executive director of NBDF's Nebraska chapter. "We use it as a resource for families if they are being introduced to the bleeding disorders world and they're trying to figure out all the things that go with it. Since it walks you through the big issues at every stage in life, there's something for everyone to learn."

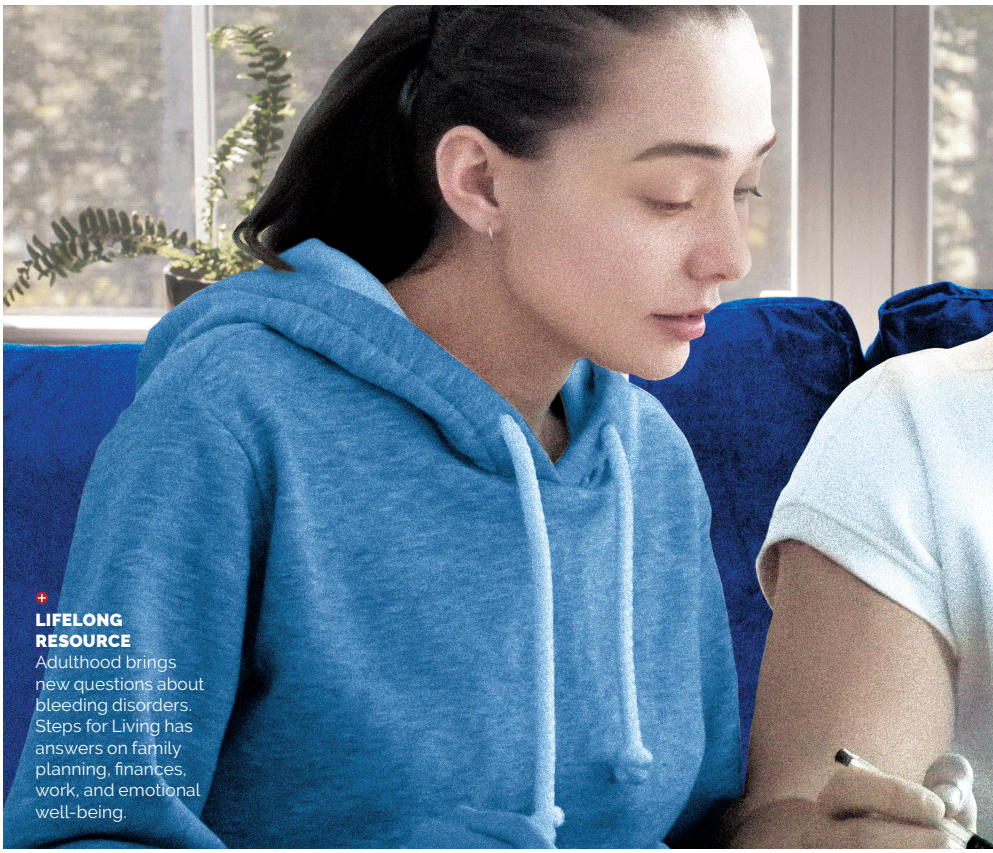
The Nebraska chapter links to Steps for Living in its newsletter, uses the content for educational sessions, and provides links to people as a resource.

Providers commonly use Steps for Living to guide their patients through milestones in life. It educates parents and helps young people become more independent as they age, says Cindy Bailey, PT, DPT, OCS, SCS, ATC, director of physical and occupational therapy for the Orthopaedic Hemophilia Treatment Center at the Luskin Orthopaedic Institute for Children in Los Angeles.

She and a colleague created a checklist for providers based on Steps for Living to walk through key milestones at patients' annual exams. "We focus on age-appropriate questions, like by age 3, have they been taught to use their knee pads on the playground? At age 7 or 8, are they seeing bruises?" she says. "We want to make sure we're asking certain questions. What have they been introduced to? How are they making progress toward independence?"

The overhauled Steps for Living promises to help people, providers, and NBDF chapters better understand and communicate

about blood and bleeding disorders. Here are seven reasons to get excited about the new website, launching this summer.



LIFELONG RESOURCE
Adulthood brings new questions about bleeding disorders. Steps for Living has answers on family planning, finances, work, and emotional well-being.

