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PEN's Biennial

# BLEEDING DISORDER

## Resource Guide 2021

Laurie Kelley

We are pleased to present the community with a variety of resources to help in educating patients, finding support, and living with bleeding disorders. This guide contains a variety of resources including books, digital content, movies, apps, kits, and tools.

## BOOKS

### Raising a Child with Hemophilia: A Practical Guide for Parents (5th ed.)

Laureen A. Kelley

LA Kelley Communications, Inc. 2016  
Free to hemophilia families  
kelleycom.com • paperback or e-book  
amazon.com

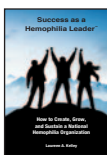


World's first parenting book on hemophilia written by a parent of a child with hemophilia. Practical, easy-to-understand info on medical treatment, genetic transmission, child development, consumer issues, school, sports. Includes advice from experienced parents, compiled from interviews with more than 180 families. Sponsored by CSL Behring.

### Success as a Hemophilia Leader

Laureen A. Kelley

LA Kelley Communications, Inc. 2018  
Free to qualified Hemophilia Leaders  
kelleycom.com • paperback or e-book



World's first guide to founding, managing, and growing a grassroots hemophilia organization or improving an existing one. Advice on creating vision and mission, forming a board, fundraising, producing a newsletter, programming, establishing an office, working with a medical advisory board.

### Teach Your Child About Hemophilia

Laureen A. Kelley

LA Kelley Communications, Inc. 2020  
kelleycom.com • paperback or e-book



Explains what your child with hemophilia may be thinking at different ages when experiencing a bleed, receiving an infusion, visiting the hospital. Interviews with dozens of children and teens examine their understanding of cuts, healing, blood, factor, severity levels, blood clotting, infusions, genetic transmission, and a cure. Helps prepare you to educate your child about hemophilia in a positive, effective, age-appropriate way.

### Dear Hemophilia: Finding Hope Through Chronic Illness

Cazandra Campos-MacDonald 2019  
amazon.com



Tender, passionate, often humorous story told in letters written to hemophilia, as if the disorder were a person. Throughout her journey to handle first one son's and then another's diagnosis, treatment, and new way of life, the author leans on

self-discovery, a great medical community, a supportive husband, her resilient children, and most of all, her faith.

### The Gift of Experience II: Conversations with Parents About Hemophilia

Laura Gray, LICSW, Ziva Mann, and Allie Boutin  
Boston Hemophilia Center 2014  
amazon.com



Compilation of personal stories from parents and caregivers of hemophilia patients; offers insights into the daily life of raising a child with hemophilia.

### Legacy: The Hemophilia of Yesterday

Matt Barkdull 2014  
amazon.com



First published diaries of hemophilia, the true story of a teenager with a passion for writing in a western farming community before World War II. He records his hopes, dreams of independence and romance, suffers excruciating pain, yet expresses profound faith.

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This is a sampling of the many resources available in the bleeding disorder community. We decided to focus on less publicized resources, so most are not from National Hemophilia Foundation (NHF), Hemophilia Federation of America (HFA), or World Federation of Hemophilia (WFH). Find more resources by viewing the publications or resources section of each website, or by

contacting the company or organization. Visit nhf.org, hemophiliafed.org, and wfh.org for many more resources, and search amazon.com. Most of the resources listed here are free.

# welcome



The pandemic has stranded most of us at home, with our regular routines upended. I'm sure Apple and Amazon are seeing movie downloads and rentals soaring. I'm guilty of lots of movie watching too (I'm a huge fan of the Marvel Universe), but I also read 50 books in 2020! And work has not slowed down, except for the lack of travel.

Have you been able to spend more time reading? Or maybe investigating more resources about bleeding disorders to increase your knowledge? If not, now's the time! We've made it easy for you, by compiling as many resources as we can. And most are free. In this issue, also check out our newest column, Physio & Fitness, with Mike Zolotnitsky, DPT, a man with hemophilia who is a born educator. You can catch his videos on YouTube, and read his new PEN column about getting in shape and treating your joints well.

With the new year, make a commitment to read more and learn more. And maybe exercise more? Stay safe...and hopeful!

## inbox

I AM HAPPY TO BE on the PEN distribution list. I so enjoy the information you put forward. It is very helpful and very well produced, not to mention very up-to-date with even the smallest progress in the field.

