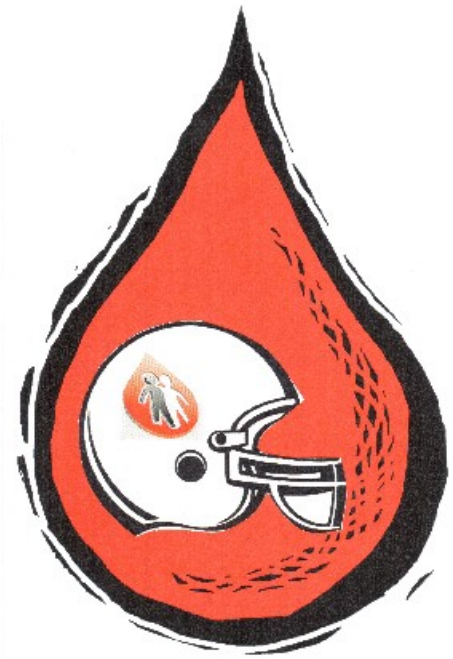

BIG RED FACTOR

2019—Issue IV

Inside this issue:

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION
www.nebraskanhf.org

Nebraska Chapter News

Misti and I want to thank you from the bottom of our hearts for a great 2019. We loved being able to provide you with education, networking, fundraising fun and so much more this year. We adore the bleeding disorder family here in Nebraska and you are what makes this community so great. Thank you to everyone who attended events, advocated with us, attended and fundraised at Walk and helped us to reach our community all across the State. Thank you to our sponsors, our donors and our Board for everything they do to make our Chapter and community stronger.

We are excited to go into our 35th Anniversary year in 2020, celebrating who we are, where we've come from and looking forward to new growth and opportunities.

May your holidays be safe and happy. See you in 2020!

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION
www.nebraskanhf.org

Our Mission:

The National Hemophilia Foundation—Nebraska Chapter is dedicated to finding better treatments and cures for inheritable bleeding disorders and to preventing the complications of these disorders through education, advocacy & research.

Staff

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Development Manager
Misti Mitchell

Advisory Board of Directors

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The material in this newsletter is provided for your general information only. The Nebraska Chapter does not give medical advice or engage in the practice of medicine. NHF-NE does not recommend particular treatments for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.

2020
First Quarter Events

January

Happy New Year!

January 9

Sanofi Genzyme Dinner– Omaha

February

February 1, 2020

Advocacy Workshop

February 26-28, 2020

Washington Days

March: Hemophilia Awareness Month

March 20-22, 2020

FAB (Females and Bleeding) Conference

March 24-25, 2020

Nebraska Advocacy Days

2020 Save the Dates

May 16-17th

Family Education Weekend

June 5 -7th (NEW DATE!!)

Family Camp

combined health agencies drive
MEMBER CHARITY

Nebraska Chapter
National Hemophilia Foundation

2020 Calendar of Events

2020 Dates are Subject to Change- Please check our website for current programs and dates!

January - March

January 9- Omaha Education Dinner
January 31st- Pfizer Education Dinner
February 1- Advocacy Workshop
February 26-28- Washington Days
March 20-22- FAB Women's Conference
March 24- Omaha Advocacy Dinner
March 25- Lincoln Advocacy Day

Teen Program Event- TBD

April- June

April 11- VWD Education Day
April 25- Spring PING
May 16-17- Family Education Weekend
June 5-7 Family Camp

Spring Teen Program- FEW

July- September

July 19- Infusion Bloody Mary Mix Off
August 6-8- NHF's Bleeding Disorder Conference- Atlanta, GA
August 29- Kearney Education Day

Teen Program Event- Early September

October- December

October 3- Unite for Bleeding Disorders Walk
October 17- Harvest Festival
November 14- Industry Symposium
December 5- Winter PING!

Teen Year End Retreat- TBD

SAVE THE DATE

FAB (Females and Bleeding)

Women's Conference

Sponsored by:

octapharma

March 20-22, 2020

Honey Creek Resort

Moravia, IA

Harvest Fest 2019

Nebraska is beautiful in the Fall and we were so happy to host our Harvest Fest in October at the Roca Berry Farm again this year. It was absolutely wonderful weather to spend out at the pumpkin patch with those in our community. We understand how important it is to get together for fun and networking without an education piece. Thank you to everyone who came out, to our sponsors who make this event possible and for celebrating the season with us with delicious donuts, juice and family.

We love spending time with you and making this opportunities possible.

Thank you to our 2019 sponsors who made this event possible!

novo nordisk

SUPERIOR BIOLOGICS

FACTOR ONE SOURCE FAST PHARMACY

Could a Cure for Hemophilia Be Personalized?

Laurie Kelley

Has your hematologist ever asked that your child have pharmacokinetic (PK) testing? Chances are, the doctor wants to know how factor behaves in your child's body, so factor dosing can be tailored to get the maximum factor coverage on your child's most active days. *Personalized medicine* is the tailoring of medical treatment to your individual biological characteristics, and to your lifestyle, for the best therapeutic results. Personalized medicine in hemophilia often refers not only to when you dose with factor concentrate, but also to PK testing to discover your child's half-life—how long factor lasts in his body—so you can choose the right dose, dosing schedule, and product.