1 A MORE USER-FRIENDLY SITE

One primary objective of the Steps for Living relaunch is to make it easier for people to find what they need on the site.

"The Steps for Living site has been around for a long time, and it has grown throughout those years. More topics and a lot of new content have been added," Volland says. "As a result, the website became difficult to navigate. This redesign will make the site much more user-friendly so everybody can get all the information they need in as few clicks as possible."

And while the current site's content had various writers over the years, a concerted effort has been made to rewrite the content in a consistent voice and tone that's easy for readers to understand.

2 EXPANDED CONTENT

Last fall, the National Hemophilia Foundation became the National Bleeding Disorders Foundation in a move to be more inclusive of all inheritable blood and bleeding disorders.

"Steps for Living will not be hemophilia-centric. It covers a wide variety of bleeding disorders," Volland says. "We have information on von Willebrand disease, ultra-rare factor deficiencies, platelet dysfunctions, and more. And we will continue to add to that, because this is a living, always-expanding site."

3 UP-TO-DATE INFORMATION

A lot has changed in the world of blood and bleeding disorders over the past decade. With the new Steps for Living site has come a recommitment to up-to-date information.

"There are new treatments that are either on the horizon or that have been approved, such as gene therapy," Volland says. "We needed additional treatment information as well as additional resources for our community."

The NBDF team has been working through the more than 600 pages of content to make sure each piece features the latest resources.

"And it's not just our own resources but also other resources that might be helpful that we are linking to," Volland says. "It's also about more than treatment. It's about the overall quality of life and the information families need to live a healthy life with a bleeding disorder."

4 KID-FRIENDLY CONTENT

The new site will include expanded content specifically for children with blood and bleeding disorders.

"We cover topics that are important for school-age children. And the beautiful part for this particular age group is that it's not only written for parents but for the kids as well," Volland says. "At this age, a lot of children are starting to become more independent and are wanting to learn more, so all topics are also written through a kid-friendly lens."

Visitors can move a toggle switch to indicate whether they want to read an article from a parent's perspective or a child's perspective. The language has been updated to make everything understandable and appropriate for each audience.

5 SPANISH TRANSLATION FOR ALL MATERIALS

Through its market research, NBDF understands that many Steps for Living website visitors read the information in Spanish, so it was important to continue — and expand — its translated content.

"Not only will the articles for each step be translated, but all of the kid-focused content will be available in Spanish as well," Volland says. "We also made sure that any outside resources we are providing are offered in Spanish so that people can find the information they need in the language that is most suitable to them."

6 ADDITIONAL INFORMATION FOR ADULTS

Adults of all ages and experiences have different needs, and the new Steps for Living will help them understand a wide range of health and personal issues.

"We are adding topics on work, financial independence, and aging," Volland

FIZKES / GETTY IMAGES

JOIN US AT BDC IN SEPTEMBER

At the 2024 Bleeding Disorders Conference, the National Bleeding Disorders Foundation's education team will lead a session on the updated Steps for Living website, highlighting the different content areas and providing insights into how best to navigate and use the site.

EVENT DETAILS

What:
76th Annual Bleeding Disorders Conference

When:
Sept. 12–14, 2024

Where:
Atlanta

Learn more:
bleeding.org/bdc

says. "We talk more about menopause, living arrangements, and all the way to end-of-life planning. We really go through the age spectrum."

As a physical therapist, Bailey is particularly excited about the additional content for adults because there are a lot of considerations. "For example, if you have children, what will that mean for you physically? Do you need a certain kind of stroller?" she points out. "Then, there's your choice of occupation. Do you want to go to college or trade school? If you have a laborer's job, how long do you want to keep it? What accommodations do you need to think about to help save your joints as you get older? Those are the types of things we introduce to adults."

7

EXPANDED RESOURCES

The new Steps for Living website will have an updated and expanded resource section with useful tools.

"For example, we have a school toolkit as well as documents to use for individualized education program discussions with your school," Volland says. "People will also find a babysitter toolkit, financial planning tools, insurance support, checklists for subcutaneous injections and infusions, and more. We've really worked to integrate a lot of incredibly useful, fantastic tools to help people live their lives."

The team is also expanding the site's interactive material.

WATCH FOR THE NEW SITE

The new website, which has been in the works for several years, is expected to launch this summer.