*Deya Corzo, MD, FACMG*  
Chief Medical Officer, Sigilon Therapeutics, Inc.  
MASSACHUSETTS

I WANTED TO THANK YOU for all the books and info you sent me. I also love getting your emails. You're very inspirational and so kind to help us with our concerns about little Liam, who was born in November. So glad there are people out there like you. Thanks again!

*Mary Shortell*  
IOWA

WE DID OUR FIRST HEMLIBRA injection at home today. I wanted to make sure to include our unaffected three-year-old so he doesn't feel left out, but I was super nervous because, well, three-year-olds are emotional and impulsive.

We used the *My First Factor* infusions book and just changed a few words since we are doing Hemlibra for prophylactic care. We read the book together first, and then I had Sam hold the book and turn the pages to "tell me what to do" step-by-step, and it went great! Thank you so much for providing these books!

*Michelle Krakowiak*  
SOUTH CAROLINA



In the past, it was incorrectly believed that only men could have hemophilia, and women with the gene were labeled asymptomatic "carriers." It's now recognized that women are not just carriers of hemophilia, but can also have hemophilia and experience symptoms if less than 50% of their factor is active. Most diagnosed patients are male. For editorial simplicity in PEN articles, when we refer to a person with hemophilia, we may alternately use "he," "she," or "they."

## PARENT EMPOWERMENT NEWSLETTER FEBRUARY 2021

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as i see it

# Patient Education Through Social Media in the COVID Era

Laurence Woollard

The unparalleled, seismic societal shifts over the past year have made many of us readjust in ways we'd never imagined. As I write this, here in the United Kingdom we are facing yet another national lockdown. Strict physical distancing has meant that people are using digital social networks to interact and share information on a historic, extraordinary scale. According to *We Are Social*, by October 2020, the number of people using social media worldwide surpassed the 4 billion milestone, with an average of 2 million new users joining every day.<sup>1</sup>

While the environment created by the pandemic has bred many falsehoods on social media from so-called armchair epidemiologists, the rise in online traffic from housebound, captive audiences has also inspired more entrepreneurial hustle. Social media is unique; it places people at the center of a vast network, and shifts power by allowing anyone to become an “influencer.” This relatively new phenomenon has grown with the mania for online video content, and YouTube is the dominant platform. YouTube influencers, through their informality and authenticity, can be seen as models for observational learning; they have the potential to guide or change the beliefs of their followers. One of the standout “heroes” during the pandemic was Joe Wicks, known as “The Body Coach,” who had over 75 million views globally of his daily “PE with Joe” fitness sessions on YouTube.

For many people in the bleeding disorder community under lockdown, decreased physical activity may have negatively impacted their joints and muscles.<sup>2</sup> In response, patient advocacy groups have been forced to adopt and improve virtual operations and e-learning approaches using social media to promote their members' well-being. For example, the European Haemophilia



Laurence Woollard: Hemophilia influencer

Consortium (EHC) hosted its physical activity campaign #thisway through monthly Facebook live sessions with a specialist physiotherapist.

Social distancing has put a strain on the mental health of many individuals. For young adults in particular, the World Health Organization (WHO) has suggested that staying connected with peers through social media can help them remain positive and challenge mental health stigma. The explosion in popularity of the entertainment-based platform TikTok—with over 30 million monthly users in the US alone—has demonstrated the potential not only to convey important health information, but to address these aspects of the pandemic as well.<sup>3</sup>

Even pre-COVID, more and more people were using social media to gain knowledge and share their health experiences.<sup>4</sup> As a result, social media has been promoted as an inexpensive means for patient education, to enable and empower consumers in their health and healthcare-related interactions.<sup>4,5,6</sup> This is particularly significant for people living with chronic conditions, where management and care can be self-guided, fostered through online peer-to-peer interaction and validation, or assisted by a facilitator or healthcare professional.<sup>4</sup> The

