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Telemedicine surged during the pandemic. What does the future hold for this technology?

HEM AWARE

The Bleeding Disorders Magazine

 NATIONAL HEMOPHILIA FOUNDATION | hemaware.org



TEENS AND
MENTAL HEALTH

Breaking Down Barriers to Care

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Help the hemophilia community by taking part in a research study

Sponsored by **B:OMARIN**

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

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

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

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

How does the study work?

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

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

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

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



Hear what the experts are saying

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



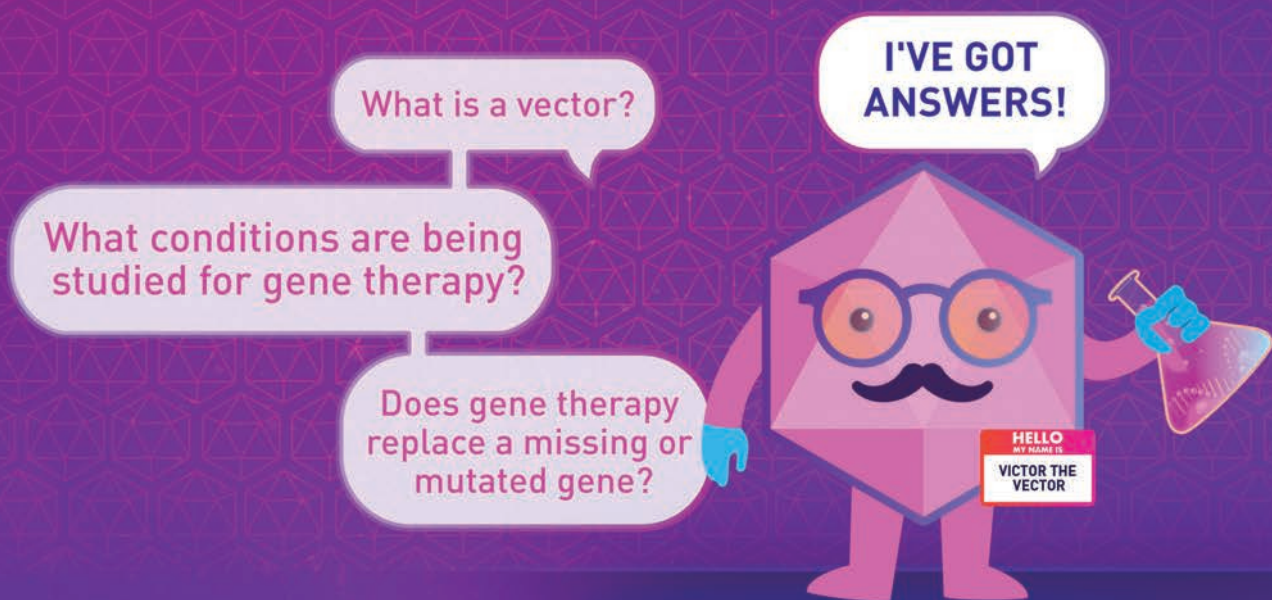
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Dawn Rotellini
Chief Operating
Officer, NHF

Watch the video at saavy-study.com

DO YOU HAVE QUESTIONS ABOUT GENE THERAPY RESEARCH?



DID YOU KNOW

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



WANT TO STAY IN THE KNOW?

Just sign up for the *Gene Therapy Learning Academy* and get the latest news on gene therapy research and educational resources—delivered right to your inbox.



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Photo illustration by Stephanie Dalton Cowan





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In Focus

PRESIDENT AND CEO LEONARD A. VALENTINO, MD



**It was wonderful
to reconnect with
so many of you
at BDC 2022, and
to welcome first-
time attendees to
the conference.**

A Time of Gratitude and Celebration

As we close out this year, we at the National Hemophilia Foundation (NHF) have a lot to celebrate — and a lot to look forward to in 2023.

In August, after two years of not being able to meet in person, we were delighted to gather our community in Houston for the 74th Annual Bleeding Disorders Conference.

It was wonderful to reconnect with so many of you at BDC 2022, and to welcome first-time attendees to the conference.

This year, we also celebrated the 50th anniversary of the Judith Graham Pool Postdoctoral Research Fellowship. NHF's premier research fellowship program was named in honor of the scientist whose remarkable discovery of cryoprecipitate in 1965 opened the door to a series of research

developments that would revolutionize treatment for hemophilia. You can read more about the fellowship and some of its recipients on Page 24.

Speaking of anniversaries, we're gearing up for a big one. In 2023, NHF will celebrate its 75th year. To commemorate this momentous occasion, NHF is launching a celebratory and reflective campaign called The Red Thread, which will tell the organization's history and evolution since its 1948 founding. Learn more about what's in store on Page 9.

The end of the year is also a time to give thanks, and I'm especially grateful for all of you who have made a positive impact in our community. I'm excited about all we can achieve together in 2023 and beyond.

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YouTube: youtube.com/NHFvideo

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



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HemAware (ISSN 1088-7008) is published two times annually by the National Hemophilia Foundation (NHF), 7 Penn Plaza, Suite 1204, New York, NY 10001, 212.328.3700, toll free 800.42.HANDI, fax 212.328.3777. The opinions expressed are those of the authors alone and do not represent the opinions, policies or position of NHF, the Board of Directors or editorial staff. This material is provided for your general information only. NHF does not give medical advice or engage in the practice of medicine. NHF under no circumstances recommends 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. All manuscript submissions should be typed double space, between 500 and 1,000 words in length and sent by email to donna.behen@manifest.com. HemAware retains the right to edit submissions. The content of the article is the responsibility of the author(s) and must be original work that has not been, nor will be, published elsewhere. A copyright agreement attesting to this and transferring copyright to NHF is required.

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CONGRATULATIONS, Elvira and Michelle!



ELVIRA GOODY

Executive Assistant, 2010 - 2022

MICHELLE WITKOP, DNP, FNP-BC

Vice President of Research Strategy,
2017 - 2022



NHF thanks these longtime employees for their service and dedication to the inheritable blood and bleeding disorders community, and congratulates them on their retirement.



NATIONAL HEMOPHILIA FOUNDATION

for all bleeding disorders



**'TIS THE SEASON
TO GIVE BACK!**

**IS NHF ON
YOUR GIFT LIST?**

Wrap up 2022 with a tax-deductible
donation in support of our friends and
family with inheritable blood disorders.

Give now at hemophilia.org/donate



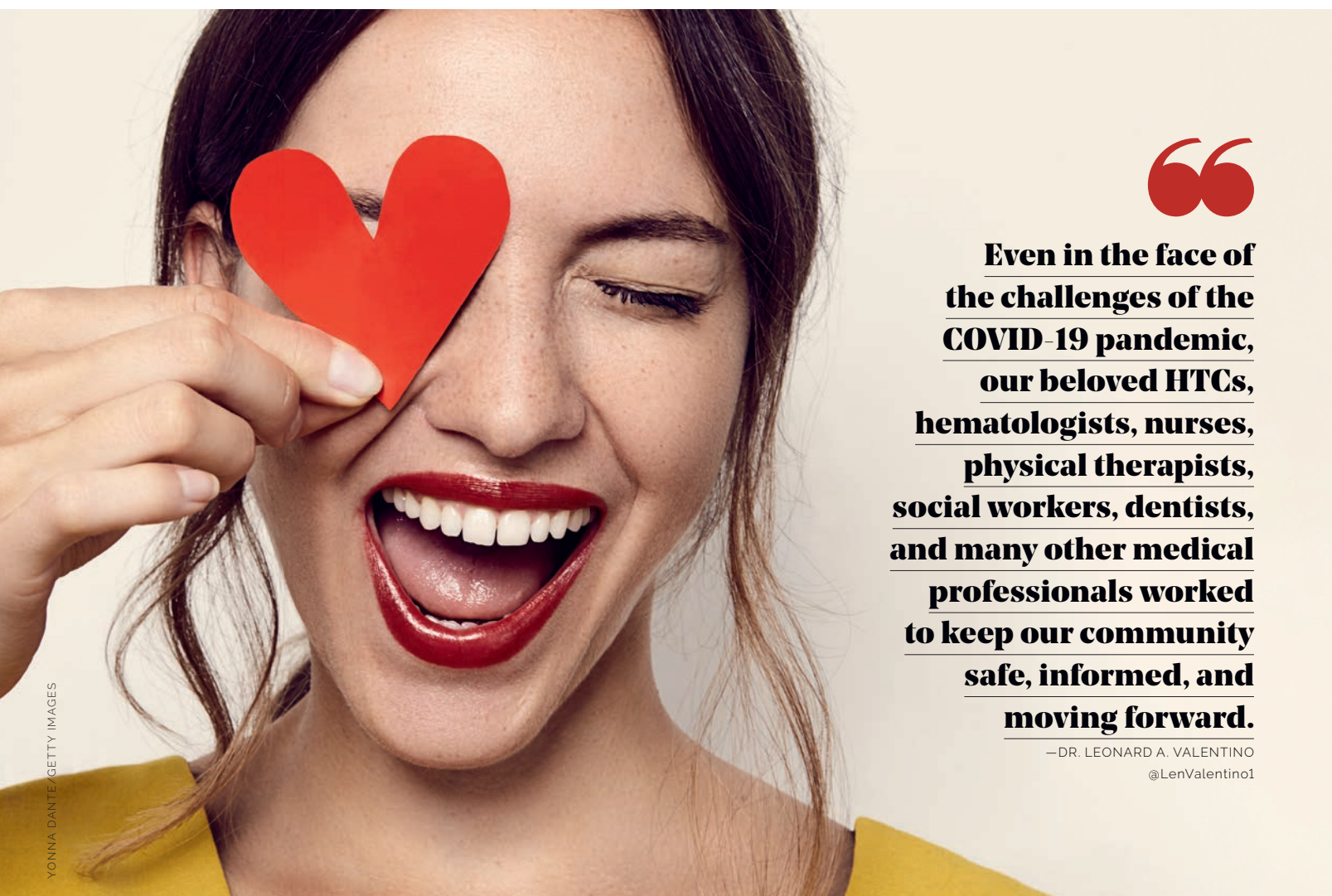
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for all bleeding disorders

Healthy Start

STATS, FACTS AND NEWS YOU CAN USE

Spreading the Love

We asked community members, "What do you love about your health care provider?"
Here are some of your answers:



Even in the face of the challenges of the COVID-19 pandemic, our beloved HTC's, hematologists, nurses, physical therapists, social workers, dentists, and many other medical professionals worked to keep our community safe, informed, and moving forward.

—DR. LEONARD A. VALENTINO
@LenValentino1

Our Hem/Onc team listens! They care about every aspect of living with a bleeding disorder! Couldn't do it without them!

—@COFFEEWHILEREAD

My physical therapist is a true advocate for her patients, and always encouraging us to achieve our goals.

—SERENA C.

They take time to LISTEN to me about what is going on with MY body as the subject matter expert living with factor VII deficiency.

—@CONNIEM886



EQUITY FOR PEOPLE WITH BLEEDING DISORDERS

The National Hemophilia Foundation (NHF) held its first Health Equity Summit for people with inheritable blood and bleeding disorders in June. The two-day event in Atlanta focused on ways to address health disparities among people within the blood disorders community. The summit demonstrated NHF's "commitment to health equity, our community, and understanding their concerns as it pertains

to access, mental health, payer/policy and health-system navigation," says Keri L. Norris, Ph.D., vice president of health equity, diversity, and inclusion. Doctors, researchers, community members, and other experts spoke about ways to decrease health disparities and increase access to care to positively impact health outcomes among people from all communities. "We all are committed to moving

Debunking Myths About Clinical Trials

Have you considered participating in a clinical trial? When researchers are investigating the effectiveness of potential new medical treatments, they need patients who meet specific criteria to volunteer, which helps them determine whether new drugs, medical devices, or other interventions could expand treatment options for people with that condition.

Some people are hesitant to participate in clinical trials because they believe the myths they've heard about the process. Learning the truth may encourage you to join a clinical trial that may help to advance treatment for your blood disorder or another health condition.



MYTH: RESEARCHERS CAN DO WHATEVER THEY WANT TO YOU, AND THEY DON'T HAVE TO TELL YOU.

FACT: Informed consent is an important part of the onboarding process for all clinical trials. Researchers must let you know what they will be doing before you sign on, and they're required to prioritize your safety. They're also independently monitored by an institutional review board, which ensures that participants are protected, and the research is ethical.



MYTH: ONCE YOU SIGN UP FOR A TRIAL, YOU'RE STUCK, EVEN IF YOU CHANGE YOUR MIND.

FACT: Because clinical trials are voluntary, it's possible to stop your participation at any time.



MYTH: ONLY WHITE MALES ARE INCLUDED IN CLINICAL TRIALS.