In this issue of PEN, we focus on gene therapy, which may be available commercially in the next few years. Is it possible that gene therapy—or any form of a cure—could be personalized to meet the needs of individual patients? To find out, we asked the experts at three biotech companies involved in gene therapy research: uniQure, Spark Therapeutics, and Sigilon Therapeutics.*

What About Gene Therapy Could Be Personalized?

Personalization for a cure in hemophilia starts with the hemophilia diagnosis. Dr. Rogerio Vivaldi, CEO of Sigilon, says, "Gene therapy is, by its nature, personalized to a patient's genetic profile. It seeks to deliver healthy copies of the specific gene that is dysfunctional in a given patient. The healthy genes are paired with a promoter that prompts them to express the specific enzyme, protein, or factor that the patient is missing. Restoring the balance in expression of that enzyme, protein, or factor should reduce or even eliminate symptoms and improve the patient's health." He adds, "However, it's important to note that these therapies aren't customized to individual patients. They are designed to cover all patients with a specific genetic mutation—for example, all patients with low factor VIII levels."

Clearly, the diagnosis matters, notes Dr. Leonard Valentino, Medical Strategy Lead at Spark Therapeutics. "We don't have a universal approach to apply to the diagnosis of hemophilia in general. There are differences in trials for hemophilia A and B." So your diagnosis will determine how your gene therapy might work.

Another influence is factor levels. Danielle Day, Medical Science Liaisons, GlobalMedical Affairs at uniQure, notes, "With gene therapy, we've identified the missing or altered gene, and can deliver a functional copy of it…but across all gene therapy studies, there appears to be variability in how much factor is produced."

Challenges to Personalization

A major challenge to personalizing gene therapy is the "neutralizing antibody profile." That is, does the patient have an *antibody* (inhibitor) to the vector (the virus being used to deliver the gene therapy into the liver)? According to Vivaldi, more than 40% of patients who might benefit from gene therapy have preexisting antibodies to the vector. This makes them ineligible for treatment. Also, patients who have liver disease, as well as pediatric patients (who have rapidly growing livers), are ineligible for gene therapy.

"Right now investigational gene therapies are 'one and done,'" notes Valentino. "Currently you can't redose gene therapy after the initial dose, due to the neutralizing inhibitor. Maybe down the road, if you used an AAV8 vector and it didn't work, you could go back and try an AAV5 vector, but this is not really a reality right now in clinical trials. Currently, you can't personalize based on different vectors. You can only personalize based on your personal antibody profile. So we still need to resolve the redosing issue."

Another puzzle to solve is why some patients in clinical trials show higher factor level expression than others. Could this become an area of personalization someday? Could patients pick their gene therapy based on how much factor expression they want? "When using wild type factor IX," Day reports, "levels were increased but not to the near-normal seen with the hyperactive Padua factor IX gene. We were just trying at that time to get levels above 1%. And then some were getting over 10%. That variability is important. In the current factor IX trials, using the hyperactive Padua factor IX gene, you see a range of 14%–80% across trials. Now you wonder, does gene therapy even need to be personalized if patients are no longer bleeding, without having to be infused?"

Is Cell Therapy a Personalized Cure?

Vivaldi points out that Sigilon is developing a different approach to a genetic cure, called cell therapy: "It's another form of personalized medicine."

He explains, "First, we've engineered human cells to produce the specific enzyme, protein, or factor that the patient is missing, like a living protein factory. Separately, we've engineered a biomaterial that is meant to protect these cells. Normally when you implant any foreign tissue into a patient, the patient's immune system will identify it as a threat and attack it. Our biomaterial are tiny spheres capable of shielding our therapeutic cells from immune attack. The spheres are also designed to ward off fibrosis, which is a scarring process that normally occurs as part of the body's reaction to a foreign implant."

In this therapeutic cure, thousands of the engineered cells are nestled into the special spheres. Nutrients and oxygen flow through the sphere's matrix-like walls and nourish the cells. The proteins, enzymes, or factors that the engineered cells produce are able to flow out of the spheres and circulate through the patient's bloodstream. The spheres are implanted into the patient's abdomen in a simple laparoscopic procedure.

"We believe this will vastly improve [patients'] symptoms, delivering a functional cure for hemophilia," says Vivaldi.

This functional cure can be personalized. "A key advantage to our approach is that you can redose the patient by adding more cells loaded into spheres, if he or she needs more therapy," Vivaldi notes. The spheres also have the potential to be removed, if needed. In contrast to gene therapy, there's no issue with preexisting antibodies. "And because our cells don't integrate into the patient's DNA," he adds, "there's no concern about off-target integration causing side effects."

Future of a Personalized Cure

"We don't know what we want from gene therapy yet," says Valentino. "Do we want a cure? Freedom from spontaneous bleeding? Freedom from infusions, or maybe normal factor levels? The community needs to put a stake in the ground for what they are looking for in potential gene therapies. We've heard that it will correct genetic defects, but what will that mean practically?"

Whether gene or cell therapy, Vivaldi notes, "Both should be far more durable than today's standard of care [infusions of factor concentrate]. A single treatment should last years. Another important advantage is that both therapies should result in a steady production of the protein, enzyme, or factor the patient is missing. There should not be spikes or plateaus, unless they are deliberately designed to be part of the therapy."

Day adds, "Some patients want a guarantee; they never want to infuse again. Everyone has different expectations. What do parents, spouses, and partners think about gene therapy? It's a big decision to make, a family decision—and that's a big part of personalization, choosing what's right for the patient and their family."