"I'm so excited for the community to see the new Steps for Living. It's not just a quick, minor redesign or alignment with the brand. It's a reconceptualization of a very exciting program," Volland says. "I hope it's evident how much effort was put into this project to ensure that we're providing a resource that can truly help the members of our community live their healthiest lives."

BOOKMARK THIS SITE: Visit the new Steps for Living website at stepsforliving.hemophilia.org

NBDF UPDATE

Washington Days 2024 Recap

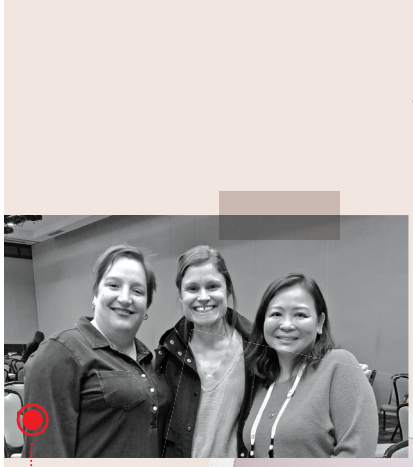
This annual event showcases the powerful impact passionate advocates can have

The National Bleeding Disorders Foundation's Washington Days 2024 event was a resounding success, bringing to our nation's capital more than 400 passionate volunteers from 45 states who are deeply invested in advocating for their family members, friends, and others affected by inheritable blood and bleeding disorders. This annual event serves as a crucial platform for advocates to voice their concerns and priorities directly to legislators and policymakers in Washington, D.C.

From March 6-8, community members engaged in more than 230 meaningful discussions and meetings with legislators, policymakers, and staff, focusing on key issues such as federal funding for bleeding disorders programs and policies aimed at making care more affordable and accessible. By sharing personal stories, data, and insights, advocates of every age highlighted the importance of prioritizing the needs of people living with bleeding disorders in the legislative agenda.

Washington Days 2024 was a powerful demonstration of the collective impact that passionate advocates can have in shaping policies and driving positive change for people and families living with bleeding disorders. Save the date for March 5-7, 2025. We hope to see you there!

FOR MORE PHOTOS OF NBDF EVENTS:
bleeding.org/events



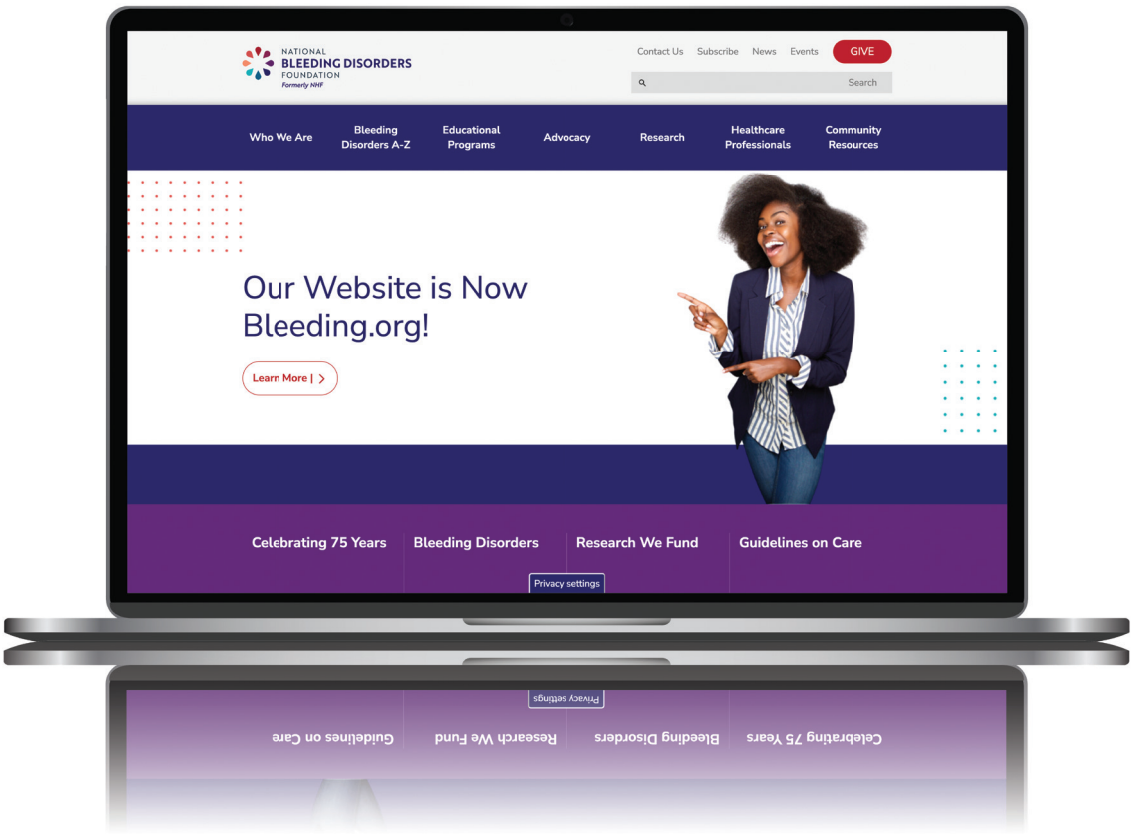
A FAMILY AFFAIR
Advocates of every age shared their stories.