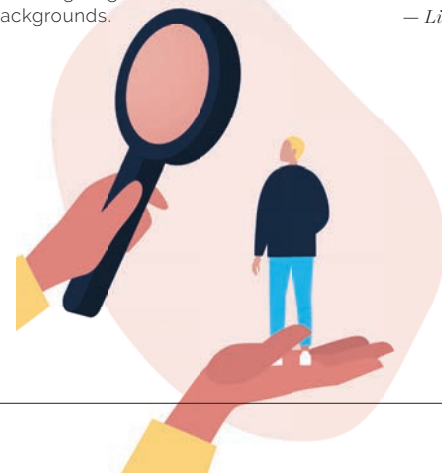
FACT: Decades ago, clinical trial rosters often focused on white males, but things have changed. Today, researchers include males, females, and people of different racial and ethnic backgrounds in clinical trials, as well as young people and older adults. This diversity helps researchers understand how diseases affect people of different ages, genders, and backgrounds.



MYTH: IF I VOLUNTEER FOR ONE CLINICAL TRIAL THROUGH MY DOCTOR OR HEMOPHILIA TREATMENT CENTER, I'LL HAVE TO JOIN EVERY OTHER CLINICAL TRIAL THAT COMES UP.

FACT: Joining one clinical trial doesn't give you license to participate in other trials. Each one is independently run, with its own goals and criteria. Even if you're selected for one study, you may not qualify for another, based on those researchers' needs.

— Lisa Fields



FOOD DISORDERS

forward with understanding our inherent biases, working within the social determinants of health to address health equity, and elevating the voice of subject matter experts in leading the work," Norris says. "That would include engaging those hard-to-reach populations at every level, and to ensure that they are working toward equitable access to care, quality treatments, and mental health resources needed."



The Red Thread: NHF's 75th Anniversary

NHF celebrates its 75th anniversary in 2023! To honor this momentous occasion, the organization will soon be launching a celebratory and reflective campaign called "The Red Thread" — which will tell the organization's history and evolution since its 1948 founding. Throughout this yearlong campaign, NHF will tell the story of its 75 years of service, advocacy, and education, as well as growth, resilience, and community.

You're invited to be a part of NHF's anniversary celebrations and its next chapter. Be sure to follow NHF on social media to see historic moments, tributes, and more. And stay tuned for a soon-to-launch website that will feature a historical timeline.

Help honor 75 years of hope and progress by sending an email to communications@hemophilia.org to share your memories. Your reflections may appear in an upcoming issue of *HemAware* or on NHF's social media!



GIVING TUESDAY

Giving Tuesday is a global day of giving held each year on the Tuesday following Thanksgiving. At NHF, it's "Giving Shoesday." Now in its third year, the fundraising campaign gives limited-edition NHF Converse and Nike sneakers to individuals who make minimum donations. A new shoe design will be revealed exclusively for this event. Don't miss out!



LEARN MORE AT
hemophilia.org/givingtuesday

CLOCKWISE FROM TOP LEFT: KLAUS VEDEFELT/GETTY IMAGES, WARREN JONES/UNSPLOASH, AMIRALI MIRHASHEMIAN/UNSPLOASH, NADIA BORMOTOVA/GETTY IMAGES

One in a Million

Bridget Edwards doesn't let her rare bleeding disorder define her

Bridget Edwards was diagnosed with a rare bleeding disorder as a newborn, after a routine heel stick wouldn't stop bleeding. Doctors found that she had afibrinogenemia, a lack of fibrinogen (factor I). This autosomal recessive inherited disorder is so rare, it affects just 1 in 1 million people. Her first hematologist hadn't treated afibrinogenemia before, so he wasn't sure what to recommend to Edwards' parents.

"We learned by trial and error," says Edwards, 27, of Brewer, Maine. "My parents did a great job. I was never told, 'You can't do this, you might get hurt.'"

Edwards grew up playing sports, making modifications as needed. She never slid into bases during softball, for example. In high school, she played volleyball,

becoming captain of the school team and playing for the Junior Olympic team, which traveled throughout New England.

She wasn't always happy about the attention she sometimes received because of having afibrinogenemia. When a volleyball injury caused an internal bleed that required frequent infusions, she needed a central catheter. "I received a lot of uncomfortable looks and stares, feeling as though I was being put into a category of 'she has something that's wrong with her,'" says Edwards, who noted that sometimes peers had negative reactions. "If I was injured due to an internal muscle bleed and said, 'I can't participate in practice today,' they couldn't physically see what was wrong. I'd hear, 'You're faking it for attention.'"



GAME ON!
Edwards has strived to succeed, on and off the court.

"No matter what happens to me medically, I never let that impact my ability to succeed."

Growing up, Edwards didn't know anyone with afibrinogenemia, which was isolating. Going to college inspired her to talk about her condition, raise awareness, and find others who are missing factor I. "Through that, I have met many more people than I ever knew growing up who have afibrinogenemia," Edwards says. "I didn't realize, until I went to college, how much I was holding in."

Sharing her story helped Edwards connect with the Hemophilia Alliance of Maine, where she later worked. She's currently on its board of directors. "I try as much as I can to encourage more advocacy and awareness about rare bleeding disorders," Edwards says. "The more awareness that occurs, the more knowledgeable medical providers become, which in turn can make it easier for us living with these rare deficiencies."

In June, she married Jonathan, whom she's known since elementary school. "I find that a lot of people with chronic medical conditions can often feel like a burden on their loved ones, as I myself sometimes have, and Jonathan has never made me feel that way. He is definitely one of my biggest supporters," says Edwards, who has a master's degree in health psychology and works as a college success specialist for Jobs for Maine Graduates at Eastern Maine Community College.

"No matter what happens to me medically, I never let that impact my ability to succeed," she says. "I strive to let other people know that even though someone may have a rare disorder, they're still capable of living a fulfilling life."

—By Lisa Fields



FOR MORE INFORMATION ABOUT BLEEDING DISORDERS IN WOMEN: stepsforliving.hemophilia.org/basics-of-bleeding-disorders/bleeding-disorders-in-women
VISIT VICTORY FOR WOMEN WITH BLOOD DISORDERS: victoryforwomen.org

Warning Signs on Memory Lane

Your brain changes as you age. Here's how to tell what's to be expected and what's cause for concern.

LOSING THE REINS
Struggling to do familiar activities could mean cognitive trouble.



George Stone recently began having trouble with his financial record keeping, a new experience for him. "All of a sudden, in the last few months, I've screwed up," says Stone, 68, of Lake Frederick, Virginia. "I think I paid bills only to find out that I didn't."

It's confusing because other signs tell him his mind is doing fine. Not long ago, he missed only one out of 50 questions on a highly technical test to upgrade his ham radio license.

His wife has noticed when there are problems, too. As the two of them handle the bills together now, Stone, who has severe hemophilia A, wonders if he's experiencing normal signs of aging or something more serious, such as dementia.

Over the past several decades, the life expectancy of people with bleeding disorders has risen. That's good news, of course. But as people get older, age-related cognitive decline and neurological conditions such as Alzheimer's and Parkinson's diseases become more common.

How do you know what's normal and what's not?



Normal: Slow to Process



Not: Forgetting the Familiar



Concerned? See a Doctor

"Your doctor can decide whether what you're experiencing is normal or not."

1 WHAT'S NORMAL

As you age, your brain tends to slow down a bit. For example, it might take you longer to develop plans or answer questions. "Just because you're slower at processing information, it does not necessarily mean there is cause for concern," says neurologist Douglas Scharre, M.D., director of cognitive neurology at the Ohio State University Center for Cognitive and Memory Disorders in Columbus, Ohio.

With age, you also may have a harder time learning new things and need reminders for taking medication and other daily tasks, says Beril Yaffe, Ph.D., a neuropsychologist at Lenox Hill Hospital in New York City. "It is normal for people to decline in certain areas like these," she says.

2 WHAT'S NOT NORMAL

Misplacing your phone now and then is one example of normal cognitive decline. But if you regularly lose track of your phone or you can't remember how it got there when you find it, that could indicate a deeper problem.

Getting lost or turned around in familiar places also is a red flag. "If your barbershop is 3 miles from your house, and you can't remember which side of the street it is on, that's not normal," Scharre says.

Another sign that may warrant attention: asking the same question repeatedly because you don't remember that you've asked it before.

Yaffe says that if you begin to have trouble with activities you were previously able to

do, such as shopping and preparing meals, something more serious than normal aging might be to blame.

3 WHAT YOU SHOULD DO

If you have concerns about changes in your cognitive abilities, see your doctor. That's exactly what Stone is doing — he already has an appointment with his psychiatrist.

"You should go in as soon as you can," Scharre says. "Your doctor can decide whether what you're experiencing is normal or not. If you're diagnosed with an underlying condition, your doctor can also suggest next steps that could potentially help slow cognitive decline."

— By Matt McMillen



5 WAYS TO BOOST YOUR BRAIN HEALTH: Do these five things to help keep your mind sharp and stave off age-related cognitive decline: hemaware.org/mind-body/protect-cognitive-health

SPEAK UP

Let your elected officials know that contraception isn't just for birth control.



Safeguarding an Essential Treatment for Heavy Periods

What contraception bans would mean for the bleeding disorders community

In June, when the Supreme Court overturned *Roe v. Wade* and ended the constitutional right to abortion, women's health advocates around the country expressed concern that the right to contraception might also be at risk.

"This issue is hugely important to our community," says Nathan Schaefer, vice president of public policy for the National Hemophilia Foundation. "Many people with bleeding disorders rely on birth control — in a variety of forms (IUDs, pills, etc.) — to control their condition. Without access to birth control, the risk of uncontrolled bleeding, the need for a hysterectomy, or even potentially fatal incidents may increase."

In addition, pregnancy can be more dangerous in people with bleeding disorders, so they may choose to use hormonal contraceptives to avoid it.

WHY MIGHT CONTRACEPTION BE BANNED?

Many myths persist that emergency contraception and IUDs cause abortion, despite scientific evidence to the contrary. "Legislators conflating abortion and certain methods of birth control — in particular intrauterine devices (IUDs) and emergency contraception (EC) — are taking advantage of a lack of knowledge about these methods, and people's understanding of how birth control works," states the National Women's Law

“It shouldn’t be a divisive issue, because this is a documented, evidence-based, FDA-approved treatment for patients who have heavy menstrual bleeding and have bleeding disorders.”

Center. “These policymakers are preying upon abortion stigma, believing that if they can convince people that birth control methods are abortion, they can successfully restrict access to birth control — or ban it altogether.”

Another concern is that pharmacists and store clerks may decide not to fill or sell contraceptives if it goes against their religious principles. In the summer of 2022, a Walgreens clerk reportedly refused to sell a married couple condoms because of his faith. “That could create areas of lesser access to care. This, to me, seems more imminently likely, but who can predict what will occur?” says Shannon L. Carpenter, M.D., M.S., a hematologist at Children’s Mercy Hospital in Kansas City, Missouri.

“It’s unethical for pharmacists to not fill prescriptions that have been written,” adds Maureen Baldwin, M.D., MPH, associate professor of obstetrics and gynecology at Oregon Health & Science University. “In addition, pharmacies are required under federal civil rights law to fill prescriptions without making determinations about the suitability of a prescribed medication for a patient.”

NOT JUST FOR BIRTH CONTROL

Today, providers prescribe hormonal contraception for a wide variety of reasons other than preventing pregnancy. “For people with bleeding disorders, it’s about regulating their menstrual cycles, but it can also help with PMS symptoms, cramps, anemia, endometriosis, ovarian cysts, PCOS (polycystic ovary syndrome), and more,” says Marybec Griffin, Ph.D., an assistant professor at the Rutgers University School of Public

Health who studies women and periods. “A ban would impact a lot more than just somebody’s ability to get pregnant. Wherever we see limited access to abortion and limited access to contraception, we see worse health outcomes in a lot of different ways, too.”

Baldwin believes that if hormonal contraception is banned for use in preventing pregnancy, it won’t be banned for all other uses. “There are many hormonal medications that have indications other than contraception. If they are FDA-approved for management of heavy menstrual bleeding, among other things, they won’t be banned for those indications.”

Providers should write the indication that they’re prescribing the medication for on records and documents, Baldwin recommends, and avoid using the terms birth control or contraception unless they are using it for that indication. “This may help with reluctant pharmacists and with patients who are put off by the stigma of birth control,” she says.

ADVOCACY IS KEY

Carpenter urges health care providers, whether they are pro-choice or anti-abortion, to start advocating for their patients. “There are going to be people within the bleeding disorders community who may feel that overturning Roe was the right choice, but that doesn’t mean they can’t still advocate for bleeding disorders patients to get this medication without betraying their beliefs,” she says. “This isn’t a pro-life or pro-choice stance. It shouldn’t be a divisive issue, because this is a documented, evidence-based,

FDA-approved treatment for patients who have heavy menstrual bleeding and have bleeding disorders.”