Vivaldi sums it up: "This is a very exciting time where we're seeing the future of medicine unfold before us. There's still a lot of work to be done, but there are a lot of terrific ideas and promising technologies."

Teen Program & Retreat 2019 and Beyond

We have needed a teen program for a long time and now it's finally a reality! On Saturday, October 26, 2019 NE-NHF held our inaugural teen event where 6 affected teens and unaffected siblings gathered for a day of outdoor activities and team building. Executive Director, Maureen Grace, led an interactive dialogue, gathering suggestions for 2020 teen programming and strengthening the relationship for this demographic at the chapter level. The teens in attendance stepped up to our challenge to own the program, lead the content and be active and involved in the chapter year-round.

We are planning great things for 2020 with a lot of the programming being led by the teens and what they want to learn and how they want to be engaged with the chapter. We will have a teen get together in the next couple months so please stay tuned for the date for a fun bowling and education evening. The teens will also have their own track at Family Education Weekend that they planned themselves. We want this to be a safe and fun space for all teens in our community whether they are affected, an unaffected sibling or a the child of someone with a bleeding disorder. The goal for 2020 is to have one event per quarter, to have established teen leadership and involvement and a meaningful programs that meets teens where they are at and helps them grow into capable adults and leaders in our community. We are SO excited to watch this program grow over the next year and for our teens to truly own the process and their program. Here's to a great year with our teens YEAR ROUND!

2019 Teen Retreat
Mahoney State Park

Special Considerations for Girls

The Onset of Menstruation May Be a Time to Focus on a Hemophilia Care Plan

For more information, visit b2byourvoice.com to download *Hemophilia B: Her Voice, Her Life.*

This content is brought to you by Pfizer.

Puberty on its own can be a difficult experience, but it can become even more complicated with a hemophilia diagnosis. The start of menstruation is one of the many aspects of a girl's life that may be affected by a bleeding disorder. For girls who are showing symptoms of hemophilia, puberty may be a good time to identify a health care team and develop a care plan.

Identifying a Health Care Team

The lack of knowledge about how hemophilia impacts girls can affect the level of medical care and emotional support received by a girl with this condition.[1] It may be recommended that a girl who is diagnosed with hemophilia wear a medical identifier at all times so that medical personnel are aware of her bleeding disorder in an emergency. It is important for every female with hemophilia to enlist a team that includes a primary care physician, a gynecologist, and a hematologist who can coordinate care and needs.[2]

Tools that can help girls manage symptoms include[3]:

- Care plans designed for patients by their team of health care providers to help facilitate care coordination
- Apps that allow patients to track their hemophilia symptoms and care
- Self-monitoring assistance for better symptom accuracy

Heavy Menstrual Bleeding

Periods with heavy blood loss (called *menorrhagia*) can lead to anemia and have a negative effect on quality of life. Girls with bleeding disorders who are experiencing symptoms of menorrhagia should have a discussion with their health care team in order to coordinate management and care.[4]

The signs and symptoms of menorrhagia include[4]:

- Having a menstrual period that lasts longer than 7 days
- Needing to change pads or tampons at least every 2 hours
- Passing blood clots larger than a quarter
- Bleeding that affects daily activities

> "I am a true testament to the fact that factor replacement therapies can help when it comes to being a woman with hemophilia. Not only do we have to deal with the joint bleeds that men do, but we also have specific issues as women."
>
> - ELIZABETH
> Has hemophilia B

Tips for Parents[4]

Parents of a girl with a bleeding disorder can ease their daughter's transition into puberty by preparing her for the experience of having periods and helping her learn how to manage them. It can also be helpful for parents to ensure that a supply of feminine products is available and provide a way for their daughter to carry the products discreetly if needed. Parents can also help by providing honest, accurate information about menstruation and the impact hemophilia may have.

The beginning of menstruation, which can already be a confusing and demanding time in the life of any girl, brings special concerns for those showing symptoms of hemophilia. It's important for girls to talk with health care providers and caregivers about their periods, especially if bleeding becomes heavy.

References: 1. Aldridge S. The carrier barrier: women push for mild hemophilia diagnosis. HemAware.org Web site. www.hemaware.org/story/carrier-barrier. Published July 19, 2012. Accessed February 20, 2019. **2.** Canadian Hemophilia Society (CHS). Precautions for pregnant women with a bleeding disorder. Hemophilia.ca Web site. www.hemophilia.ca/en/women/precautions-for-pregnant-women/. Accessed February 20, 2019. **3.** Carr S. A new look at patient communications in outcomes-driven healthcare. Pharmaphorum.com Web site. http://pharmaphorum.com/views-and-analysis/a_new_look_at_patient_communications_in_outcomes-driven_healthcare/. Published November 5, 2012. Accessed February 20, 2019. **4.** National Hemophilia Foundation (NHF). Effects of puberty on girls with a bleeding disorder. StepsforLiving.Hemophilia.org Web site. https://stepsforliving.hemophilia.org/next-step/maintaining-a-healthy-body/growing-up-puberty/effects-of-puberty-on-girls-with-a-bleeding-disorder. Accessed February 20, 2019.

PP-HEM-USA-1168 April 2019

Hemophilia: A Woman's Journey to Effective Treatment

Milora Morley, MPH

I never thought at age 27 that I would begin a journey as a woman diagnosed with mild hemophilia A. My brother, as well as several men in my family, have all been diagnosed with severe hemophilia A. I'm no stranger to this disorder.