COURTESY OF NBDF

STRONGER TOGETHER Several chapter groups met with legislators.

NBDF's Website Has a New Name: Bleeding.org

The transformative change will empower NBDF to connect with a broader audience in need of information and support



The National Bleeding Disorders Foundation changed its web domain from hemophilia.org to bleeding.org in April — a shift that perfectly encapsulates the organization's commitment to serving a diverse community of people affected by inheritable bleeding disorders. By embracing this change, NBDF hopes to enhance its online visibility and reach even more people seeking information about blood and bleeding disorders. Be sure to add the bleeding.org domain to your safe senders list to continue receiving emails and stay informed.

FOR DETAILS AND TO EXPLORE THE SITE: bleeding.org

Subscribe and Stay Connected

Get the latest news, research, and treatment updates about inheritable blood disorders, as well as education and services that improve the lives of people living with hemophilia, von Willebrand disease, and ultra-rare bleeding disorders. To stay connected, follow NBDF on social media **@nbd_foundation** or visit **bleeding.org/subscribe**.

WEDNESDAY WEBINARS

Join NBDF twice a month for a free, one-hour educational webinar series aimed at sharing knowledge about new therapies, the latest research developments and breakthroughs, and advocacy news for the bleeding disorders community. The webinars begin at 2 p.m. EST. Visit **bleeding.org/events** for dates and to sign up.

HELEN DATSKO / GETTY IMAGES

ATLANTA, GA

SEPTEMBER 12 - 14, 2024

We are excited to bring the bleeding disorders community together once again for three incredible days of educational sessions, valuable networking opportunities, and exciting exhibits at the Georgia World Congress Center in downtown Atlanta. For more information and to register visit our website.

BLEEDING.ORG/BDC

BLEEDING DISORDERS CONFERENCE
#BDC2024
Atlanta, GA • September 12–14, 2024

NATIONAL
BLEEDING DISORDERS
FOUNDATION
Formerly NHF

Making Space for Learning, Sharing, and Support

Each year, hundreds of women, girls, and people with the potential to menstruate (WGPPM) who are affected by bleeding disorders gather at retreats across the country. These events are invaluable opportunities for attendees to learn from experts and share information with one another in a supportive and fun environment. Here's a recap from three chapters that have held women's retreats this year.

IDAHO CHAPTER OF THE NATIONAL BLEEDING DISORDERS FOUNDATION

Set against the serene backdrop of Idaho's picturesque Sun Valley landscapes, our annual Victory for Women Retreat offers a much-needed sanctuary for women from all corners of the state. This year, we hosted ladies age 14 and up.

We offered insightful education tailored specifically for women with bleeding disorders and those who are caregivers. Through workshops and seminars led by female experts, attendees gained invaluable knowledge and resources, empowering them to be their best.

Beyond the educational aspects, the retreat is a haven for creating lasting friendships and unforgettable memories. The unique blend of relaxation, education, and socialization not only rejuvenates the spirit but also strengthens the bonds among women who share life with a bleeding disorder.

Learn more about the Idaho Chapter of the NBDF: idahoblood.org

WELCOME RESPITE
The retreat enabled participants to relax and truly step away from the hectic pace of everyday life.