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1. *We Are Social*, Digital 2020 (2020), [datareportal.com/reports/digital-2020-october-global-statshot](https://datareportal.com/reports/digital-2020-october-global-statshot) 2. H. De la Corte-Rodriguez, et al., “What COVID-19 Can Mean for People with Hemophilia Beyond the Infection Risk,” *Expert Review of Hematology* 13, no. 10 (2020): 1073–79. 3. C. H. Basch, et al., “COVID-19 on TikTok: Harnessing an Emerging Social Media Platform to Convey Important Public Health Messages,” *International Journal of Adolescent Medicine and Health*, Aug. 10, 2020. 4. L. Zhou, et al., “Harnessing Social Media for Health Information Management,” *Electronic Commerce Research and Applications* 27 (2018): 139–51. 5. M. Stellefson, et al., “Evolving Role of Social Media in Health Promotion: Updated Responsibilities for Health Education Specialists,” *International Journal of Environmental Research Public Health* 17, no. 4 (2020): 1153. 6. H. Korda, et al., “Harnessing Social Media for Health Promotion and Behavior Change,” *Health Promotion Practice* 14 (2013): 15–23.





Paul Clement

## Inhibitors 101

Part 1 of 2

For many parents of children newly diagnosed with hemophilia, the word “inhibitors” soon becomes part of their vocabulary. And although they may not know at first what an inhibitor is, they may have learned to associate the word with something fearful. But for people with hemophilia A and inhibitors, things aren’t as bad as they once were.

What exactly is an inhibitor? Who gets them? What happens when you get an inhibitor? How do you treat bleeds if you have an inhibitor? Do inhibitors go away on their own, or can you grow out of them or eliminate them?

### What Is an Inhibitor?

Inhibitors are specialized proteins called *antibodies*. They’re a part of the immune system that protects us from bacteria, viruses, and foreign proteins—in other words, anything that the body identifies as not belonging, and as being potentially harmful. But sometimes the immune system makes mistakes: it may even attack the body itself, as in autoimmune diseases including rheumatoid arthritis or multiple sclerosis. With hemophilia, the immune system also makes a mistake: it misidentifies a helpful agent—infused clotting factor—as something harmful, and then mounts an immune response against the factor to neutralize it and mark it for removal from the body.

Inhibitors are very efficient. When an inhibitor is present in hemophilia, some or all of the infused factor is neutralized within minutes. This prevents the factor from participating in the clotting process to stop bleeding. And it means that people with inhibitors can’t use standard clotting factor concentrates to control bleeds.

Unfortunately, the alternative therapies we have for treating bleeds with inhibitors aren’t as effective as standard factor at controlling bleeds. As a result, people with inhibitors tend to bleed longer, develop target joints (joints that bleed frequently), and suffer from joint damage more often than people without inhibitors.

Fortunately, for people with hemophilia A and inhibitors, treatment has improved dramatically over the past three years.

### Diagnosing Inhibitors

How do you know if you have an inhibitor? There are usually no outward signs. Inhibitors are sometimes diagnosed during routine hemophilia treatment center (HTC) clinic visits; and sometimes inhibitors are suspected after you notice that factor infusions fail to adequately control bleeding. Your HTC should test for inhibitors at least annually and always before any surgery, and you should request a test whenever you feel that bleeds aren’t being controlled effectively with your usual dose of factor.<sup>1</sup>

When an inhibitor is suspected, a diagnostic test called a *mixing study* (activated partial thromboplastin time, or aPTT) is performed: the patient’s blood plasma is mixed with normal plasma to see if this corrects the clotting time. In someone with hemophilia without an inhibitor, a mixing study results in a normal clotting time; but if an inhibitor is present, then the clotting time is abnormally prolonged. If this happens, then another test, the Bethesda inhibitor assay, is done to determine how much of the inhibitor-causing antibody is present.<sup>2</sup> The Bethesda assay is a quantitative assay, meaning that it measures the amount of inhibitor and the results are expressed in numbers.

Note: Testing for inhibitors is a bit tricky. It’s best to have a Bethesda assay done at an HTC, because the lab techs there have more experience performing the tests, and the results are more likely to be accurate when compared to tests done at other hospitals.

### Strength of the Inhibitor

To develop a strategy for treating bleeds, your doctor will need to know the strength, or concentration, of the inhibitor. The inhibitor strength is reported as a “titer” and is expressed in Bethesda Units (BU).<sup>3</sup> Inhibitor titers can be as low as 1 BU

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1. You can get free inhibitor testing at federally funded HTCs by participating in the Centers for Disease Control and Prevention’s (CDC) Community Counts Registry for Bleeding Disorders Surveillance program. 2. There are several different types of inhibitor assays; the Bethesda assay is the most widely used. 3. A Bethesda Unit (BU) is the amount of an inhibitor that will neutralize 50% of factor VIII in normal plasma after 120 minutes’ incubation at 37°C.