NHF’s Schaefer says patients should be vocal advocates, too: “Contact your elected officials. Write letters, call, email, share on social media. Explain to them that for you, birth control is more than just a measure of reproductive rights — it’s a tool to control your health writ large. Explaining the difference that birth control has made in your bleeding disorder journey can educate others to the many important medical uses of birth control.”

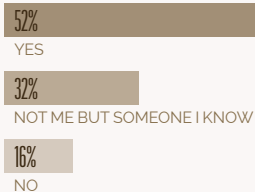
“We have heard from a significant number of chapters that access to reproductive care for women with bleeding disorders is uncertain,” says Kristi Harvey-Simi, NHF’s director of chapter development. “NHF is eager to listen to community members’ concerns and do everything we can to ensure access to health care — both treatment and prevention — is guaranteed, for all people impacted by bleeding disorders.”

—By Beth Levine

A Widespread Issue

NHF asked community members* on social media:

“Would a ban on birth control impact your blood/bleeding disorder?”



*400 respondents



FOR MORE INFORMATION ABOUT BLEEDING DISORDERS IN WOMEN: stepsforliving.hemophilia.org/basics-of-bleeding-disorders/bleeding-disorders-in-women
VISIT VICTORY FOR WOMEN: victoryforwomen.org >> THE NORTH AMERICAN MENOPAUSE SOCIETY: menopause.org >> FOOD AND DRUG ADMINISTRATION CONSUMER INFORMATION ABOUT MENOPAUSE: fda.gov/ForConsumers/ByAudience/ForWomen/WomensHealthTopics/ucm117978.htm

Cloud of Concern

Vaping is all the rage these days. Discover why it's so dangerous for your teen and what you can do about it.

It's a pervasive problem facing young people. More than 2 million U.S. students in grades 6 to 12 vape, with e-cigarettes the most used tobacco product by the group, according to the 2021 National Youth Tobacco Survey.

Vaping is the use of an electronic device (e-cigarette) that heats a liquid into an aerosol that is inhaled. The liquid often has nicotine but also can have THC (the ingredient in marijuana that produces the "high"). Even e-cigarettes that claim to be tobacco-free contain some nicotine. E-cigarettes often look like actual cigarettes but can be shaped like a USB flash device or a pen.

VAPING'S EFFECTS ON TEENS

Parents need to know that vaping can be detrimental to growing minds.

"There are many serious health effects, including coughing, wheezing, seizures, and vomiting. There is also a potential for severe lung injury," says Pat Aussem, associate vice president of consumer clinical content development for Partnership to End Addiction.

Truth Initiative — a public health organization devoted to preventing tobacco use and nicotine addiction — says nicotine can affect key brain receptors and make it easier for youths to become addicted. It can harm brain development, exacerbate depression and anxiety, and increase stress.

CONCERNS FOR TEENS WITH HEMOPHILIA

Vaping products containing nicotine and THC have been associated with a type of lung injury that has its own name: e-cigarette or vaping use associated lung injury, or EVALI.

"Given reports of diffuse alveolar hemorrhage [bleeding in the lungs], there is concern that people with bleeding disorders would be at higher risk for hemorrhage and have increased severity of bleeding," says Stephanie Prozora, M.D., pediatric medical director of the Yale Hemophilia Treatment Center. "Available data suggest vaping THC may specifically worsen bleeding risk."

BEWARE
Vaping may be even more dangerous for those with bleeding disorders.



"Available data suggest vaping THC may specifically worsen bleeding risk."

HOW TO ADDRESS THIS WITH YOUR TEEN

If you discover that your teen is vaping, find out why they vape and how it makes them feel. Are they bored? Suggest a new activity they might enjoy. Are they trying to self-medicate a mental health issue? Connect them with a therapist. Are they pressured to fit in with peers? Give them an out: If they are with someone who is vaping and they're uncomfortable, have them text you a code, and you can call right back with an excuse to pick them up.

Also, teach your teen about the health problems vaping can cause. Remind them of how this can interfere with what they want to do in life. Finally, if they are addicted, help them quit. Resources are available at Truth Initiative (truthinitiative.org), Smokefree.gov, and Partnership to End Addiction (drugfree.org).

—By Beth Levine



MORE TIPS TO HELP YOUR HOUSEHOLD: *HemAware* has plenty of resources for the health issues facing your family. Visit hemaware.org/life/family-matters

Signs Your Kid Is Vaping

The Partnership to End Addiction says to look for these clues:

- Smell. Many e-cigarettes add flavoring, which produces a light scent.
- Strange packages showing up for your teen. Check your credit card for unknown charges.
- Increased thirst, loss of appetite, nosebleeds, and headaches.
- Equipment in their room that you can't identify.
- Cutting back on caffeine; vaping causes sensitivity to the stimulant.
- Increased irritability and anxiety and difficulty concentrating.

Halting High Cholesterol

Nearly 94 million Americans have this risk factor for heart disease. Here's what people with bleeding disorders can do about it.

Jennifer DeGlopper found out she had high cholesterol — a risk factor for heart disease — in her 20s during a regular screening. She was active and maintained a healthy weight, but she also had a family history of heart disease to consider, plus she has von Willebrand disease and hemophilia B.

DeGlopper's father died of a heart attack at 58. "As I get closer to that age, I'm trying to be more proactive," the 56-year-old says.

And so, when she experienced chest pain, she saw a cardiologist. She discovered she still had high cholesterol levels, defined as more than 200 mg/dL (milligrams per deciliter). It was time for some changes.

DeGlopper and Barbara Konkle, M.D., a hematologist at the Washington Center for Bleeding Disorders in Seattle, offer this advice for managing high cholesterol with a bleeding disorder.



STICKY SITUATION

Too much bad cholesterol can clog artery walls, reducing blood flow and increasing heart disease risk.

“Everyone should be sure that their blood pressure and cholesterol are well controlled.”

START WITH LIFESTYLE CHANGES

Maintaining a healthy lifestyle is key to preventing and managing high cholesterol: Eat better, exercise more, stop smoking, and limit alcohol intake.

In particular, the Centers for Disease Control and Prevention recommends avoiding foods high in saturated fat, trans fat, and sodium.

DeGlopper lost 30 pounds on a plant-based diet, but her cholesterol was still too high. "I was always told to change your diet, lose a little weight, and it will go down — but it never did," she says.

Konkle reminds patients that these changes have considerable cardiovascular benefits, even if it doesn't lower cholesterol.

TAKE CHOLESTEROL-LOWERING STATINS

"When high cholesterol isn't controlled by diet, then medically the first step is a statin," Konkle says.

Statin medications block a cholesterol-producing enzyme to reduce low-density lipoprotein, or LDL, also known as the "bad" kind of cholesterol. Konkle says they're

extremely effective at not only decreasing cholesterol but also preventing heart attacks and strokes.

DeGlopper started taking cholesterol and blood pressure medications in 2021 with great success; her total cholesterol dropped from 263 to 160, in the healthy range for her age.

CONNECT BLEEDING DISORDERS AND HEART HEALTH

Studies have shown that people with hemophilia have a lower heart attack risk, Konkle explains. Others have found that men with hemophilia are more likely to have high blood pressure.

Konkle says there's a "very mild" bleeding risk with statins, but their effectiveness greatly outweighs the risk.

She recommends avoiding fish oil, garlic, and turmeric supplements, which carry an increased possibility of bleeding without proven benefits.

"Everyone should be sure that their blood pressure and cholesterol are well controlled," she says. "This is as important for individuals with bleeding disorders as it is for anyone else."

—By Celeste Sepessy



5 HABITS FOR BETTER HEART HEALTH: Getting screened for high cholesterol is one way to avoid heart disease. See other heart-healthy habits here: hemaware.org/mind-body/how-prevent-heart-disease

DEPRESSION CHECKLIST FOR PARENTS:

DIFFICULTY
SLEEPING, SUCH
AS SLEEPING
TOO MUCH OR TOO
LITTLE

PROBLEMS
CONCENTRATING

REGULAR
THOUGHTS
OF DEATH OR
SUICIDE

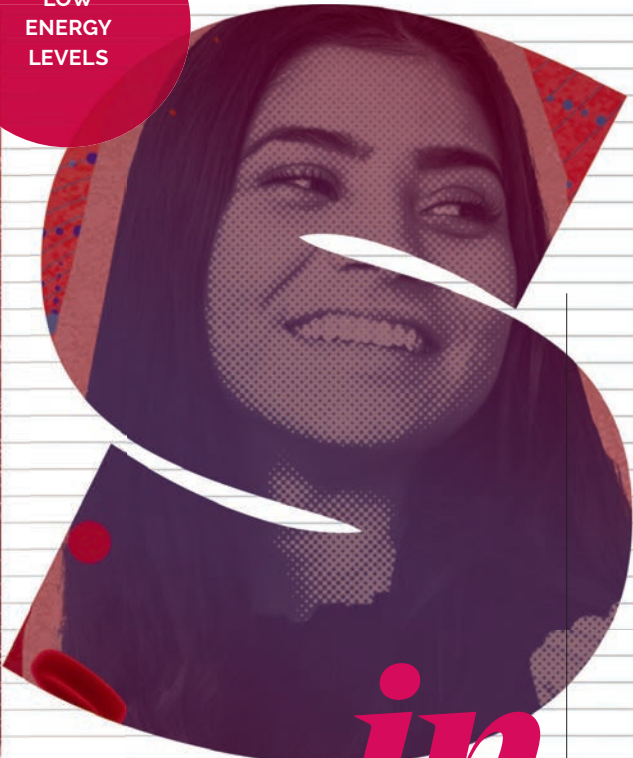
Adolescents in the blood disorders community who have mental health

Checklist source: National Institute of Mental Health



PROBLEMS
WITH EATING,
SUCH AS EATING
TOO MUCH OR
TOO LITTLE

LOW
ENERGY
LEVELS



in



FEELINGS OF
WORTHLESSNESS



THEIR DEPRESSIVE
EPISODES WEREN'T
CAUSED BY MEDICAL
ILLNESS, SUBSTANCE
USE DISORDERS, OR
MEDICATION



WRITTEN BY RITA COLORITO | ILLUSTRATIONS BY STEPHANIE DALTON COWAN

Health issues often face added barriers to serious behavioral health care.

DENIED CARE Teens (and adults) with bleeding disorders are sometimes barred from inpatient behavioral health care because they need factor infusions.



The last time Jennifer Feldman, RN, 20-year-old with severe hemophilia A, he asked for help to turn his 2021, and Derick was in the emergency room once again — not for because of the mental health and substance use disorders he had st



saw Derick,* a then life around. It was late March his bleeding disorder, but ruggled with since his teens.

“His last ER experience really scared him. He knew this was an uphill battle. The only difference was, this time he was actually ready to get the help he needed,” says Feldman, nurse coordinator for the New England Hemophilia Center at UMass Memorial Medical Center in Worcester, Massachusetts.

At the time, the inpatient psychiatric care beds at UMass Memorial, where Derick had been hospitalized before, were full. Feldman called other rehab facilities throughout central Massachusetts. The ones with openings refused to take Derick.

“I was told that it was not something that they could do because having a patient who required infusions or injections is a liability to them,” Feldman says. After Derick’s death, Feldman sounded the alarm to the National Hemophilia Foundation and other bleeding disorders organizations. Could other teens and adults with bleeding disorders be denied inpatient mental and behavioral health care because they need factor infusion?

Despite Feldman’s best efforts to get him round-the-clock care, by early August 2021 Derick had died from his substance use disorder. Having hemophilia had kept this “big teddy bear,” as Feldman described him, from getting the inpatient care that was his best hope for long-term recovery.

“Derick was discriminated against because of his underlying condition. I thought this can’t be an isolated incident. And sure enough, it certainly wasn’t,” Feldman says. (See sidebar, “Changing Access to Mental and Behavioral Health Care.”)

Like Feldman, Gillian Schultz, director of programs for the Bleeding Disorders Foundation of North Carolina, has faced roadblocks to inpatient care for her 11-year-old son, who has struggled with attention-deficit/hyperactivity disorder and a mood disorder since he was 5.

Two years ago, after an emergency hospital stay, doctors recommended that Schultz’s son receive inpatient care. Knowing such facilities are often fully booked, Schultz tapped her connections. Even when she could find an opening, her son’s hemophilia tripped an automatic denial. Her hemophilia treatment center (HTC) social worker and nurse made calls and wrote letters. “Nobody was able to help,” she says.

Schultz’s son was eventually placed in a weekday day program. “They would accept him there because he didn’t need infusions while he was there,” Schultz says. “But it was not an ideal situation because he needed a more intense level of care. And he fought with us when it was time to go each day.”