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 **communications@bleeding.org**.

HEMOPHILIA FOUNDATION OF NORTHERN CALIFORNIA

The Female Factor Retreat hit double digits at its cozy 10th bash by the Santa Cruz seaside, where even the rain couldn't rain on our parade. Women and girls 12 and older came together for a weekend jam-packed with workshops and talks on bleeding disorders.

Between the waves crashing below and the seagulls putting on a show, we planted succulents, danced, played games, and toasted marshmallows by the bonfire. Some even got a crash course in infusions from the dynamic duo of Stanford Medicine's Kristin Yarborough, R.N., and UC Davis Health's Angela Mamangun, R.N.

For many, the retreat was more than just a getaway; it was a warm hug from the community during tough times. As the weekend wrapped up, faces glowed with joy, new friendships blossomed, and hearts felt full.

Learn more about the Hemophilia Foundation of Northern California: hemofoundation.org

VIRGINIA HEMOPHILIA FOUNDATION

The Virginia Hemophilia Foundation hosted its annual Women's Retreat in person for the first time in more than four years, bringing together 27 women affected by bleeding disorders.

Held at the Williamsburg Lodge, the weekend retreat offered a haven for the women to connect, learn, and find solace. The program featured a session on personal health literacy, a yoga break, a period kit-making project (with the kits donated to local hemophilia treatment centers), and a roundtable discussion with chapter board members.

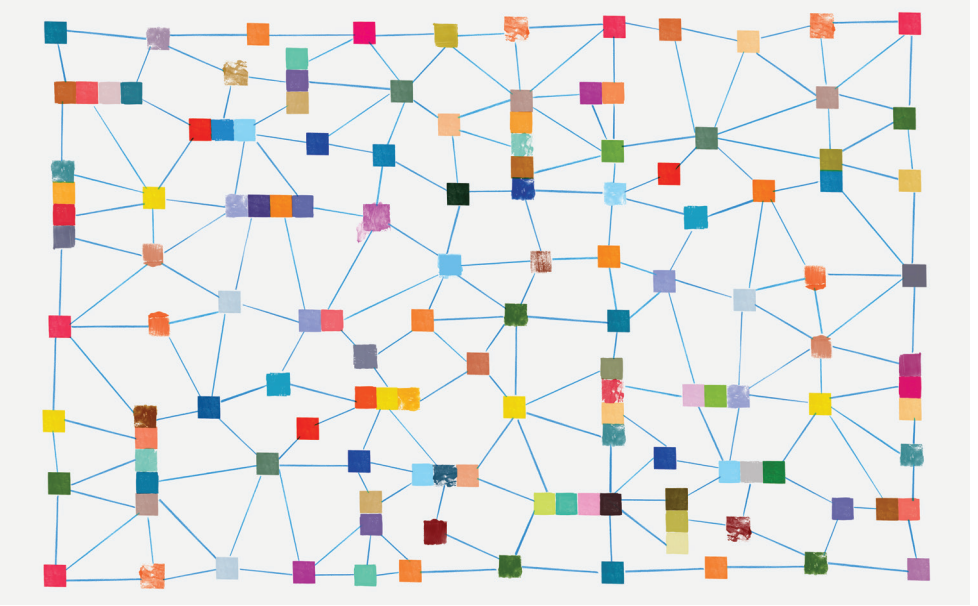
Participants ended the retreat with a unique exercise: Women wrote something they wanted to overcome on a board, then broke it in half to symbolize their ability to break through obstacles. This uplifting exercise left everyone feeling empowered and excited for future programming.

Learn more about the Virginia Hemophilia Foundation: vahemophilia.org

COURTESY OF THE NBDF CHAPTERS

WFH's Shared Decision-Making Tool

At a time when treatment options for people with hemophilia have expanded rapidly, it's an effective way for patients to ensure that they make the best choices for themselves



"Shared decision-making is a valuable approach incorporating patient preferences and goals to inform treatment decisions over time."

—AMY DUNN, M.D., DIRECTOR OF PEDIATRIC HEMATOLOGY AT NATIONWIDE CHILDREN'S HOSPITAL, COLUMBUS, OHIO

Shared decision-making (SDM) is a process in which a patient and their health care team work together to make decisions about medical care, based on the belief that patients have the right to be informed of all their relevant treatment choices and should be a part of their own treatment decisions.