Richard J. Atwood

Linda Weaver's Studio



## A Passion for Music: The Currence Brothers

**H**ave you heard of the Currence Brothers? This band from West Virginia played and recorded gospel, bluegrass, and country music in the 1970s. The band members were known for their musical talent, especially with the banjo and fiddle. And two Currence brothers, plus their nephew, happened to have hemophilia. These band members overcame hardships to fulfill their passion for music.

For five generations, the musically inclined Currence family lived in High Germany on the Randolph-Upshure county line in central West Virginia. They lived in a six-room house on a small farm, four miles from a paved road. The nearby one-room school in High Germany housed 45 students in eight grades.

Every member of the large Currence family—including seven boys and five girls—either played a musical instrument or sang, though none was trained or ever had a music lesson. The children and their parents would listen to a battery-powered radio or hear live entertainment, then pick out the tune on guitar, fiddle, or mandolin. At home or in church, the family members sang and played musical instruments.

There appeared to be no family history of hemophilia. That is, until one of the Currence boys, Junior (1923–1925), died at age two after he bled out from a bumped nose, and hemophilia was later suspected. Junior's younger brothers Jimmie (1932–1992) and Loren (1934–1987) grew up with bleeding issues, but they never visited a doctor until they were teens. There was no ice for treatment and nothing for pain, though the reason for this is unclear. The brothers applied high-powered liniments from Blair products for hemorrhages into their joints.

Jimmie and Loren seem to have visited at least one local doctor, and they were treated as “bleeders.” It's possible that lack of money, transportation, and awareness of the need for a hematologist may have prevented visits to an appropriate specialist or hematology lab. Before factor VIII factor concentrates became available, the brothers received blood transfusions. Loren once received sixteen pints of blood for a kidney bleed. When Jimmie had a stomach bleed as a teenager, he received a pint of blood from his brother-in-law. The boys were often laid up from

hemorrhages into their joints or from other internal bleeds.

Jimmie and Loren didn't have their hemophilia diagnosed until the early 1960s. Dr. Mabel M. Stevenson, a hematologist at Morgantown University Hospital, diagnosed severe classical hemophilia in both brothers, who then called themselves “severe hemophiliac bleeders with near-zero clotting factor.” Neither brother could ever find full-time employment or get insurance. They both received supplemental social security, and the state of West Virginia paid for their medicine because they couldn't afford it. Both brothers married, and each had three daughters.

Brothers Jimmie, Loren, Marvin (“Shorty”), and Buddy—along with Malcomb Pastine, a nephew who also had hemophilia—formed the Currence Brothers Band. Loren played guitar, sang, and managed the band. Jimmie played fiddle and banjo, winning banjo and fiddle contests in four states: West Virginia, Maryland, Pennsylvania, and Virginia. Jimmie won the fiddling championship in West Virginia for four consecutive years, and was undefeated grand champion in Maryland in 1970. Then his elbow bleeds forced him to switch from fiddle to banjo. Malcomb played guitar, and even did turkey calls. Buddy sang, while Marvin played the fiddle.

The Currence Brothers began spreading the gospel by playing their music at church functions. They traveled the country, usually in their van, to play in concerts and on radio or television. The band even recorded on their own, or with notable musicians, producing several albums in a genre they called Gospel Grass, along with a mixture of popular tunes. They collaborated with musicians including Larry Groce, who they met at a talent show at the firehouse in Parsons, West Virginia; Woody Simmons, who lived in Randolph County; Clyde See; and “Country” Charlie Jordan.

You can find Currence Brothers music on old vinyl records and in a few YouTube videos. Check out the following albums:

- The Currence Family, *Precious Memories* (Lark Records, n.d.)
- Larry Groce and the Currence Brothers (Peaceable Records, 1975)
- Larry Groce, *Junkfood Junkie* (Warner Bros. Records, 1976)

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Michael Zolotnitsky, PT, DPT

## Keep Your Lower Extremity Joints Moving!

The pandemic impacted everyone's life in 2020, making it especially hard to stay healthy. While many of us began exercising from home, some of us have completely stopped exercising because of the increased stress of working from home, managing our children's school schedules, gyms closing, expensive home exercise equipment, or just being out of routine. This isn't good for anyone, but if you have a bleeding disorder, lack of exercise can cause major setbacks, including joint stiffening, that put you at greater risk of joint bleeds and more joint damage.