Day programs are a transitional step after someone reaches stability with inpatient care. Without inpatient care, it took her son nearly a year for his mental health to stabilize, says Schultz, who worries about what may happen as he gets older.

Schultz’s experience prompted her state bleeding disorders chapter to launch a mental health initiative. Its subsequent survey of people with bleeding disorders across North Carolina found that 37% of 11- to 19-year-olds deal with a mental health issue. Schultz, who develops programs for teens at her chapter, wasn’t surprised by the findings.

*HemAware is using Derick’s first name only, out of respect for patient confidentiality.

Changing Access to Mental and Behavioral Health Care



Concerned that people with bleeding disorders are being denied critical care for their mental health and substance use disorders, the National Hemophilia Foundation joined with the Hemophilia Federation of America, local bleeding disorders chapters, several hemophilia treatment center (HTC) nurses and social workers, and community members to form the Bleeding Disorders Substance Use and Mental Health Access Coalition. Gillian Schultz and Lucy Ramirez both serve on the coalition.



To get a handle on the problem, the coalition conducted a nationwide survey of HTC doctors, nurses, social workers, and staff. The results were sobering: 78% of those who had tried to secure placement for their patients at a mental health or substance use residential treatment facility had been denied care. They often came across outdated concepts of what it means for someone to have a bleeding disorder and concern over

infusion needles being used for substance use or self-harm.



"We found that it was an issue across the country," says Kate Reinhalter Bazinsky, MPH, the coalition's chair and a board member of the New England Hemophilia Association. "We had cases in 16 different states, and we had 28 different individual provider reports of this happening. And often the providers had multiple patients who had been denied care."



The American Society of Addiction Medicine (ASAM), which sets admission criteria for substance use treatment centers, is expected to release new guidelines in 2023. So, the coalition decided to tackle substance use care first, says Bazinsky, providing its own set of recommendations in hopes of addressing concerns and misinformation over admitting people with bleeding disorders. Because it's not an accrediting organization, ASAM can't force facilities to implement the guidelines, but many

states incorporate ASAM's criteria into their regulations, so it's an important step to get the organization to rethink its policies, Bazinsky says.



"Basically, we defined what medical stability means for a bleeding disorders patient. They are safe within an inpatient facility as long as they have the ability to infuse, they have access to their medications, and there's somebody to monitor them during the infusion," Ramirez says. To make sure its proposed recommendations to ASAM passed medical muster, the coalition submitted them to NHF's Medical and Scientific Advisory Council (MASAC), which drafted a letter to ASAM endorsing the group's work.



Through the survey, the coalition also learned what worked for providers who were able to get their patients into residential mental health and substance use facilities. The coalition developed these best practices into a resource guide, which providers received in August ↓

ICONS: RAMBO182/GETTY IMAGES



"ADVOCATING FOR YOUR CHILD'S MENTAL HEALTH NEEDS IS AS IMPORTANT AS ADVOCATING FOR CARE FOR THEIR BLEEDING DISORDER."

A NATIONWIDE EMERGENCY

derick's and Schultz's stories illustrate the escalating mental health crisis among the nation's children and adolescents — and the extra hurdles that people with bleeding disorders face when seeking care.

According to the Substance Abuse and Mental Health Services Administration (SAMHSA), 15.7% of adolescents ages 12 to 17 experienced a major depressive episode in 2019, up significantly from 8.1% in 2009.

Of these, 11.1% experienced a depressive episode so severe that it interfered with their school, home, and social lives. By comparison, 5.8% of teens in 2009 reported having major depression with such severe impairment.

The COVID-19 pandemic placed additional mental health burdens on children and teens. According to the Centers for Disease Control and Prevention, 37% of high school students in 2021 reported experiencing poor mental health during the pandemic. In the 12 months before the survey, 44% had felt persistently sad or hopeless.

Among her child and teen patients at Rush Hemophilia and Thrombophilia Center in Chicago, Lucy Ramirez, MSW, LCSW, has seen the mental health effects of living through the pandemic. "A lot of them were not happy about virtual learning. That was the main impact," she says. "Some of them seemed to give up on it. They became more depressed and more anxious about that aspect."

Missing out on milestones such as school dances and graduation also affected kids' mental well-being, Ramirez says. And even returning to school proved difficult for some. "They were kind of out of practice being with friends and socializing again," she says.

For children and teens who already had depression, lockdown and isolation measures compounded their symptoms, Ramirez says. "More of the adolescents that we work with were a bit more withdrawn socially," she says. "They were having a harder time communicating with their family members, and with their friends especially."

Ramirez also saw an uptick in teens and young adults not following their normal prophylaxis schedules. "They weren't doing anything or going anywhere, so they didn't feel the need to treat," she says. "But doing a deeper dive into what those emotions were, it was an overall neglect of self-care."

Like many who struggle with mental health and substance use disorders, Derick stopped taking care of his physical health, often missing his HTC appointments. A change in physical health, appearance, and hygiene are often signs of depression and other mental health issues, says Denise Lowery, LCSW, who works with teens at the UC Davis Hemostasis and Thrombosis Center in Sacramento, California.

CRISIS MODE

A 2021 CDC survey revealed that 37% of high school students reported experiencing poor mental health during the pandemic.



during NHF's Annual Bleeding Disorders Conference. "It gives providers a blueprint for what they should say and not say when talking with facilities to maximize their chances of getting residential care for their patients," Bazinsky says.



In the meantime, if you or your child needs residential mental health or substance use services, don't give up. Ramirez says. "We have had some patients who

have been successful getting treatment for substance use on their own, who made the calls to get into rehab. One adult was even able to bring his infusions to treat himself. So, it can be done," she says. "But we're trying to increase those success stories so that all people with bleeding disorders are able to get into treatment when they need it."



If you or your provider has been denied access to inpatient

mental health or substance use care, the Bleeding Disorders Substance Use and Mental Health Action Coalition wants to hear from you. Email bdsumhac@gmail.com, or contact Marla Feinstein, NHF's senior policy and healthcare analyst (mfeinstein@hemophilia.org or [212-328-3734](tel:212-328-3734)) or Mark Hobracczk, senior manager for policy and advocacy at the Hemophilia Federation of America (m.hobracczk@hemophiliafed.org or [813-965-2127](tel:813-965-2127)).

SAFEGUARDING YOUR CHILD'S MENTAL HEALTH

the sooner children receive treatment for a mental or behavioral health issue, the better their chances at managing the disorder and preventing it from getting worse. That's why it's important to recognize how widespread and interconnected these issues are for children and teens.

Like Schultz's son, mental and behavioral health issues can start well before the teen years. One in 6 U.S. children ages 2 to 8 has a diagnosed mental, behavioral, or developmental disorder, according to the CDC.

The latest data from the National Survey of Children's Health found that 22.6% of children ages 3 to 17 had at least one reported mental, behavioral, emotional, or developmental problem or needed a related screening. As they enter their tween and teen years, diagnoses of ADHD, depression, and anxiety increase, according to the CDC.

Before prophylaxis treatment became available, Lowery says her patients experienced more anxiety and depression related to their bleeding disorders.

To help kids and teens maintain their mental health, HTC social workers and nurses often recommend that parents encourage them to participate in programs offered through NHF, their local chapters, and other organizations. At the height of the pandemic, many of these events took place virtually. Bleeding disorders summer camps — which help kids and teens feel a sense of belonging — went remote.



TIMING MATTERS

The sooner teens receive mental health treatment, the better their chances of managing the disorder and preventing it from getting worse.

Schultz's son, who looks forward to summer camps every year, refused to go to virtual camp. She's seen a change in his attitude for the better since he could attend in person this year.

To get teens to connect during the pandemic, Lowery held a virtual teen group. "They were chatting, playing their video games, and I think it was really helpful for them to just talk about nothing, because they were just interacting," she says.

Advocating for your child's mental health needs is as important as advocating for care for their bleeding disorder, Lowery says. While she's seen an increase in anxiety and depression among children and teens, Lowery says that may be the result of feeling more comfortable talking about mental health issues now than in the past. When they don't talk about it, she says, children and teens struggle with these issues



longer than necessary, simply because it takes so long to get a diagnosis and then treatment.

For parents who are concerned about their child's mental health, Lowery recommends first reaching out to their HTC social workers and nurses.

And because mental health issues can strain parents and families, Schultz advises parents to focus on their own self-care. "Seeking community and support from others who are in similar situations — especially for parents — is really important, too," she says.

As was the case with Derick, substance use disorder often coexists with mental illness. According to SAMHSA, nearly 3% of adolescents ages 12 to 17 struggle with both major depression and substance use disorder (illicit drug or alcohol use or both).

Often, teens will use drugs or alcohol as a coping mechanism. Experiencing a major depressive episode increases the chances that teens will use illicit drugs. The most recent SAMHSA data from 2020 found that 28.6% of adolescents ages 12 to 17 who had a major depressive episode reported using illicit drugs within the past year, compared with 10.7% of those with no such episodes.

Feldman says she wishes she could have done more for Derick, who wanted to go back to college to study forensic science. He had attended college briefly in 2018 on an academic scholarship.

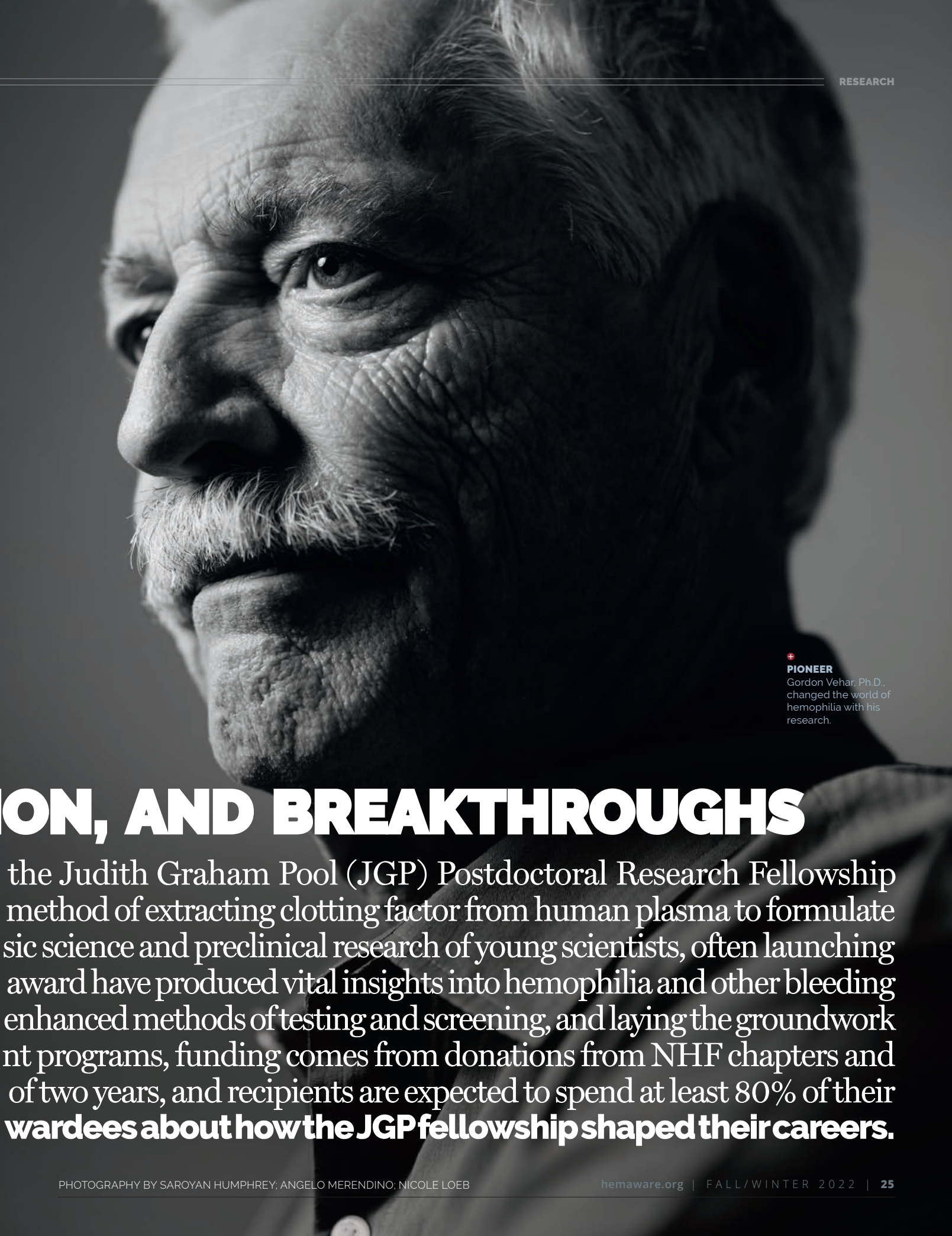
"He was just a really nice kid, super smart. He definitely could have gone places," Feldman says. "You just never think you'll run out of time."