In SDM for hemophilia, people with hemophilia and their health care teams work together to make decisions about the management and treatment of their condition. This includes careful consideration of their relevant life goals, aspirations, and preferences, along with a balanced consideration of the available evidence, risks, and benefits associated with each treatment option.

Studies have shown that SDM keeps patients engaged in their treatment, ensures that they are educated about their options, leads to higher longitudinal health care quality, improves treatment adherence, and increases patient satisfaction.

MANY CHOICES The tool allows patients to explore treatment options without bias.

There is growing interest in SDM in the scientific literature on hemophilia, supported by evidence indicating that well-designed SDM tools can help people compare treatment options and make more informed decisions. In response to signals from the scientific literature on the urgency for an SDM tool in hemophilia in an evolving and increasingly complex treatment landscape, and the absence of evidence that unequivocally supports the use of one treatment option over other reasonable options for all patients, the World Federation of Hemophilia (WFH) saw the need to develop an SDM tool for the community.

Partnering with people with hemophilia, patient advocacy groups, and health care practitioners, WFH developed the tool over two years and launched it in August 2023. The online platform guides patients and health care teams through the process of identifying patient preferences and priorities, provides balanced information on the risks and benefits of all treatment options, and helps to facilitate treatment-related discussions between patients and health care teams.

Since its launch, more than 3,500 people from 110 countries have used the tool. It is available in English, French, and Spanish, with translations in German, Dutch, and Japanese underway. The tool will be updated every six months to incorporate new data and evidence on treatments.

LEARN MORE: sdm.wfh.org

THE LAUNDRY ROOM / STOCKSY

CHRIS GASH / THEISPOT

Patient Preferences Evolve Along with Treatments

In a recent study, researchers looked at how people with hemophilia view treatment in light of less burdensome novel therapies

CHOICES
Patients and caregivers value an active lifestyle more than reduced bleeds.

In recent years, the arrival of novel hemophilia treatments that are effective and less burdensome to administer have opened up new possibilities for the consumers of these therapies. This begs the question: How might this evolving landscape be impacting treatment preferences for people with hemophilia?

Investigators sought answers to this question in a new study, "Patient and Caregiver Preferences for Haemophilia Treatments: A Discrete-Choice Experiment," which was published in the journal *Haemophilia*. The study was informed by a literature review and a survey open to adult males with

hemophilia age 18 and older, and caregivers of teen/adult males with hemophilia age 17 or younger.

The surveys, which were submitted online from February to April 2022, generated a sample of 151 affected adults and 151 caregiver respondents. Each respondent evaluated hypothetical hemophilia treatment profiles defined by six attributes via a discrete choice experiment (DCE). The DCE was used by the authors to quantify preferences and learn more about trade-offs individuals consider when making decisions about available treatments.

Respondents answered questions based on six attributes: number of annual spontaneous bleeds; ability to live a more active lifestyle; how a medicine is prepared/administered; frequency of administration; risk of an inhibitor; and risk of hospitalization due to treatment side effects.