I grew up in the south Florida hemophilia community. As my brother's keeper, and older sister, I had a lot of responsibility as a secondary caregiver. I knew about doctor appointments, factor brands, and clinical trials. But when the roles change, and you're the one who may need caregiving…everything changes.

I remember I encouraged my brother to be positive and live his best life. Hemophilia was not the end of the world! Now, here I am years later, feeling discouraged and defeated, as if hemophilia were indeed the end of the world. My personal journey has been difficult. Witnessing my brother's experience made me hope that I'd also get the attention and treatment I need to move forward. But I was wrong. As much as health professionals may know about hemophilia, it seems that they know it only as it pertains to males. In my experience, the specialists aren't always educated about this bleeding disorder, or maybe they truly don't want to believe that a woman can have hemophilia.

A Surprise Diagnosis

In 2016 I lived in Atlanta, Georgia, and became my brother's legal guardian; he had recently relocated from Miami, Florida, to complete his senior year of high school. During his transition, he lost Florida Medicaid and was also ineligible for Georgia Medicaid. He was left with no insurance.

Later in the semester, he had a hip bleed. We rushed to the ER. Thankfully, the hospital social workers helped him receive emergency Medicaid, which covered his visit. While he was being treated, they suggested I have free genetic testing for parent or guardians, so I did.

My brother wrapped up his final semester as a senior, and I moved to Los Angeles, California, in October 2017. In November my brother called, asking me to contact his nurse. When I called her, she explained that they had been trying to reach me for the last few months. I was in a bit of shock as she told me that I have a factor level of 38%—mild hemophilia A—and that I should register with a local hemophilia treatment center (HTC). I asked her what hemophilia looks like in women. I reflected on my past and current health issues, particularly my prolonged menstrual bleeding. At age 16, I began experiencing irregular menstrual bleeding due to abnormal hormone levels, but by age 22 my periods became more irregular and very prolonged. My menstrual cycle would stop for a day or two and start all over again; this went on for months. Doctors diagnosed me with pre-polycystic ovarian syndrome (PCOS), and started me on birth control right away.

Before my hemophilia diagnosis, my ob/gyn refused to take me off birth control. We agreed that once I reached my weight goal, she would take me off so we could address the underlying issue of what was causing my PCOS or prolonged bleeding. I lost nearly 90 pounds to combat PCOS, and achieved my weight goal, only to have the doctor change her mind and tell me that I should just stay on birth control. I felt defeated. I eventually decided to stop birth control and explore my own options. By this time, I had already relocated to LA and had just received my hemophilia A diagnosis, but I was also having prolonged bleeding. I thought it best to start the journey with the mindset that maybe this bleeding was not just PCOS but also influenced by my new hemophilia diagnosis. So I connected with my PCP and decided to get back on birth control while I figured this all out. My PCP sent a referral for me to be seen by the local HTC in LA. However, my insurance denied it, as well as the appeal, due to "lack of medical necessity." At this point, I didn't have many options, so I figured I should see an in-network specialist.

"Women are just carriers"

On April 5, 2018, I had my first appointment with an oncologist who supposedly had experience treating patients with hemophilia. I told him about my strong family history and that I thought hemophilia could possibly be the real cause of the menstrual issues I'd been having, and not PCOS, as stated by past physicians. Although he had received a copy of the official diagnosis, the oncologist told me that it's unlikely for women to have hemophilia, and that women are just carriers. He suggested I be tested by a lab that he trusted. He also suggested I have an ultrasound performed to look at my ovaries. I complied.

A month later, I returned to this doctor, who said, "Your results came back, and you have mild hemophilia A." This was now my second diagnosis. He told me that I needed to book another appointment in three to four weeks for him to give me a plan for treatment.

Feeling a bit frustrated, I asked, "And what about my menstrual cycle?" The doctor acted confused, as if I'd never talked to him about my irregularities and PCOS. He told me nothing could be done about my irregular bleeding. I asked him, "Aren't you alarmed that I have prolonged bleeding when off birth control, and isn't that a symptom of hemophilia in women?" He then became defensive, and I became even more frustrated because two things became clear: First, the doctor hadn't listened to me during my first visit, due to his assumptions about women with hemophilia; nor had he properly prepared for my visit. When diagnosing me, he spoke as if we had never discussed my family history of hemophilia, or any of the things I'd brought up.

Second, the doctor had never had a female patient with hemophilia. When I asked if he would be open to consulting with Hemophilia Foundation of Southern California or the local HTC to determine a treatment plan for my menstrual cycle, he declined, stating that he could do his own research. I left the office in tears and didn't feel comfortable returning to this doctor. I was still left looking for answers.

In November 2018, I switched insurance plans through a new employer and tried the process again. This journey to finding the underlying issue is important for me. My new PCP, who was also a hematologist, admitted that he didn't think he'd be the most suitable hematologist for me because he was out of practice in that specialty and not familiar with women with hemophilia. He asked me what I wanted to do, and I said I wanted to go to the local HTC. He agreed to send in a referral through our medical group. Again, my referral to be seen by the HTC was denied by insurance because they believed they had specialists more than capable of providing me the care I needed in-house. I obliged once again, and decided to see a specialist they referred me to in-network. But this time I was a bit more optimistic, because the specialist was a woman and this was one of the best health systems in greater LA. There was no reason I wouldn't be able to find the care I needed…right?