50

YEARS OF INSIGHTS, INNOVATION

In 1972, the National Hemophilia Foundation (NHF) introduced the JGP fellowship program — named after the legendary researcher who discovered cryoprecipitate. Over the years, the JGP fellowship has funded the best of their careers. The projects of the 104 researchers who have received the award have contributed to the development of safer concentrates and for current advances in gene therapy for hemophilia. Unlike many grants, the award provides up to \$52,000 a year for a maximum of one year time on the funded research project.

We spoke with five a



PIONEER
Gordon Vehar, Ph.D.,
changed the world of
hemophilia with his
research.

ION, AND BREAKTHROUGHS

the Judith Graham Pool (JGP) Postdoctoral Research Fellowship method of extracting clotting factor from human plasma to formulate basic science and preclinical research of young scientists, often launching award have produced vital insights into hemophilia and other bleeding enhanced methods of testing and screening, and laying the groundwork nt programs, funding comes from donations from NHF chapters and of two years, and recipients are expected to spend at least 80% of their **wardees about how the JGP fellowship shaped their careers.**

“
**I KNEW HEMOPHILIA WAS A
 DISEASE OF COAGULATION, BUT IF
 YOU'RE NOT A DOCTOR TREATING
 PATIENTS, IT'S JUST COLD, HARD
 CHEMICALS AND TEST TUBES.**
 ”

GORDON VEHAR, PH.D.

1977-79

CLOTTING FACTOR PIONEER

Like many scientists, Gordon Vehar, Ph.D., got where he is today through a combination of hard work and happenstance. He was finishing his Ph.D. in biological chemistry from the University of Cincinnati College of Medicine when his Ph.D. supervisor, who grew up in Seattle, recommended he do his postdoctoral work at the University of Washington in a lab focused on coagulation. Not just any lab. The most preeminent lab in the field of hemophilia, one whose director, Earl Davie, Ph.D., was among the first in the world to propose the coagulation cascade and isolate factor XIII.

“I knew nothing about coagulation,” Vehar says. “Nothing about Seattle. It was totally circumstance.” Circumstance that eventually changed the lives of people with hemophilia.

Vehar’s postdoctoral work introduced him not only to the biochemistry of coagulation, but also to the real people affected by it — those with hemophilia. “I knew hemophilia was a disease of coagulation, but if you’re not a doctor treating patients, it’s just cold, hard chemicals and test tubes,” he says. To counter that, the lab’s director hired students with hemophilia to work there. “We got to meet them and see some of the joint damage that was going on and how it impacted them,” he says. “That made it personal.”

His mentor encouraged him to apply for the JGP fellowship. The topic: “Structure and Function of Factor VIII.” It would be the only grant he’d write in his career, and it would end up changing the world of hemophilia. Just nine years after winning the grant, Vehar led the scientific team at Genentech that cloned the gene for factor VIII, enabling the production of an artificial, genetically engineered clotting factor that transformed the lives of people with hemophilia.

Today, Vehar is vice president of external innovation at BioMarin Pharmaceutical Inc., in San Rafael, California, where he is responsible for identifying, evaluating, and overseeing all externally funded early research programs. His name is on more than 40 patents and dozens of scientific publications.



LEADER

Bin Zhang, Ph.D., runs a laboratory at Cleveland Clinic focused on early secretory pathway deficiencies in human diseases.



BIN ZHANG, PH.D.

2001-03

FIRST TIME'S THE CHARM

When Bin Zhang received his Ph.D. from Michigan State University, he wanted to study something directly relevant to human disease. So instead of finding a postdoctoral position in the field he was working in — spore formation in bacteria — he joined the lab of David Ginsberg, M.D., one of the country's premier researchers on blood clotting, at the University of Michigan in Ann Arbor and entered the world of bleeding disorders.

Zhang quickly latched onto a project, which involved finding the gene that causes a rare bleeding disorder called combined deficiency of factor V and factor VIII. When Ginsberg told him about the JGP fellowship, Zhang knew his work was well within the scope of the award. He was also aware of the prestigiousness of the fellowship and what it could mean for his future. So, he put together his first-ever grant proposal and won.

"I was excited to receive the award," he says. "It was an encouragement and recognition of the significance of my work by experts in the field." The fact that he could write a grant and get the award "told me that I have potential, that I could go on with an academic career."

The fellowship was the first domino to fall. Thanks to it, he won NHF's three-year Career Development Award and funding from the March of Dimes. When it was time to apply for a job, he received several offers and picked the Lerner Research Institute at Cleveland Clinic, where he runs his own laboratory focused on early secretory pathway deficiencies in human diseases, the same thing responsible for combined deficiency of FV and FVIII.

He's also paying forward his good fortune. Last year, two of his postdoctoral Ph.D.s applied for and won the JGP fellowship. "The award is very important in supporting young scientists," he says.

“

**THE AWARD WAS AN
ENCOURAGEMENT AND
RECOGNITION OF THE
SIGNIFICANCE OF MY WORK.**

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LACRAMIOARA IVANCIU, PH.D.

2010–12

ESSENTIAL SUPPORT EARLY IN A CAREER

Lacramioara Ivanciu, Ph.D., received the Judith Graham Pool fellowship soon after starting her post-doctoral training at Children’s Hospital of Philadelphia in the lab of Rodney Camire, Ph.D., a pioneer in the basic science of coagulation.

She was thrilled to learn of her selection for the award. “It was a starting point for my academic career in basic and translational research in bleeding disorders,” she says. Her goal was to generate recombinant coagulation for factor X variants and evaluate their therapeutic potential for the treatment of hemophilia in acute situations. “This work was significant for the preclinical development of FX variants as bypass agents for treatment of hemophilia A and B,” she says. Ivanciu published the findings from her fellowship work in the journal *Nature Biotechnology*.

Obtaining the fellowship provided her with the necessary support to acquire skills and knowledge related to her career development. “That had a major contribution to my faculty appointment,” she says. It helped her get other grants as well, and today she is starting her own laboratory at the Perelman School of Medicine at the University of Pennsylvania and Children’s Hospital of Philadelphia, focusing on explaining the regulation of clot formation in vivo.

“The National Hemophilia Foundation and the JGP and their other grants provide excellent support for early career investigators,” she says. “They also provide opportunities for therapeutic development that will have a substantial effect on the quality of life of people living with bleeding disorders.”

Her advice to young scientists is to go for it. “Apply for these grants, because they can really make an impact on your career,” she says. “Trust in yourself. Do what makes you passionate.”

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**THE NATIONAL HEMOPHILIA
FOUNDATION AND THE JGP
AND THEIR OTHER GRANTS
PROVIDE EXCELLENT
SUPPORT FOR EARLY CAREER
INVESTIGATORS.**

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CATALYST

Lacramioara Ivanciu, Ph.D., says receiving the award was a starting point for her academic career.



+

COMMUNITY

Andrew Yee, Ph.D., says an added benefit of the JGP award has been working with other JGP awardees.



Honor Roll: Judith Graham Pool Award Recipients

Since 1972, 104 researchers have received the National Hemophilia Foundation's Judith Graham Pool Postdoctoral Research Fellowship to fund their research of hemophilia and other bleeding disorders.

- 1972-73:**
Andrew Weiss, M.D., University of North Carolina at Chapel Hill
- 1972-74:**
Judith Anderson, M.D., Duke University School of Medicine, Durham, North Carolina
- 1973-75:**
Margaret Rick, M.D., University of Connecticut, Farmington
- 1974-76:**
Eric Jaffee, M.D., Cornell Medical School, New York
- 1974-76:**
Charles Heldebrant, Ph.D., University of Washington, Seattle
- 1975-77:**
James Brown, Ph.D., University of California, San Diego
- 1975-77:**
John Lazarchick, M.D., University of Connecticut Health Center, Farmington
- 1976-77:**
Myatt Lipscomb Jr., Ph.D., Temple University, Philadelphia
- 1977-78:**
Mary Ellen Switzer, M.D., Duke University, Durham, North Carolina
- 1977-78:**
Annita Weidenbach, Ph.D., Oklahoma Medical Research Foundation, Oklahoma City
- 1977-79:**
Jonathan Goldsmith, M.D., University of North Carolina at Chapel Hill
- 1977-79:**
Gordon Vehar, Ph.D., University of Washington, Seattle
- 1979-80:**
David McCarroll, Ph.D., University of Tennessee, Knoxville
- 1979-80:**
Michael Chopek, M.D., University of Washington, Seattle
- 1979-80:**
Frederick Ernst, Ph.D., Wright State University, Dayton, Ohio
- 1982-83:**
Karen Kolba, M.D., University of Arizona Health Sciences Center, Tucson, Arizona
- 1982-84:**
James Bussell, M.D., Cornell University Medical College, New York
- 1983-84:**
Carolyn Beach Daul, Ph.D., M.D., Tulane University School of Medicine, New Orleans
- 1983-85:**
Peggy Weinrub, M.D., University of California at San Francisco
- 1984-85:**
Gordon Lewis Bray, M.D., Children's Orthopedic Hospital and Medical Center, Seattle
- 1985-86:**
Bruce Harold Odegaard, Ph.D., University of Vermont College of Medicine, Burlington
- 1985-87:**
Barbara Schach, M.D., University of Washington, Seattle
- 1986-87:**
Michael Katzman, M.D., Case Western Reserve University, Cleveland
- 1986-88:**
Paula Silverman, M.D., University Hospitals of Cleveland
- 1986-88:**
Nancy Collins O'Brien, M.D., University of Texas System Cancer Center, Houston
- 1987-88:**
Wadie Bahou, M.D., University of Michigan, Ann Arbor
- 1988-89:**
Monica Traystman, Ph.D., Johns Hopkins University School of Medicine, Baltimore
- 1988-89:**
James Evans, M.D., University of North Carolina at Chapel Hill
- 1989-90:**
Jack Rayner, DVM, Blood Center of Southeastern Wisconsin, Milwaukee
- 1989-91:**
Josephine Carew, Ph.D., Dana-Farber Cancer Institute, Boston
- 1990-91:**
Jay Nelson Lozier, M.D., Ph.D., University of North Carolina at Chapel Hill
- 1991-92:**
Theo Palmer, Ph.D., Fred Hutchinson Cancer Research Center, Seattle
- 1991-93:**
Athan Kuliopulos, M.D., Ph.D., Harvard Medical School, Boston
- 1991-93:**
Frederick Askari, M.D., Ph.D., University of Michigan Medical Center, Ann Arbor
- 1991-93:**
William Nichols, Ph.D., University of Michigan Medical Center, Ann Arbor
- 1992-94:**
Sylvia Singer, M.D., Children's Hospital Oakland, Oakland, California
- 1993-95:**
Dwight D. Koeberl, M.D., Ph.D., University of Washington, Seattle
- 1994-95:**
Qicheng Yang, Ph.D., Salk Institute, San Diego
- 1994-96:**
Scott Tenenbaum, Ph.D., Tulane University School of Medicine, New Orleans
- 1995-97:**
David Bruce Schowalter, M.D., Ph.D., University of Washington, Seattle
- 1996-98:**
Degang Zhong, Ph.D., American Red Cross Holland Laboratory, Rockville, Maryland
- 1996-98:**
Dewey McCafferty, Ph.D., Harvard Medical School, Boston
- 1997-99:**
Beth Bouchard, Ph.D., New England Medical Center, Boston
- 1997-99:**
Steven Shahin Fakhrazadeh, M.D., Ph.D., University of Pennsylvania, Philadelphia
- 1998-2000:**
Paul Edward Monahan, M.D., University of North Carolina at Chapel Hill
- 1998-00:**
Jonathan Bernard Rosenberg, Ph.D., Blood Center of Southeastern Wisconsin, Milwaukee
- 1999-2000:**
Bing-guan Chen, M.D., Ph.D., University of Michigan, Ann Arbor

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**IT'S AN INCREDIBLE
HONOR TO BE A JGP
AWARD RECIPIENT.**

”

ANDREW YEE, PH.D.

2009-11

A HAPPY CHANGE IN DIRECTION

Andrew Yee, Ph.D., thought he wanted to be a chemical engineer. At least, that's what he received his Ph.D. in. Then he spent some time in a bioengineering lab looking at endothelial cells and, suddenly, “I wanted to learn about biology and genetics,” he says. “I wanted to work with animals.” This led him to a postdoctoral position with David Ginsburg, M.D., at the University of Michigan.

Yee immediately launched into a project to identify all of the genetic variants in von Willebrand factor (VWF) that caused bleeding disorders. Then Ginsburg told him about the JGP fellowship. He read about Pool's discovery, which piqued his interest in factor VIII. Specifically, he wondered, how did it interact with VWF?