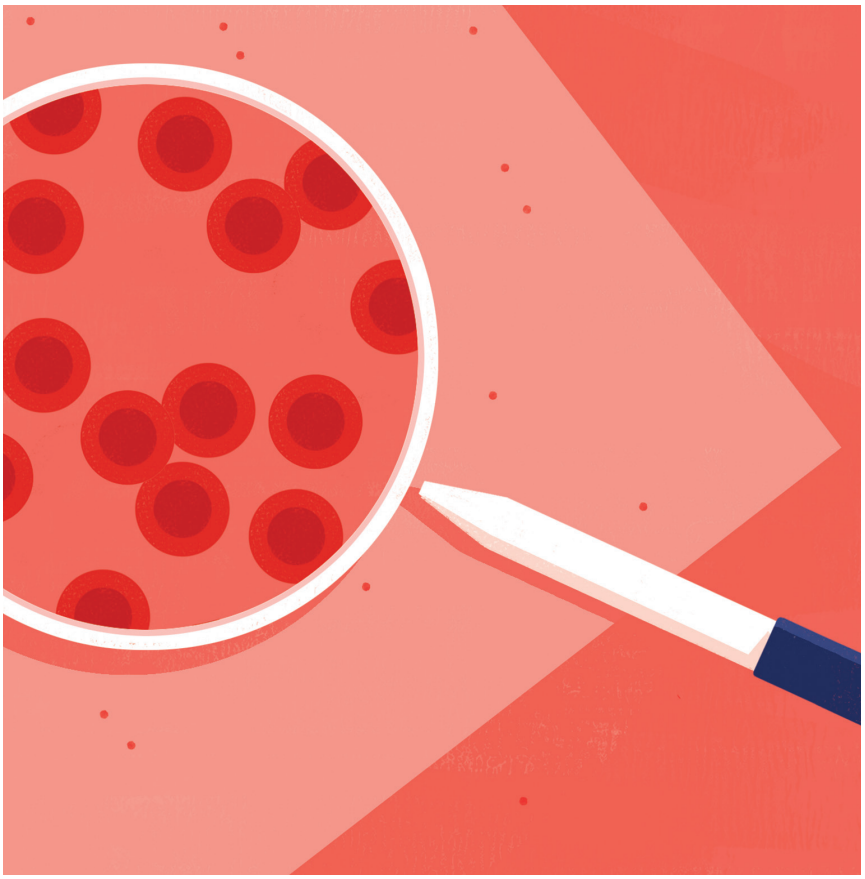
The burdens of treatment administration (intravenous and subcutaneous) and storage were also explored through a best-worst scaling (BWS) exercise, used to assess an individual's priorities. It captures extremes including best and worst items, most and least important factors, and biggest and smallest influences.

"In the BWS exercise, adult respondents and caregivers had overall similar preferences regarding the burden of treatment administration features," the investigators reported. "Both samples found frequent and longer IV infusions most burdensome and a sub-Q injection every two months least burdensome."

DCE results indicated that both adult patients and caregivers preferred treatments that enabled a more active lifestyle and are associated with a lower inhibitor risk. Notably, both groups valued an active life more than reducing spontaneous bleeds.

"These findings suggest that adults with haemophilia and caregivers of children with haemophilia are willing to make trade-offs for potential improvements in lifestyle not offered by clotting factor concentrates, bypass agents, or activated factor VIII mimetics," the authors explained.

TAKEAWAY
The findings underscore the importance of a shared decision-making approach.



The paper also acknowledged limitations. The sample generated from the survey included English speakers exclusively, and respondents were predominantly white and highly educated. In addition, the survey was only available online, which would exclude individuals/families who do not have internet access.

The authors posit that this study underscores the importance of a shared decision-making (SDM) approach to hemophilia care. Through SDM, health care providers and patients and caregivers may arrive at decisions that factor in the current treatment landscape and individual preferences.

LEARN MORE ABOUT BLEEDING DISORDERS RESEARCH: bleeding.org/research

Ad Index

Advocacy and Equity Are My Passion

Chancellor Donald, M.D., is committed to helping address and solve health disparities for people with hematologic disorders and malignancies

I've known that I wanted to be a physician since I was 5 years old. I fell and cut my leg, and when the doctor stitched me up, it was almost as if it was divinely appointed. From that moment on, I knew medicine was my calling.

During medical school, I vividly remember one hematologist's lecture about blood: something that transports oxygen and nutrients throughout our bodies. Her powerful description captivated me, and from then on, again, I knew. I directed my energy toward hematology as well as oncology, which go hand in hand.

Today, I am an assistant professor of clinical medicine at Tulane University, and I'm also the medical director of cancer management and oncology services at the University Medical Center New Orleans.

I spent nearly 10 years in private practice and saw physician burnout firsthand, and COVID served to accelerate the problem. From a provider perspective, I believe that setting boundaries can help. There's been a cultural shift in terms of what's expected from a physician. When I started out, there was more built-in separation of work and personal space.

COURAGE It's a virtue Donald says he works on every day.

Now, those guardrails no longer exist. Someone can always be "present," whether by email, Zoom, cellphone, etc. So, it's even more difficult for physicians to establish boundaries, and it's more important than ever that they do.