I was excited to meet this new specialist on January 7, 2019. She was an oncologist, but she also had experience with bleeding disorders. However, what this really meant was that she was experienced with treating women with von Willebrand disease (VWD). During my first visit, she suggested that my factor levels be tested once again. Although I had provided two prior diagnoses, I agreed. This time, my third diagnosis came back with much lower levels—23%. Most likely, this was due to no longer being on birth control.

Breakthrough

I really wanted to work with this specialist to figure out the best options for me. A friend of mine connected me with the Women's Bleeding Disorder Coalition, which helped educate me about what hemophilia looks like in a woman. I thought it would be a good idea to connect my new specialist to the coalition. Surprisingly, she agreed, and I thought, wow, this is great! They provided my specialist with more information on hemophilia in women. I was happy and excited to hear that my specialist had taken that step on my behalf. But during a follow-up visit, I was taken aback when my new specialist (who I had been bragging about) made comments invalidating the information that was shared with her through the Women's Bleeding Disorder Coalition, because they weren't "medical professionals." I was crushed.

Sadly, I left that appointment with no treatment plan to address my menstrual bleeding, and the only medication offered to me was one most commonly used in women with VWD. Here we are, April 2019, and I still have irregular or prolonged menstrual bleeding. I've been fortunate to see an endocrinologist, who has been working very hard to determine my underlying issue. But at this point, he can't pinpoint the actual cause. The reality is that he is not a hematologist, so he can't help me access factor and attempt a trial treatment to determine whether my prolonged bleeding is in fact hemophilia.

I have also been working with Hemophilia Foundation of Southern California to get access to the GHPP (Genetically Handicapped Persons Program) insurance, which would cover my visits to an HTC. I am currently awaiting a decision and crossing my fingers for a positive outcome. Now, at 28, I just want answers. I just want to know what's the true culprit behind my prolonged menstrual bleeding, and what I can do to control it.

I hope that one day, it won't be so difficult to be connected to a doctor or specialist who really knows hemophilia in general and how to treat women with hemophilia. I also hope that soon, women will have the option to be seen at an HTC as opposed to being forced by insurance companies to see an oncologist.

Women all over the world are gaining a voice through social movements and in politics. It's time for the medical field to give us a voice—and answer, as well.

Milora Morley lives in Los Angeles, CA. She is employed as a business data analyst for Cedars-Sinai Medical Center. She uses her free time productively, as a small business owner of Paramedical Tattoo Services.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

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

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMLIBRA may cause serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including thrombotic microangiopathy (TMA), and blood clots (thrombotic events). If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

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

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

Medication Guide
HEMLIBRA® (hem-lee-bruh) (emicizumab-kxwh) injection, for subcutaneous use

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

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

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - confusion
  - weakness
  - swelling of arms and legs
  - yellowing of skin and eyes
  - stomach (abdomen) or back pain
  - nausea or vomiting
  - feeling sick
  - decreased urination
- **Blood clots (thrombotic events).** Blood clots may form in blood vessels in your arm, leg, lung, or head. Get medical help right away if you have any of these signs or symptoms of blood clots during or after treatment with HEMLIBRA:
  - swelling in arms or legs
  - pain or redness in your arms or legs
  - shortness of breath
  - chest pain or tightness
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

See "**What are the possible side effects of HEMLIBRA?**" for more information about side effects.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.

HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

Before using HEMLIBRA, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA.
- are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

Tell your healthcare provider about all the medicines you take,

including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- **Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis.**
- **You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.**
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.
- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.
- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- You will receive HEMLIBRA 1 time a week for the first four weeks. Then you will receive a maintenance dose as prescribed by your healthcare provider.
- If you miss a dose of HEMLIBRA on your scheduled day, you should give the dose as soon as you remember. You must give the missed dose as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. **Do not** give two doses on the same day to make up for a missed dose.
- HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

What are the possible side effects of HEMLIBRA?

- See "**What is the most important information I should know about HEMLIBRA?**"

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
- Store HEMLIBRA in the original carton to protect the vials from light.
- Do not shake HEMLIBRA.
- If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator for more than a total of 7 days or at a temperature greater than 86°F (30°C).
- After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.
- Throw away (dispose of) any unused HEMLIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

General information about the safe and effective use of HEMLIBRA.

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMLIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMLIBRA that is written for health professionals.

What are the ingredients in HEMLIBRA?

Active ingredient: emicizumab-kxwh

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

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For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration
Revised : 10/2018

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

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What's the Best Way to Learn About Your Bleeding Disorder?

Laurie Kelley

Our world is biased toward visual information and learning, because humans are mainly visual beings. From advertising to teaching tools, we are all about sight, color, and shape. Studies have shown that 65% of people learn best visually.*1*

But not everyone learns visually. It's now recognized that people have individual differences when it comes to learning, and even visual learners learn in different ways. For example, one person learns by visiting art museums, but another likes watching TV documentaries; both are visual methods.

Research in the field of learning modes, or styles, led to the widely cited Theory of Multiple Intelligences, developed in 1983 by Harvard University's Howard Gardner. Gardner found nine distinct types of "intelligences," which are now used to describe how people learn. What's your preferred learning mode? And how can you harness it to learn about managing your bleeding disorder?