So, he applied for the JGP fellowship with a research proposal called “Fine Structure-Function Mapping VWF-FVIII Interaction.” It was one of the first grants he'd ever applied for and the first he won.

“I was elated when I got the news that I won,” says Yee, who is now an assistant professor in the Department of Pediatrics, Division of Hematology-Oncology at Baylor College of Medicine in Houston. Not just because he now had funding to pursue his idea for two years, but because of the input he received from the reviewers. “Their suggestions made quite an impact on how I pursued the experiments,” he says.

Yee used his JGP award to develop reagents required for the complex studies in animal models he conducted to explore VWF and FVIII interactions, work that continues today. An added benefit of the award was being able to work with other JGP awardees. “We're a small community,” he says. “It's an incredible honor to be a JGP award recipient.”



PIVOTAL

Sol Schulman, M.D., Ph.D., says the JGP fellowship has had a major impact on where and who he is today.

SOL SCHULMAN, M.D., PH.D.

2016-18

PERFECT TIMING

Although he completed his medical degree and clinical fellowship in hematology/oncology, Sol Schulman, M.D., Ph.D., says it's the research that "gets me up in the morning." So in 2015 while completing his hematology/oncology fellowship, he began looking for independent funding to pursue his research interests, which is when he heard about the JGP grant from a previous awardee. "He'd had a positive experience and highly recommended it," Schulman says. "It seemed like a perfect fit for my interest."

"A lot of times when you're applying for these types of awards, people are trying to fit a square peg into a round hole," he says. "But I felt, wow, this is the right award for the right stage of my training."

Schulman says the JGP fellowship, which funded his project looking at the role of protein disulfide isomerase in activating clotting, was critical to where and who he is today.

"Most people interested in pursuing research careers in medicine find themselves in a transition around the end of clinical fellowship; suddenly there's this great abyss out there," he says. He knew he could focus on clinical medicine and have the security of a salary and academic position. But he also knew the pressures of full-time clinical work would leave little time for the research he yearned to do. The JGP grant, he says, convinced him he could become an independent researcher and bring in his own money.

"It not only provided me with protected time to do the research I was excited about and gain mentors, but it was also a validation at the national level that I was doing something interesting and important," he says.

Today, Schulman is a principal investigator in the Division of Hemostasis and Thrombosis at Beth Israel Deaconess Medical Center in Boston. His lab works to integrate functional genomics, human genetics, biochemistry, and cell biology to identify new genes and pathways that influence blood coagulation.

“

THE AWARD WAS VALIDATION AT THE NATIONAL LEVEL THAT I WAS DOING SOMETHING INTERESTING

AND IMPORTANT.

”



1999-2001:

Jo Chao Tsai, Ph.D., Beth Israel Deaconess Medical Center/East, Boston

1999-2001:

You-Kyung Hwang, Ph.D., Emory University, Atlanta

1999-2001:

Frank Park, Ph.D., Stanford University, Stanford, California

1999-2001:

Mark Thomas Reding, M.D., University of Minnesota, Minneapolis

2000-02:

Cathryn Sanghae Mah, Ph.D., University of Florida, Gainesville

2000-02:

Yubin Kang, M.D., University of Iowa College of Medicine, Iowa City

2000-02:

Lingfei Xu, M.D., Ph.D., Washington University School of Medicine, St. Louis

2001-03:

Anja Ehrhardt, Ph.D., Stanford University, Stanford, California

2001-03:

Bin Zhang, Ph.D., University of Michigan Medical School, Ann Arbor

2001-03:

Peter Vincent Jenkins, Ph.D., University of Rochester Medical School, Rochester, New York

2002-04:

Andrey Garunovich Sarafanov, Ph.D., American Red Cross Holland Laboratory, Rockville, Maryland

2002-04:

Lisa Webb, Ph.D., Jackson Laboratory, Bar Harbor, Maine

2002-04:

Hongmin Sun, Ph.D., University of Michigan Medical School, Ann Arbor

2002-04:

Julie Fradette, Ph.D., University of Pittsburgh, Pittsburgh

2003-05:

Ting-Chang Hsu, Ph.D., Puget Sound Blood Center, Seattle

2004-05:

Christine Kempton, M.D., University of North Carolina at Chapel Hill

2004-05:

Tao Li, Ph.D., University of North Carolina at Chapel Hill

2004-06:

Jill Marie Johnson, M.D., University of Michigan, Ann Arbor

2004-06:

Zhijian Wu, Ph.D., University of North Carolina at Chapel Hill

2005-07:

Michael Herbert, Ph.D., Stanford University, Stanford, California

2005-07:

Li Liu, M.D., Ph.D., University of Florida, Gainesville

2005-07:

David Buchner, Ph.D., University of Michigan, Ann Arbor

2007-09:

Ruijin Su, M.D., Ph.D., Puget Sound Blood Center, Seattle

2007-09:

Jyoti Mathur, Ph.D., Stanford University School of Medicine, Stanford, California

2007-09:

Jun-Jiang Sun, M.D., University of North Carolina at Chapel Hill

2008-10:

Mettine Bos, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2009-11:

Andrew Yee, Ph.D., University of Michigan Life Sciences Institute, Ann Arbor, Michigan

2010-12:

Lacramioara Ivanciu, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2011-13:

Yingyu Chen, Ph.D., Blood Research Institute, Milwaukee

2011-13:

Brian Ingram, Ph.D., University of North Carolina at Chapel Hill

2014-15:

Ji Yoon Noh, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2014-15:

Elizabeth Chappell, Ph.D., University of North Carolina at Chapel Hill, Raleigh

2014-15:

Wei Cheng, Ph.D., Washington University School of Medicine, St. Louis

2014-16:

Hongxia Fu, Ph.D., Boston Children's Hospital/Harvard Medical School, Boston

2014-16:

Sudharsan Parthasarathy, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2014-16:

Colin Kretz, Ph.D., University of Michigan Life Sciences Institute, Ann Arbor

2015-17:

Laura Sommerville, Ph.D., Duke University Medical Center, Durham, North Carolina

2015-17:

Christopher Ng, M.D., University of Colorado Denver

2016-17:

Tine L. Wyseure, Ph.D., Scripps Research Institute, San Diego

2016-18:

Shekhar Kumar, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2016-18:

Sol Schulman, MS, M.D., Ph.D., Beth Israel Deaconess Medical Center/Harvard Medical School, Boston

2016-18:

Klaus Bonazza, Ph.D., Boston Children's Hospital/Harvard University, Boston

2016-18:

Megan Rost, Ph.D., University of Michigan, Ann Arbor

2017-19:

Satish Nandakumar, Ph.D., Boston Children's Hospital, Boston

2017-19:

Kasturi Pal, Ph.D., Scripps Research Institute, San Diego

2017-19:

Esther Cooke, Ph.D., University of California San Diego, San Diego

2018-20:

Laura Haynes, Ph.D., University of Michigan, Ann Arbor

2018-20:

Kari Lavik, Ph.D., University of Michigan, Ann Arbor

2019-21:

Calvin Stephens, Ph.D., Stanford University, Stanford, California

2019-21:

Seema Patel, Ph.D., Emory University, Atlanta

2019-21:

Raghu Nath Azhwar, Ph.D., University of Michigan, Ann Arbor

2020-22:

Xuejie Chen, Ph.D., University of North Carolina at Chapel Hill

2020-22:

Kaushik Das, Ph.D., University of Texas Health Science Center, Tyler

2020-22:

Jhansi Magisetty, Ph.D., University of Texas Health Science Center, Tyler

2021-23:

Sean Quinn, Ph.D., Children's Hospital of Philadelphia, Pennsylvania

2021-23:

Vishal Srivastava, Ph.D., Cleveland Clinic

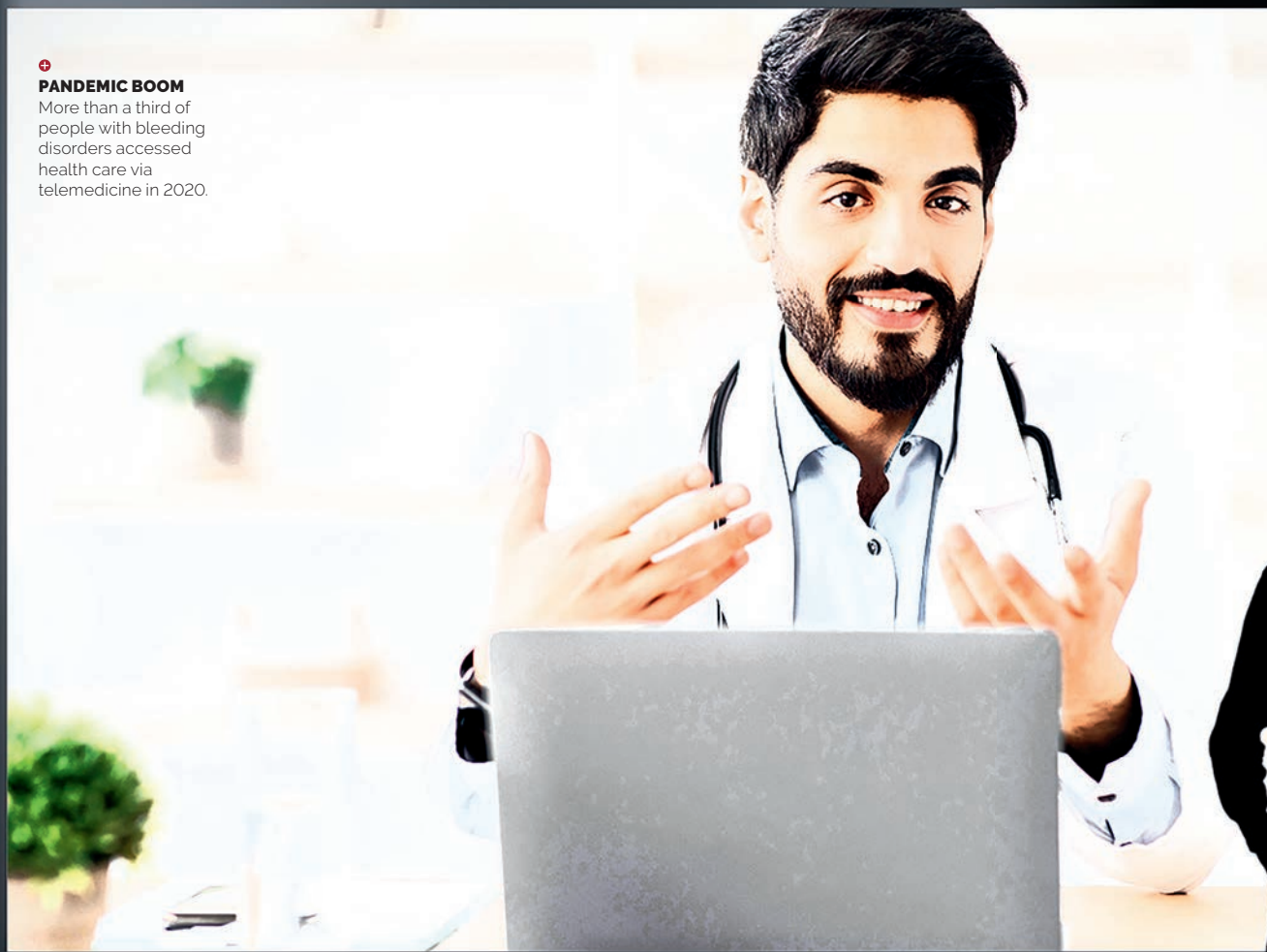
2021-23:

Yuan Zhang, Ph.D., Cleveland Clinic



PANDEMIC BOOM

More than a third of people with bleeding disorders accessed health care via telemedicine in 2020.



Telemedicine provided vital accessibility to bleeding disorders care



WRITTEN BY SHELLEY FLANNERY
ILLUSTRATIONS BY JOHN B. HANSEN

SCREEN TIME

during the pandemic. What does the future hold for this technology?

Remote work. Remote learning. Remote meetups with friends.

NEXT-GEN TELEMEDICINE

The future of telemedicine lies in remote patient monitoring, or RPM, which is the use of technology to monitor specific aspects of a patient's health outside of the provider's office.

A patient may be asked to take daily blood pressure measurements, for example, and then share the results with the provider. With RPM, the person can use a connected blood pressure cuff that automatically syncs to his or her electronic medical record, eliminating the need to regularly email readings to the office or log readings in a journal for reporting at the next visit.

"Remote patient monitoring allows us to keep closer tabs on our patients from the comfort of their homes," says Michelle Witkop, vice president of research strategy at the National Hemophilia Foundation.

Witkop says an RPM device to test for blood factor levels with a finger prick is on the horizon and could be a "game-changer" for people with hemophilia.