As a physical therapist with severe hemophilia, I'm going to focus on eight imperative workouts to perform daily without any equipment at all. And I'll explain why these are important to maintain strength and reduce areas of common joint damage!

### 1. Sit to Stand

Sit in a chair, cross your arms, keep feet shoulder-width apart, toes pointed forward, and stand up. Slowly lower yourself into the chair, repeating 9 more times for a total of 10. If this is challenging, use your arms for support until your legs build strength. If this is easy, find a lower surface.

**Purpose:** Build strength through the quads and glutes to reduce difficulty with walking and stair negotiation, while reducing pressure on the knee and hip joints.

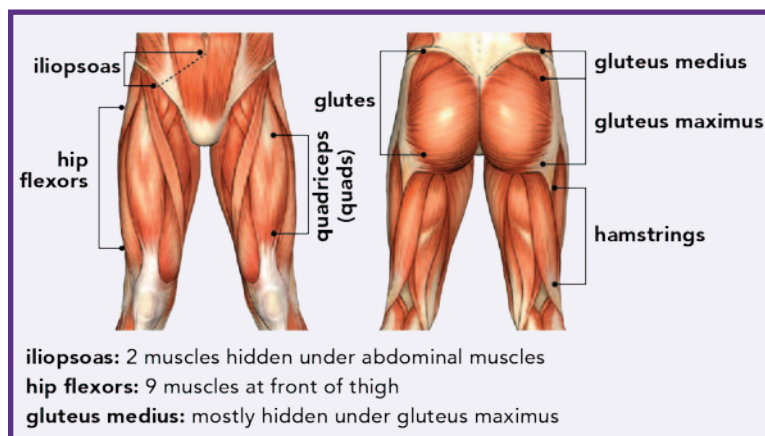
### 2. Glute Bridges

Lie on your back, making sure your spine is neutral. Bend both knees, keeping your feet on the floor. With your knees slightly apart, push through your heels to raise the hips up. If this is challenging, use your arms to help push your body up. If this is easy, progress to perform one leg at a time.

**Purpose:** Build strength through the glutes and the hamstrings. Also great for core activation and balance if performing with one leg. Building up sufficient glute and hamstring strength will also reduce pressure on the knee and hip joints.

### 3. Standing Hip Abduction

Stand facing a wall, with hands on the wall, knees shoulder-width apart. Start with one leg. Slowly bring it out to the side with toes pointing forward, and then return. Begin with 10–12 repetitions (reps) and progress to 15–20, making sure your body is not rocking sideways. If this is easy, progress to doing the reps



without holding onto the wall. While performing this on the right side, the left side is focused on stabilizing, so both hips will feel the workout!

**Purpose:** Build strength in the gluteus medius, which will keep you upright when walking and improve overall balance to reduce your risk of falling.

### 4. Standing Hip Extension

Stand facing a wall, with hands on the wall, knees shoulder-width apart. Start with one leg, and slowly extend the leg back, with toes pointing forward. Make sure the lower back is not arching backward or forward; squeeze the glute to engage proper activation. Begin with 10–12 repetitions (reps) and progress to 15–20, making sure your body is not rocking sideways. If this is easy, progress to not holding onto the wall.

**Purpose:** Build endurance to reduce difficulty in activities requiring prolonged periods, such as hiking or taking long walks. Improving glute strength and performing standing extension-based exercise is important because we are often in the flexed posture throughout the day.

### 5. Penguins

Stand with your toes pointing forward, and waddle side to side, standing in place. You'll feel this in the sides of both hips. Begin for 10–20 seconds with feet 1 to 2 inches apart, and progress to 30–60 seconds with feet 3 to 5 inches apart.

**Purpose:** Build further gluteus medius strength and improve balance. While you balance on the one lower extremity, the other is working. Balance training has been shown to reduce pain in the ankle and knee, and reduce effects of osteoarthritis.

# PROGRAMS & WORKSHOPS

Resource Guide... from cover



## Project SHARE

Save One Life  
saveonelife.net

Humanitarian program donates factor to developing countries. Recipients are patients, doctors, clinics, hospitals in countries where factor is scarce or unavailable. Since 2002, SHARE has donated more than \$200 million worth of factor to over 75 developing countries.