Nine Learning Modes

You may possess several of the nine modes listed here, with one being dominant. Or you may use different modes in various circumstances. For example, I'm a verbal learner who likes to learn in a solitary way. But I occasionally like to learn a new piece on the piano for fun, which requires visual, aural, and physical modes. You can also change your mix by developing and enhancing your less dominant modes. If you don't use particular learning modes, they may weaken.

1. Visual (spatial): pictures, images, understanding of space
2. Aural (auditory-musical): sound, music
3. Verbal (linguistic): words, in both speech and writing
4. Physical (kinesthetic): body, hands, sense of touch
5. Logical (mathematical): numbers, logic, reasoning, systems
6. Social (interpersonal): learning by being with other people
7. Solitary (intrapersonal): learning alone, using self-study
8. Natural: being outside; identifying plants, animals, maps
9. Existential: questioning human existence, the meaning of life and death, the human condition

The key to learning about bleeding disorders is to recognize how you learn best, and then to find resources that use your strongest modes to help you learn faster and more effectively.

In the Beginning, There Were Books

There was a time when virtually no information on hemophilia or von Willebrand disease (VWD) was available in any learning format, except meeting with your hematologist. That's one of the reasons I wrote Raising a Child with Hemophilia in 1990, and published all of our subsequent books: to reach the verbal-oriented people in our community, and to provide a tool for patients to use all the time, not just at in-person meetings. For me, social support group meetings weren't so useful. More experienced parents seemed to want to scare the rest of us with their bleeding horror stories! Medical journals and published articles seemed more logical and reliable.

Fortunately, for those who are not mainly verbal learners, we now have plenty of other ways to learn. Chapters hold more local meetings, which may appeal to more social-oriented people; national meetings offer scholarships for first-time participants; CDs, DVDs, podcasts, and YouTube videos are available; and now there's even a movie—Bombardier Blood. Just recently, Hemophilia: The Musical debuted!

Patrick "Big Dog" Torrey, founder of GutMonkey, offers the bleeding disorder community learning through physical and natural modes: rafting, hiking, zip-lining, and camping. No matter your learning style, there's bound to be some educational resource you'll enjoy, to help you understand your bleeding disorder.

What YOU Find Most Useful

We asked our Facebook friends what resources have helped them most. Their answers reveal preferred learning modes. Phillip Smith, for example, offered what sounded like a logical mode of learning: direct, scientific information. "I read product inserts, news releases from the company that produces the product, and company R&D pipeline info on future products."

But any learning mode may shift when you're stressed. It's normal for people to want to be with others for support, which results in learning. So social learning may become dominant for a while. Given that, it's no surprise that many Facebook friends mentioned chapter meetings, symposia, and especially meeting with hemophilia treatment center (HTC) staff to get information about bleeding disorders.

"I most value learning from the experiences of those who are living and thriving with hemophilia," commented one mother of a child with hemophilia. Jasmine Eaglin, another mother, wrote, "First I learned about NHF, then I got involved with the New York City chapter and attended every single event I could. Eventually, I made it to an HFA conference in 2017. The organizations provide so much information!" Heather Coons, mother of a 13-year-old with hemophilia, wrote, "I learned from books, but talking to the HTC staff and families helped me the most."

And not surprisingly, many who started with only books, back in the 1990s and 2000s, are now turning to online resources. Diana Lynn shared: "In the beginning it was Raising a Child with Hemophilia. Now that our son is older, we look to meetings and websites (mainly medical/journal articles) for our information."

Allison Pohl summed up this change in information sources: "When our son was diagnosed in 2000, the main resource for information on hemophilia was our HTC staff, and Raising a Child with Hemophilia. There was no social media back then, and most information we received was passed face to face or by snail mail."

With all the options available, Allison still relies mainly on her HTC. But, she noted, "In addition, we read PEN, speak to product reps, and go to our local chapter meeting. If I want info about products or trials, I usually google scientific journals. I try to stay away from anecdotal info and stories on social media. While I like the personal experiences, sometimes the advice given is not correct."

Social Media: Not Just for the Young and Brave

Young people with bleeding disorders seem to gravitate toward social media for information. Dakota Rosenfelt cited Twitter ("HUGE," he wrote), NHF's website, and even the European Haemophilia Community website. One mother of a two-year-old with hemophilia wrote,

"I love online resources, and found most of social media to be an amazing resource."

Knowing that learning styles are changing, and tapping into the way young people learn about their world, Patrick J. Lynch's company Believe Ltd. has focused on social media

and digital resources like podcasts (BloodStream Media), videos ("HTC Guided Tour"), plays (Stop the Bleeding), forums ("Powering Through"), and now musicals (Hemophilia: The Musical). These resources represent a combination of learning modes, including social, aural, physical, and visual. Indeed, multimedia is becoming the best way to reach all types of learners.

On the Fringe

But a preferred learning mode may not matter when you have limited resources. People with VWD or rarer bleeding disorders, as well as those living in developing countries, don't always have the luxury of choice in learning modes.

Leticia Nevarez, a Facebook friend, points to our book A Guide to Living with von Willebrand Disease as the first resource she ever had about VWD. Fortunately, a national conference (NOW; see page 11) offers a weekend of presentations and socializing, as well as Facebook social media pages where patients can share VWD experiences. Helen Smith, a woman with Glanzmann's thrombasthenia, started Glanzmann's Research Foundation, a nonprofit organization dedicated to offering information to patients. She now wants to hold a weekend retreat to allow patients to share in person.