Americans have gotten much more comfortable conducting critical (and non-critical) tasks virtually since the start of the COVID-19 pandemic. And that includes medical care.

"During the first lockdown in 2020, the only way you could get care, unless you were very sick, was by telemedicine," says Joseph Kvedar, M.D., immediate past chair of the board of the American Telemedicine Association. "And so there was a huge increase in utilization in just a few months."

Use among the bleeding disorders community was higher than national averages: More than a third of people with bleeding disorders accessed health care via telemedicine in 2020, according to that year's National Hemophilia Treatment Center Patient Satisfaction Survey, compared with a quarter of all Americans.

TELEMEDICINE ADVANCEMENTS

Prior to the pandemic, most telemedicine visits — in which health care services are provided remotely via telecommunications — took place from a provider's office or hospital room to connect with another provider, such as a specialist, who was not on-site. With the proliferation of mobile devices, telemedicine visits between a patient and a single provider became more common. But it wasn't until the pandemic that telemedicine, also called telehealth, really took off.

In response to the sudden, massive need for virtual health care, the federal Coronavirus Aid, Relief, and Economic Security (CARES) Act earmarked \$200 million to establish the COVID-19 Telehealth Program. The program was designed to get more providers to offer telemedicine visits so people could stay home while still getting the care they needed.



The policies also temporarily loosened regulations. Providers could now conduct telemedicine visits with patients regardless of their geography, be reimbursed for telemedicine visits at the same rate as office visits, and use popular videoconferencing platforms without violating privacy laws.

These regulatory changes are set to expire soon after the COVID-19 public health emergency declaration ends, so it's possible this great progress in telemedicine could be erased in the near future.

"We really don't know what will happen yet," Kvedar says, "but we certainly hope we don't lose any ground, given how useful telemedicine has proved to be."

VIRTUALLY ENDLESS BENEFITS

Although many people were introduced to telemedicine as a means to maintain



MANY BENEFITS
 For people with bleeding disorders, telemedicine provides easier access to specialized treatment and also can prevent unnecessary ER visits.

a bleed was an emergency,” Kulkarni says. “Now you can see them live. You can say, ‘OK, move your wrist’ or ‘Let me see how you walk.’ To me, that’s the biggest advantage of telemedicine.”

Telemedicine allows for more comprehensive care. Patients and providers can easily connect with specialists across the country and get second opinions when necessary. Also, “if a patient is in their primary care provider’s office having a telehealth visit with the HTC specialist, that makes the primary care provider part of that team,” Witkop says. “That allows them to understand better what’s involved in hemophilia care and gives them a connection with the HTC.”

TELEMEDICINE IN PERSPECTIVE
 For all of its upsides, telemedicine does have its drawbacks, chief among them being the lack of personal connection.

“You just have more well-rounded visits in person,” Witkop says. “You really get more of a sense of a person’s well-being when you’re face to face.” In-person visits also give providers the opportunity to palpate (feel) the abdomen and joints to check for inflammation and other abnormalities.

As such, telemedicine should not replace in-person visits, particularly with comprehensive follow-ups, when patients meet with their entire bleeding disorders team, including their physician, nurse, physical therapist, and dietitian.

“Telemedicine should be viewed as complementary, not a replacement, for in-person care,” Witkop adds. “Patients should be seen in person at least every year or year and a half, depending on what their HTC recommends.”

physical distancing and avoid the spread of COVID-19, patients and providers benefit in numerous other ways.

Telemedicine makes specialized treatment accessible. There are 149 hemophilia treatment centers (HTCs) in the U.S., but many people still have to travel great distances to reach one. Roshni Kulkarni, M.D., a pediatric hematologist/oncologist at Michigan State University’s Center for Bleeding and Clotting Disorders in Lansing, Michigan, treats patients around the state, some of whom drive eight hours for visits. Yet it’s not only people in remote areas who benefit from telemedicine. “Patients in urban areas, such as New York, have similar challenges,” Kulkarni says. “Your HTC may be just across town, but it might take you an hour or more to get there, depending on time of day. That’s a lot of missed work.”

Telemedicine is cost-efficient. “Certainly, when we saw the price of gas going up this summer, that was a burden for people having to travel to an HTC,” says Michelle Witkop, a nurse practitioner and the vice president of research strategy at the National Hemophilia Foundation.

Telemedicine saves people from unnecessary visits to the ER. When evaluating a laceration or hematoma virtually, a provider can get a good sense of whether the situation necessitates emergency care or can be treated at home with clotting factor. “Previously, we had to rely on a patient’s description or a photograph to determine if



WHAT YOU SHOULD KNOW ABOUT TELEHEALTH APPOINTMENTS: Get the most out of your next virtual provider visit by following these tips: hemaware.org/life/what-parents-should-know-about-telehealth-appointments



Awards of Excellence: 2022 Ho

NHF recognizes those who have made significant contributions to the inheritable blood and b



PHILANTHROPIST OF THE YEAR

Jim Christensen, Omaha, NE

Jim Christensen, who lives with hemophilia A, has revolutionized the affordability of bleeding disorders treatment options by introducing the inSOURCERx prescription drug card. His work has significantly lowered the cost of treatment for millions of people and directed the card's profits to organizations including NHF.



RYAN WHITE YOUTH AWARD

Mikey White, Baldwinsville, NY

At 15 years old, Mikey White has become not only a phenomenal high school athlete who refuses to let hemophilia B slow him down, but also an extraordinary role model for people of all ages — including his younger brother, Westin, who also has hemophilia B.



ADVOCATE OF THE YEAR

Briana Reinking, Centennial, CO

A mother of two children with hemophilia, Briana Reinking has propelled the voice of her local chapter by helping to pass legislation that would inhibit step therapy and by empowering those around her to be advocates for the inheritable blood and bleeding disorders community.



LORAS GOEDKEN OUTSTANDING LEADERSHIP AWARD

Walter Justus, Boise, ID

Through his service on his chapter's board, his advocacy leadership, plus hours put in at summer camps and mentoring others, Walter Justus has helped NHF's Idaho chapter grow into the powerhouse it is today.



CHAPTER VOLUNTEER OF THE YEAR

Mariel Laureano, Guaynabo, Puerto Rico

Mariel Laureano of the Asociación Puertorriqueña de Hemofilia y Condiciones de Sangrado has created unity and progress toward a better life for the inheritable blood and bleeding disorders community. As team captain for the 2022 Unite Walk in Puerto Rico and a guest on a local radio show, Laureano has made tremendous strides in educating people on bleeding disorders.



RESEARCHER OF THE YEAR

Keith Hoots, M.D., Bethesda, MD

Recently retired as the director of the Division of Blood Diseases and Resources at the National Heart, Lung, and Blood Institute, Keith Hoots helped pioneer the creation of longitudinal follow-up of hemophilia cohorts, gene therapy trials, and clinical trials of new clotting concentrates.



PHYSICIAN OF THE YEAR

Kimo Stine, M.D., Little Rock, AR

For decades, Kimo Stine has been a champion of well-being and health progress for generations of families with hemophilia. His effect on personal care and encouragement of trial participation means everything to the people he serves.



SOCIAL WORKER OF THE YEAR

Connie Thibodeaux, New Orleans, LA

Social work is a key part of bleeding disorders care, and Connie Thibodeaux goes above and beyond to help fellow social workers better serve people in the bleeding disorders community. Thanks to her, Thibodeaux's community has access to educational resources on bleeding disorders.



norees

leading disorders community



GENETIC COUNSELOR OF THE YEAR

Jamie McCreery, Milwaukee, WI

Jamie McCreery has opened the world of genetic counseling to people across the country, assisting those around her in understanding testing options, responsible test utilization, and the role genetics plays in hemophilia, von Willebrand disease, and other rare bleeding disorders. She is set apart by her expertise and remarkably patient-centric approach.



PHYSICAL THERAPIST OF THE YEAR

Anita Wood, PT, New Orleans, LA

Anita Wood uses her position as a physical therapist to uplift and empower her patients, often accompanying them during orthopedic appointments, and to educate fellow PTs on treating those with inheritable blood and bleeding disorders.



NURSE OF THE YEAR

Brenda Nielsen, BSN, RN, Chapel Hill, NC

Brenda Nielsen's 30 years of service to the bleeding disorders community have been marked by a wide and inimitable range of accomplishments, including being the nurse coordinator at the University of North Carolina Hemophilia and Thrombosis Center, founding the first camp for people with bleeding disorders in North Carolina, and spearheading the passage of legislation that established an advisory council on rare diseases.



MARY M. GOOLEY HUMANITARIAN OF THE YEAR

Esmeralda Vazquez, Chicago, IL

Within the inheritable blood and bleeding disorders community, Esmeralda Vazquez's name is synonymous with health equity and patient-centric care. Through her championing of subject matter expert voices and her work embedding patient input within the research process, Vazquez has dedicated her life to ensuring that everyone is heard.



LIFETIME ACHIEVEMENT AWARD

Randy Curtis

Walnut Creek, CA

THROUGHOUT HIS CAREER IN PUBLIC HEALTH AND INDUSTRIAL RELATIONS, RANDY CURTIS HAS WORKED TIRELESSLY FOR A MULTITUDE OF ORGANIZATIONS HELPING THE INHERITABLE BLOOD AND BLEEDING DISORDERS COMMUNITY.

JOHN INDENCE AWARD

Hemophilia of Michigan, Ypsilanti, MI

During Bleeding Disorders Awareness Month in March, the Hemophilia Foundation of Michigan created educational programming and spearheaded outstanding political advocacy on behalf of the inheritable blood and bleeding disorders community, resulting in 26 legislative meetings with state representatives and greater community awareness in Michigan.

VAL BIAS AND TODD SMITH INNOVATION IN CAMP AWARD

Camp Bold Eagle, Muskegon, MI

Every summer, including during the height of the COVID-19 pandemic, Hemophilia of Michigan's Camp Bold Eagle serves as a haven of fun, connection, and education for children ages 6 to 13 who have hereditary bleeding disorders. The camp provides an exceptional opportunity for kids with bleeding disorders to connect with and learn about their community.

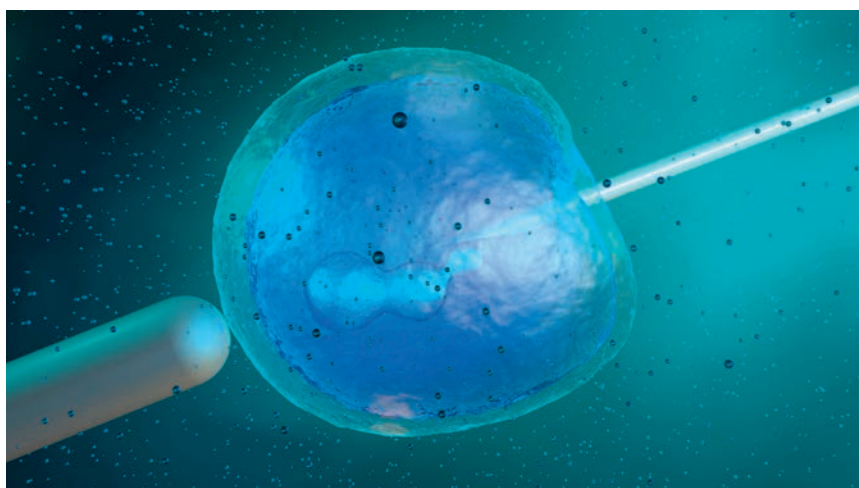
ZIGGY DOUGLAS INNOVATION IN YOUTH PROGRAMMING AWARD

Pacific Northwest Bleeding Disorders, Corvallis, OR

With the help of the Hemophilia Center at Oregon Health & Science University, Pacific Northwest Bleeding Disorders introduced the PEAK Teen Program and the YETI Conference, both of which have encouraged connection and agency among young bleeding disorders community members and have become leading standards in youth bleeding disorders programming across the country.

Introducing the WFH Gene Therapy Registry

New registry will enhance knowledge of gene therapy and keep patients safe



GLOBAL DATA

Pooling information from all countries will ensure that all adverse events are detected.

The World Federation of Hemophilia (WFH) is a nonprofit organization dedicated to improving and sustaining care for people with inherited bleeding disorders around the world. Over the next few years, several new therapies will revolutionize the treatment landscape, leading to new opportunities, but also potentially greater disparities. We have a responsibility to promote research and to advocate for more equitable access to novel therapies. The WFH Gene Therapy Registry (GTR) is a global initiative aimed at collecting long-term data on people with hemophilia who receive gene therapy. These data will enhance our understanding of gene therapy and ensure the hemophilia community has access to timely evidence on the long-term safety and efficacy of gene therapy.