AN ASYMMETRICAL SYSTEM

These days, I'm in academic practice, with a particular focus on advocacy and health disparities for patients with hematologic disorders and malignancies. Addressing these disparities is complicated, but my instinct is to always follow the scientific method — the first step of which is to identify the problem.

We need to take an honest, broad, comprehensive look to figure out everything that affects a person's ability to receive proper health care. Does that person have access to all stages of their care? Is there a specialist they can visit to manage their therapy? What are that person's living conditions? What are the things that might interfere with them receiving the most appropriate, up-to-date care?

In addition, we need people to speak out against inequities. Certain individuals benefit from a system staying as it is, and it takes courage to be in those spaces and to speak to fairness and justice.

Courage is something I work on every single day. My aim is to never be complicit with an arrangement that I know is damaging. I very much relate to the Maya Angelou quote "I am convinced that courage is the most important of all the virtues. Because without courage, you can't practice any other virtue consistently."

I've been honored to have received several awards throughout my career. And while I've been humbled at this recognition, my greatest reward is when I hear that a patient or their family has felt seen and cared for. This is what keeps me going and keeps me grateful.

—By Chancellor Donald, M.D., as told to Leslie Pepper

FOR MORE INFORMATION ABOUT THE NBDF'S WORK, VISIT: bleeding.org

COURTESY OF CHANCELLOR DONALD, M.D.

Patient Information **Rx Only**

ALTUVIIIO™ (al too'vee oh)
[antihemophilic factor (recombinant), Fc-VWF-XTEN fusion protein-ehtl] for intravenous use after reconstitution only

Single-dose vial

Please read this Patient Information carefully before using ALTUVIIIO 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 ALTUVIIIO?

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 ALTUVIIIO so that your treatment will work best for you.

What is ALTUVIIIO?

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

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

Who should not use ALTUVIIIO?

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

What should I tell my healthcare provider before using ALTUVIIIO?

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 ALTUVIIIO may harm your unborn baby.
- Breastfeeding. It is not known if ALTUVIIIO passes into the milk and if it can harm your baby.

How should I use ALTUVIIIO?

You get ALTUVIIIO 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 ALTUVIIIO.

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

What are the possible side effects of ALTUVIIIO?

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

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

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

What are the ALTUVIIIO dosage strengths?

ALTUVIIIO 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 ALTUVIIIO?

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

What else should I know about ALTUVIIIO?

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ALTUVIIIO for a condition for which it was not prescribed. Do not share ALTUVIIIO 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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Derek and Brooklynn, father and daughter | Real ALTUVIIIO® patients

ALTUVIIIO®
Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl

SWITCHING IT UP

made a difference for Derek and Brooklynn

Derek and Brooklynn are promotional speakers compensated by Sanofi and received free product through Sanofi's Patient Support Program.

"When the doctor mentioned once-weekly infusions, my ears perked up. I began taking ALTUVIIIO, and now, I infuse once a week. I appreciate being able to go longer between my infusions." Derek

Derek and Brooklynn aren't the only ALTUVIIIO Peers who made the switch.

Visit ALTUVIIIO.com to hear more from Derek and Brooklynn—and other patients like you.

"Since starting ALTUVIIIO, I have not had any breakthrough bleeds, and I have higher sustained Factor VIII levels."* Brooklynn

*ALTUVIIIO was studied in 159 adults and adolescents (12 years and older) with severe hemophilia. Of these, 133 people switched to ALTUVIIIO prophylaxis from prior prophylaxis therapy. The other 26 switched from prior on-demand therapy to ALTUVIIIO on demand for 26 weeks, and then to ALTUVIIIO prophylaxis for another 26 weeks. Routine prophylaxis with ALTUVIIIO resulted in a mean ABR of 0.7 and a median ABR of 0 based on treated bleeds. Average FVIII trough levels were 18% for adults and 9% for adolescents aged 12 years to under 18 years.

INDICATION

ALTUVIIIO® [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 ALTUVIIIO when you have surgery.

IMPORTANT SAFETY INFORMATION

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

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 ALTUVIIIO so that your treatment will work best for you.

Who should not use ALTUVIIIO?

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

What should I tell my healthcare provider before using ALTUVIIIO?

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 ALTUVIIIO?

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

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

These are not the only possible side effects of ALTUVIIIO. 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, or call 1-800-FDA-1088.

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

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