Patients and families in developing countries are perhaps most limited. Without books translated into their national language, and with travel to events almost impossible, they rely mainly on the internet, accessed through their phones. This includes Facebook, which offers translation.

Patients from Kenya to Pakistan, from Nigeria to the Philippines shared their thoughts on accessing information. Samad from Pakistan learns mostly from his doctors; Sarah from Kenya learns from other patients; and one doctor from Cambodia cites Twitter and Facebook as his primary source of info. WhatsApp is popular with youth everywhere, allowing them to share experiences as a private group. When learning about your bleeding disorder, think about how you learn in general. Which modes make you happiest? Which help you learn best? Seek out bleeding disorder resources that match your preferred learning modes.

Suzanne Harpell-Smedley summed up how multimedia learning can work: "Initially, we devoured anything by NHF. Then we found LA Kelley Communications' books. With medical complications, we researched online and have auto-email updates sent to us. We regularly attend inhibitor summits and occasionally our local hemophilia groups. Last of all, we turn to Facebook for real-world experiences." Whatever your preferred learning mode, the key is to find out about your disorder, and to make learning effective and fun!

1. Richard M. Felder and Linda Silverman, "Learning and Teaching Styles in Engineering Education," Engineering Education 78(7), 674–81 (1988). This study later became a foundation for a standardized test called the Index of Learning Styles (ILS). Available at www.wiley.com.

PING (Parent Information Networking Group)

It's the most wonderful time of the year and we finalized our event calendar with our Parent Information and Networking Group (PING) event at the Lincoln Children's Museum. We had a great education session on the unaffected sibling and dinner provided by Sanofi Genzyme. That was followed by a visit from the Jolly man himself, Santa! He gifted all the kids Target gift cards and the adults got to enjoy hot chocolate in new NENHF mugs. This event is for our families with kids 14 and under to get together, play, network and have fun. It's a great way to end out year and let everyone enjoy some social time as the kids take over the museum. Happy Holidays from the Board and Staff to all of you!

Thank you to our 2019 PING Sponsors:

Aptevo, Brother's Healthcare, CSL Behring, CVS Specialty, Novo Nordisk, Sanofi Genzyme, Takeda, Superior Biologics and Genentech.

A HUGE Thank you to Colburn Keenan Foundation for sponsoring Santa, presents for the kids and mugs for the adults. It was such a special treat to be able to give to our community.

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Hemophilia Science Update: Dr. Alex Nester

Hemophilia Science Update:

At the recent American Society of Hematologists meeting in Dec 2019, a special highlight session was held to discuss Hemophilia A and B, as they continue to have positive signs for new treatments with gene therapy likely available by the end of 2020.

In short, gene therapy consists of using a viral vector such as adeno-associated virus (a cousin to the common cold not known to cause disease in humans) to introduce missing or damaged genes into a patient's cells. As both hemophilia a and B are the result of single protein deficiencies, they represent choice targets for single gene treatments, especially as moderate increases in protein levels can have drastic changes in their disease state.

While initial gene therapy trials were disappointing in their transient increase in factor levels, more recent studies have shown persistent improvement in factor levels, currently 8 years after therapy! Currently, hemophilia A gene therapy trials are in phase 1/2 for evaluation of safety and efficacy, however hemophilia B gene therapy trials are currently in phase 3 for comparison against current treatment. This is generally the last step prior to FDA review and final release to the public.

Obstacles to gene therapy include a need for close monitoring of liver function testing and may need use of steroids to avoid liver damage. The cause of this is currently unknown. Additionally, people with antibodies or immunity to the AAV8 virus have been excluded from trials and may not respond to treatment. Finally, as with all new therapies, there will need to be close monitoring for any adverse events occurring long after therapy.

About Dr. Nester:

I originally grew up in Sioux Falls, South Dakota with family roots in Minnesota and North Dakota. I did my undergraduate studies in Minnesota, with some graduate studies and lab work in Washington DC before returning to South Dakota for medical school. I graduated in the spring 2013 and, despite interviewing during a blizzard, chose to do my internal medicine residency at UNMC in Omaha, Nebraska. I then stayed on for my hematology and oncology training in the new Buffett Cancer Center with an additional externship in benign hematology at the University of North Carolina. After graduating, my wife and I realized that we had found our home, and I am staying on at UNMC with a specialty in benign hematology. At home, we are happily busy raising our daughter (who is teaching me to cook) and twin sons (who I am teaching about Star Wars and Marvel superheroes).

University of Nebraska Medical Center
42nd and Emile, Omaha, NE 68198
402-559-4000

Thank you for a wonderful year!

Nebraska Chapter
National Hemophilia Foundation

In 2019, we:

- Served more than 75 families through the expansion of enhanced programming opportunities to underserved populations such as women, teens, and those in our rural areas.
- Awarded over $7,000 in travel grants and financial assistance to help community members attend Washington Days and NHF's annual Bleeding Disorders Conference, as well as clinic or camp.
- Provided over 18 bleeding disorders specific educational opportunities across Nebraska, including: two education weekends, eleven education dinners, and five targeted learning programs focusing on personal advocacy, women who bleed, transition care, and teaching self-infusion.

As the year comes to a close, you can make a true impact by helping us provide much-needed resources and support to our fellow Nebraskans living with a bleeding disorder.

Looking forward to a healthy & happy 2020 with you!

Thank you to our 2019 sponsors and donors!