As the likely first approval of a gene therapy product for hemophilia approaches, it is imperative that we consider the long-term impacts on patients. The number of people with hemophilia who receive gene therapy may be limited at first — and dispersed over many countries — making it a challenge to identify outcome patterns and compare individual patient outcomes in a meaningful way. By pooling data from all countries, the WFH GTR will make it easier to perform robust evaluation of safety events and establish the durability of gene therapy. In this way, the registry is the best way to ensure that rare adverse events are detected, even in smaller and geographically dispersed populations.

“Gene therapy has the potential to change the lives of many people with hemophilia,” explains Alain Baumann, WFH CEO. “The WFH GTR is an

efficient and powerful way to help gather the data that researchers and clinicians will need to monitor the efficacy and safety of gene therapy globally.”

The WFH GTR is an online system that collects uniform and standardized data on all patients who receive gene therapy, either via a clinical trial or via a gene therapy product post-approval. The core dataset was developed by a multistakeholder steering committee and is available online. Data will be directly inputted by centers participating with the GTR, and indirectly added through our Gene Therapy Data Integration Program, which allows existing registries to link with the WFH GTR, thus eliminating data entry duplication.

Patient safety is a shared responsibility. All people with hemophilia who have received, or who will receive, gene therapy in the future can participate in the WFH GTR regardless of where they are in their treatment process. The WFH looks forward to working with the HTCs and national hemophilia registries that will take part in this global initiative.

A Collaborative Effort

The WFH GTR was developed in collaboration with the American Thrombosis and Hemostasis Network, the European Association for Haemophilia and Allied Disorders, the European Haemophilia Consortium, the International Society on Thrombosis and Haemostasis, the National Hemophilia Foundation, and industry gene

therapy development partners and regulatory liaisons. The WFH GTR is supported by exclusive funding from the following generous sponsors:

Founding visionary partners
BioMarin
Pharmaceutical Inc.
CSL Behring
Pfizer
Spark Therapeutics

Collaborating partner
Takeda



LEARN MORE: Visit wfh.org/data-collection/#gtr.

A Fun Field Day with a Cause

With the help of their parents and grandparents, Steven and Adam Kopenitz are getting an early start on their philanthropic endeavors.

When Scott and Emily Kopenitz signed up their 5-year-old son, Steven, who has severe hemophilia A, for day camp this past summer, the administrators shared the schedule with them.

“One of the first things they asked us was, ‘What can he not do?’” Scott says. “We looked through the list, which included a golf camp and a boat trip on a lake and a bunch of other activities, and he could do all of it.”

That was welcome news to Scott and Emily, who say they are determined to give Steven and his 2-year-old brother, Adam, who also has severe hemophilia A, as normal a life as possible. “Yes, they

both have a bleeding disorder, but that shouldn’t hold them back,” Emily adds. “We want them to be able to live a full and active life with no limitations.”

That “no limitations” mindset inspired the Odessa, Texas, couple to think of ways that they and their sons could get more involved in their local community and help spread awareness that people with blood disorders can play and live like anyone else.

Scott and Emily approached the National Hemophilia Foundation (NHF) about creating a field day event that would bring together children and adults — with and without bleeding

“We want them to be able to live a full and active life with no limitations.”

disorders — for a day of ring toss, relay races, and other fun activities.

In September 2022, the Kopenitz family hosted the first No Limitations Field Day in Odessa, and other community members around the country hosted their own field days in September and October. The events included a fundraising component, so participants could help raise money for NHF’s mission to improve the lives of people with inheritable blood disorders.

“This event is starting off small, but we’re hoping that others in the community will be inspired to host a field day, share their own stories, and help spread awareness,” Scott says. Adds Emily: “As Steven and Adam get older, we can see them getting more and more involved with planning and putting on the event. It will be a great way for them to give back to the community.”

Giving back comes naturally to the Kopenitz family. Scott’s parents, Steve and Sheila Kopenitz, have been generous supporters of NHF since 2017, and in 2019 the couple made a five-year commitment to support the Connections for Learning program, which enables families with limited financial means to attend the Bleeding Disorders Conference. More recently, the couple donated to NHF’s health equity initiatives.

“Now, with this new field day event, we see our grandsons, even though they are still very young, taking their first step into philanthropic efforts and understanding that it’s a lot more than just writing a check,” Steve says.

—By Donna Behen



COURTESY OF THE KOPENITZ FAMILY

A GIVING FAMILY

Scott and Emily say the field day will allow their boys to give back to the community.



IF YOU’RE INTERESTED IN HOSTING A FUTURE FIELD DAY EVENT, EMAIL:

Maureen Parsons, director of individual giving, at mparsons@hemophilia.org.

Side by Side

For some chapters, this summer was the first time in two years that they could hold in-person camps and other programming for the bleeding disorders community. Here's a rundown of how three chapters provided summer fun for children and adults alike.



PLENTY OF FISH
Men's retreat participants enjoyed an all-day excursion on the Louisiana fishing vessel.

LOUISIANA HEMOPHILIA FOUNDATION MEN'S EDUCATIONAL RETREAT

The Louisiana Hemophilia Foundation (LHF) held its second annual Men's Educational Retreat in late June. Men with bleeding disorders, some with male relatives in tow, came from all over the state to participate in the event in Grand Isle, Louisiana.

After a casual dinner on Saturday evening, the men woke up early to board the Louisiana, a multilevel fishing vessel. By the end of the day, they had caught more than 50 redfish, which made for a wonderful dinner for the group. There was even enough fish left for every participant to bring home a bag of fillets to share with their families.

"We are so excited that our guys had such a nice time and were able to connect with one another," says LHF Executive Director Ashley Castello. "We are also grateful to our sponsors for making this event possible."

Learn more about the Louisiana Hemophilia Foundation: lahemo.org



WANT YOUR EVENT FEATURED IN CHAPTER ROUNDUP?

We are not able to highlight all programs due to an overwhelming response. However, we appreciate your submissions and look forward to highlighting more in future issues. If you'd like to feature your chapter's event in the next issue of *HemAware*, email Donna.Behen@Manifest.com.



HEMOPHILIA FOUNDATION OF MICHIGAN CAMP BOLD EAGLE AT PIONEER TRAILS

After two summers of providing virtual camp activities, Hemophilia Foundation of Michigan (HFM) campers were thrilled to gather in person at Camp Bold Eagle, a traditional summer camp in Holton, Michigan, complete with archery, arts and crafts, canoeing, swimming, and nature walks. Campers and counselors, some of whom have bleeding disorders themselves, stay in rustic cabins in the woods and eat meals together. For some children, it is the first time they are surrounded by peers who have also faced the challenges of living with a bleeding disorder.

This year, HFM introduced Eagles Nest Family Camp, for families with children ages 5 to 9. This camp acts as a bridge for young campers to attend Camp Bold Eagle the following summer feeling confident and excited.

Learn more about the Hemophilia Foundation of Michigan: hfmich.org

NEVADA CHAPTER OF THE NATIONAL HEMOPHILIA FOUNDATION PLAYCATION FAMILY FUN EVENT

A combination of in-person and virtual events in both Reno and Las Vegas in mid-June made for a great "playcation" experience for families in the Nevada Chapter of the National Hemophilia Foundation (NHF).

Events included bowling, a virtual escape room, bingo, and an ice-cream social. An art activity using body crayons provided a fun anatomy lesson for kids, helping them learn to identify important joints and muscles and better understand how the body works, how joint bleeds happen, and how "target joints" form.

Families could also overcome some perceived limitations with an indoor rock climbing center at gyms in both locations. Indoor climbing can be a safe alternative to outdoor rock climbing for people with bleeding disorders. (NHF considers it a moderate-risk sport.)

Learn more about the Nevada Chapter of the NHF: hfnv.org

—By Christina Frank



COURTESY OF NHF CHAPTERS

Pilot Program Focuses on Precision Medicine for Genetic Diseases

With input from blood-disorders groups, a genome-sequencing tool screens newborns and identifies treatment options

The Rady Children’s Institute for Genomic Medicine (RCIGM) recently announced a pilot program designed to advance a diagnostic and precision medicine guidance tool known as BeginNGS. RCIGM is a nonprofit research institute embedded within Rady Children’s Hospital and Health Center in San Diego.

Pronounced “beginnings,” the tool uses rapid whole-genome sequencing to screen newborns for about 400 genetic diseases for which there are existing treatment options. BeginNGS is meant to identify viable treatment options before the onset of symptoms.

After a diagnosis is made, the follow-up Genome-to-Treatment tool provides physicians with immediate and specific treatment guidelines. The tool helps clinicians understand these genetic conditions and their corresponding treatment options. Depending on the scenario, the options may include therapeutics, dietary changes, surgery, medical devices, and other interventions.

BeginNGS is the product of a collaboration between RCIGM, Alexion, AstraZeneca’s Rare Disease group, and several other companies specializing in genomics, precision medicine, and data management technologies.

According to an RCIGM press release, the National Hemophilia Foundation and the Indiana Hemophilia and Thrombosis Center are helping provide input on bleeding and

clotting disorders to be included in the BeginNGS screening panel as well as guidance about treatments, causative mutations, and samples for testing.

“Genetic diagnosis was a key recommendation of the recently published diagnosis and treatment guidelines for von Willebrand disease (VWD). Access

to and reimbursement for genetic testing in VWD and many of the rare and ultra-rare factor deficiencies as well as rare platelet disorders and myriad rare blood disorders opens the door to access to care and potentially treatment,” explains NHF President and CEO Leonard A. Valentino.

“More emphasis on genetic medicines was a key point made by former NIH Director Francis Collins, M.D., Ph.D., in his keynote presentation at the 25th Annual Meeting of the American Society of Gene and Cell Therapy,” Valentino says. “Of the more than 7,000 rare disorders with a genetic basis, less than 500 have a therapy available. Programs like BeginNGS offer the opportunity to take the first step in the process — diagnosis, from which treatment may follow.”

EARLY ACTION
Newborn screening of blood disorders ensures prompt treatment.





**ENJOYING
RETIREMENT**

Randy and his wife, Jackie, at Great Basin National Park in Nevada.

I'm Still Here

After cheating death a few times over, I make the best of every day

I wasn't supposed to live past my 13th birthday. I'd been diagnosed with severe hemophilia A around my first birthday. My parents had been tossing me up in the air and catching me, like parents do all the time with babies, and I ended up covered with bruises. When my mom heard the diagnosis, she burst into tears — her brother had died of hemophilia when he was only 6 years old.

My parents did everything they could to give me a normal life and put me in public school. In order to reduce my bleeds, they put braces on my ankles and elbows, and I went to school in a wheelchair. But even with all that, the teachers had trouble keeping track of me on the playground, so I ended up spending most of my recesses in

the school offices. Over the summers, doctors put casts on my legs so I couldn't do much at all. But year-round, I continued to have bleeds, no matter how hard they tried to prevent them. When I was 8, I had a pretty bad ankle bleed that landed me in my hematologist's office. He was the doctor who told my parents, "You know, these kids don't really live past 13."

In order to reduce my bleeds, they put braces on my ankles and elbows, and I went to school in a wheelchair.



Those words echoed through my brain when I went to school the next day. I had trouble with math, so I told the teacher, “I don’t really need to learn this stuff because I’m going to die.” The principal immediately informed my dad, who worked in the school district, and my dad said I couldn’t say these kinds of things. A lot of kids with hemophilia had to go to special schools or even be home-schooled. He’d fought hard to get me into a public school, and I was lucky to be able to go there.

I made it past 13. It was just around that time that scientists discovered how to make freeze-dried concentrates of the clotting factors from blood. That transformed my life. I was able to get rid of the braces and the wheelchair, and I could infuse myself, rather than spend three or four days every month in the hospital as I had my entire life up until then.

I bypassed another death sentence in the 1980s, when about half of all people with hemophilia became infected

Not only did I live past 13, I’m still going strong at 68 years old.

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with HIV after receiving contaminated blood products. I was one of the rare lucky ones who tested negative, although my doctors still said I was going to die because there was no way I could escape HIV.

While I managed to escape that, I still had the hepatitis C that I contracted from plasma back in the ’70s. Yet I was able to cheat death again with an absolutely hellish treatment of interferon, ribavirin, and an antiviral that gave me flu symptoms every day for 48 weeks.

The drugs successfully knocked out the hepatitis C, but my liver had been so damaged that I needed a liver transplant. So once again, I was up against the threat of death. But I fought it off when I received a new liver last year.

Not only did I live past 13, I’m still going strong at 68 years old. My wife and I are getting used to retirement now and get to spend time with family. We have two sons and four grandchildren, the oldest of whom is in college. I have had a successful career in public health and given back to the hemophilia community in nonprofits and research. It hasn’t always been easy, but I’ve done my best to make the most of the hand I was dealt. And I think I’ve done pretty well, if I do say so myself.

—By Randy Curtis, as told to Leslie Pepper

COURTESY OF RANDY CURTIS



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