## Hope for Hemophilia

hopeforhemophilia.org



National nonprofit that assists patients with bleeding disorders when they face financial hardships, emotional challenges. Founded by Jonathan James, a man with hemophilia, the organization has given over \$1 million in assistance since 2009.

## Inhibitor Education Summits

National Hemophilia Foundation (NHF)

English and Spanish  
hemophilia.org

Educational summits for people living with inhibitors, covering most travel expenses for participants. The only national educational forums for inhibitor patients to meet and learn about their rare complication.

## Inhibitor Family Camp

Comprehensive Health Education Services (CHES)  
comphealthd.com



Camp addresses the unique needs of children with active inhibitors, and their families. Full weekend of education, support, fun. Held twice a year; camper costs covered.



## Danny's Dose

dannysdose.com

Organization advocating for the change of emergency medical protocols for chronic illness and rare disease.

## North American Camping Conference of Hemophilia Organizations (NACCHO)

Arizona Hemophilia Association, Inc.  
arizonahemophilia.org

Weekend workshop held early in the year for planning, organizing, operating a bleeding disorder summer camp. Nationally known presenters share camp resources and techniques; explore camp programs and activities; facilitate networking and problem-solving.



## Bayer Leadership U

Bayer HealthCare  
livingwithhemophilia.com

Paid summer internship at Bayer's US headquarters in Whippany, New Jersey. College-aged members of the hemophilia community can sharpen leadership skills while working alongside leaders at Bayer.

## Game On

Bayer HealthCare  
English and Spanish  
livingwithhemophilia.com

GAME ON

Empowers young adults with hemophilia to be accountable for their treatment, stay healthy, maintain insurance coverage, and plan for college and careers.

## And Now...Back to You

Bayer HealthCare  
English and Spanish  
livingwithhemophilia.com

AND NOW...  
BACK TO YOU

Helps adults with hemophilia rediscover goals related to career, education, or passions, and create a plan of action for success.

## Parents Empowering Parents (PEP)

Bayer HealthCare  
English and Spanish  
livingwithhemophilia.com



Provides tools, skills, and emotional support to parents of children with hemophilia.

## Transition Ignition

Bayer HealthCare  
livingwithhemophilia.com

Helps teens establish a comfort level with hemophilia and practice skills for managing treatment away from home.

## Step Up Reach Out (SURO)

Bayer HealthCare  
livingwithhemophilia.com



International youth program helps connect future leaders in the bleeding disorder community, with the goal of increasing treatment standards worldwide.

## Affirm

Bayer HealthCare  
livingwithhemophilia.com



International fellowship program helps men with hemophilia develop and expand leadership and advocacy skills by fostering networking within the bleeding disorder community.



# PROGRAMS & WORKSHOPS CONTINUED

## HeroPath™ Life Coach

*Novo Nordisk Inc.*

[mynovosecure.com](http://mynovosecure.com)

Coaching and peer support helps teens and young adults with bleeding disorders excel in daily life and chart a path forward. Based on findings from HERO (Hemophilia Experiences, Results, and Opportunities) initiative, the largest international study on the psychosocial impact of hemophilia on patients and their loved ones.

## Wingmen Foundation

[wingmenfoundation.org](http://wingmenfoundation.org)



Nonprofit founded by two men with hemophilia offers support to people with bleeding disorders through physical fitness, fitness education, advocacy, financial assistance for physical rehabilitation, exercise equipment.

## Rebuild

*Diplomat Specialty Infusion Group*

[diplomatpharmacy.com/rebuild](http://diplomatpharmacy.com/rebuild)

Collaborative preventive care program enhances physical therapy services and promotes positive therapy outcomes for people with hemophilia. Directed by physical therapist with over 20 years of hemophilia experience.

## Share Your Why

*Sanofi Genzyme*

[shareyourwhy.com](http://shareyourwhy.com)

Professionally trained community members, called CoRe, are available to share their journey in hemophilia and help participants navigate their own. Connect with CoRe through videos, at regional meetings, and via email.