Kid's Corner

New Family Focus: When your mom or dad gets married again

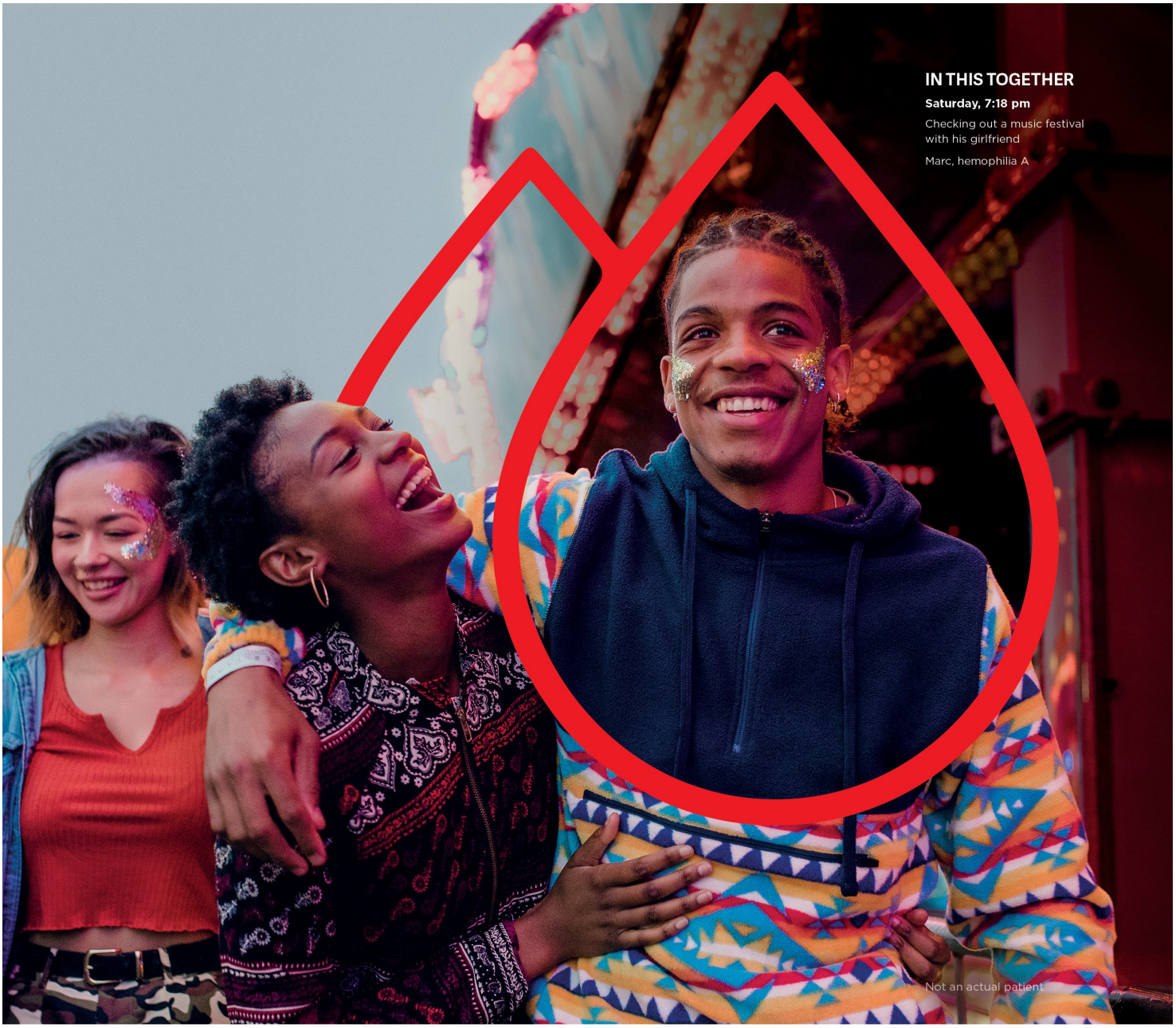
Author: Leslie Quander Wooldridge

If your mom or dad is getting married again, you might feel happy. Then again, you might feel angry, sad or even scared. Change can be hard for everyone. Here are five things to try if you're getting a new stepparent or even stepsiblings. Who knows, they might be pretty cool if you give them a chance!

Take things slowly. You'll need to adjust to having a new stepparent or new stepsiblings. If you're comfortable, see if they want to join you in doing activities you like. But remember: It takes time to get to know people, so give yourself plenty of time as your new family settles in.
Show respect. You don't need to rush to love your new family, but you should respect them. That means you should listen when your parent or stepparent gives you instructions. And be nice when you talk to your new family members, especially new sisters or brothers.
Be real with your feelings. Kids have all kinds of emotions when families come together. What you're feeling—whether you're scared, sad, excited or even annoyed—is normal, and it's OK to show it. But be real, not rude!

Share your thoughts, too. There may be lots of changes in your home life, so be honest. If you don't want your new stepmom to help with infusions or if sharing a room with your new step-brother is hard, say so. Tell your parent and stepparent what you really think. Or reach out to another adult you trust. Maybe your uncle or grandma is easier to talk to. The point is to be truthful, so that adults can consider your point of view.

Try drawing or writing. If you just can't talk about your thoughts and feelings because you're shy or don't know what words to use, try drawing or writing about how you feel. The adults in your life want the best for you. They want you to feel comfortable with changes that are happening. So say or show something, so that the people you love can help!



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

bleedingdisorders.com

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