## Generation IX

*Coalition for Hemophilia B*

[hemob.org](http://hemob.org)

Adventure education program led by Pat “Big Dog” Torrey teaches tangible mentoring skills through experiential learning in an unforgettable setting. Open to young men with hemophilia B, ages 14–30. Sponsored by Medexus Pharmaceuticals.

## Junior National Championship (JNC)

*CSL Behring*

[csljnc.com](http://csljnc.com)

First national golf, baseball, swimming competition for the bleeding disorder community. Gives children the chance to compete; provides education and information sharing for participants, parents, caregivers.



## Gettin' in the Game<sup>SM</sup> (GIG)

*CSL Behring*

[cslbehring.com](http://cslbehring.com)

Helps children with bleeding disorders participate in sports and get active. Local GIG events offer children and families sports tips from national GIG athletes with bleeding disorders.

## Common Factors<sup>®</sup>

*CSL Behring*

800-676-4266

Community-based events unite and educate members of the bleeding disorder community. Common Factors Advocates share personal experiences and give presentations on topics for the community.

## Hello Talk<sup>®</sup>

*Takeda*

English and Spanish

[bleedingdisorders.com](http://bleedingdisorders.com)



Live education program for the bleeding disorder community. More than 25 Hello Talk topics cover disease state and lifestyle education.

## Patient Notification System (PNS)

*Plasma Protein Therapeutics Association*

[patientnotificationsystem.org](http://patientnotificationsystem.org)

Confidential 24-hour communication system provides info on plasma-derived and recombinant therapy withdrawals and recalls through automatic electronic updates.

## LadyBugs

*Comprehensive Health Education Services (CHES)*

[comphealthed.com](http://comphealthed.com)



This program empowers women, age 16 and older, who have a bleeding disorder, care for someone with one, or are carriers. LadyBugs helps women advocate when making healthcare decisions for themselves or their loved ones; educates about medical developments, advocacy skills, and stress management techniques; encourages women to recognize that their health is important.



# HEMOPHILIA & HIV

## June for Joint Health

National Hemophilia Foundation/Sanofi Genzyme

[juneforjointhealth.org](http://juneforjointhealth.org)

About 80% of bleeds happen in joints, which may lead to permanent joint damage.

June for Joint Health encourages patients to develop lifelong habits that promote safe, joint-building physical activities. Webinars and videos instruct on how joints work, how to maintain healthy joints, and physical therapy.



## Looking Beyond Trough

Sanofi Genzyme

[lookingbeyondtrough.org](http://lookingbeyondtrough.org)

For hemophilia B patients. Educational charts compare hemophilia A and B; show how factor IX operates in the bloodstream; define a trough; explain how treatment can protect you beyond your trough levels and why this is important.

## KITS



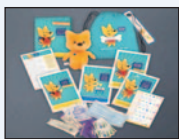
### School Preparedness for Bleeding Disorders

Diplomat Specialty Infusion Group  
[diplomatpharmacy.com](http://diplomatpharmacy.com)

Helps educators, family members, and others facilitate a safe, healthy school experience for students with

bleeding disorders. Resource binder can accompany complete educational program for teachers, nurses, staff.

### Factor Friend®



Takeda

[bleedingdisorders.com](http://bleedingdisorders.com)

Personal therapeutic play kit helps children with hemophilia become

familiar with infusing and the steps to self-care. Contact your HTC to request the kit.

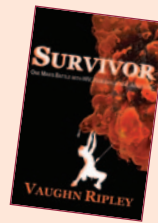
### Mental Health Matters Too (MHMT)

Debbie de la Riva

[mentalhealthmatterstoo.com](http://mentalhealthmatterstoo.com)

MHMT acknowledges the turbulent

past of the bleeding disorder community and the psychological changes that may have resulted. This program creates awareness of mental health issues in the community and provides guidance and resources for mental and emotional care.



### Survivor: One Man's Battle with HIV, Hemophilia, and Hepatitis C

Vaughn Ripley 2010

[amazon.com](http://amazon.com)

Story of courage after a diagnosis of HIV: how Ripley turned his life around to become a professional database administrator, and how medical advances allowed him and his wife to become parents.



### Dying In Vein: Blood, Deception...Justice

Kathy Seward MacKay and

Stacy Milbourn 2004

[amazon.com](http://amazon.com)

Stirring photo journal of individuals and families with hemophilia affected by HIV and hepatitis. Portrays people who suffer, become advocates, and mourn loved ones.



### Bleeder: A Memoir

Shelby Smoak 2013

[amazon.com](http://amazon.com)

Tender, exquisite memoir of Shelby's life from 1990 to 1998, with flashbacks to share college, dating, career difficulties experienced by a young adult with hemophilia and HIV. Sponsored by Diplomat Specialty Infusion Group.



### Bad Blood: A Cautionary Tale

Necessary Films 2010

[amazon.com](http://amazon.com)

Gripping documentary about how HIV and hepatitis C contaminated the US blood supply in the 1970s and 1980s; the role of various organizations and companies; effects on hemophilic families; how community advocates changed the blood-banking system.



### Vial 023: A Father's Pursuit of Justice

Gary William Cross 2012

[amazon.com](http://amazon.com)

In memoir, Cross recalls his pivotal role in the nation's "hemophilia HIV pandemic": his 17-year-old son Brad died in 1993 after becoming infected with HIV as a child through contaminated clotting factor.

# BOOKLETS & BINDERS

## Discussion Guides

Takeda

[bleedingdisorders.com](http://bleedingdisorders.com)

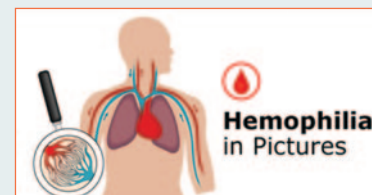
Asking the right questions can help you and your healthcare provider (HCP) find a treatment plan that works for you. Takeda's discussion guides include tips on what to track before your next visit, how to evaluate your current approach to treatment, and treatment questions for your healthcare provider.

## Hemophilia in Pictures: Educator's Guide

World Federation of Hemophilia (WFH) 2005

English, Spanish, French  
Arabic, Russian, Chinese  
[wfh.org](http://wfh.org) • PDF download

Hemophilia taught in pictures that provide detailed info for advanced learning. Includes tips for effective patient education, key talking points, review quizzes.



## LIVING WITH HEMOPHILIA B — B2B BOOK SERIES

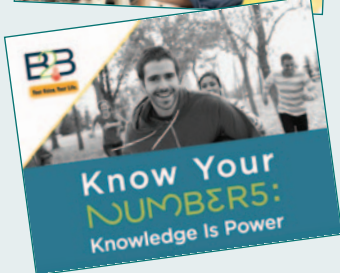
Pfizer Inc. 2018

[hemophiliavillage.com](http://hemophiliavillage.com), [hemob.org](http://hemob.org) • PDF download, e-book formats



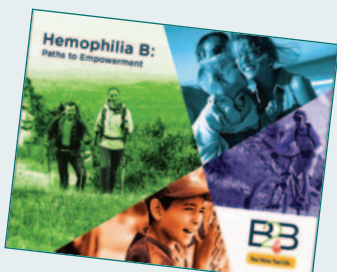
### ► Hemophilia B: Her Voice, Her Life

Provides support and encouragement to women with hemophilia B on the path to empowerment.



### ► Know Your Numbers: Knowledge Is Power

Explains importance of knowing and understanding specific sets of numbers, test values, and other hemophilia B data through all life stages.



### ► Hemophilia B: Paths to Empowerment

What empowerment means to patients and caregivers, and how it affects their lives.



### ► Hemophilia B: A Family Perspective

Real-life examples and guidance for families living with hemophilia B.



### ► Navigating the Preteen Years

Advice and support for preteens with hemophilia B.



### ► Hemophilia B: Your Point of View

Advice and tips to help adolescents and teens deal with challenges they may encounter.



### ► Young Adults and Hemophilia B

Information for young adults about dating, moving out, starting a career, starting a family.



### ► Learn from Experience: A Guide for Mature Adults

Helpful info to make life decisions and stay healthy today and in the future.



### ► Hemophilia B in Early Childhood

What to expect when raising a child with hemophilia B.

## GADGETS!



### Be A Hero Infusion Mat

Diplomat Specialty Infusion Group  
diplomatpharmacy.com

Infusion mat for kids makes infusions easier with easy-to-clean surface and colorful step-by-step instructions.



### StrapWrap™

Diplomat Specialty Infusion Group  
diplomatpharmacy.com

Medical alert device can be attached to a seatbelt, stroller, backpack: anything with a strap.



### Emergency Headrest Covers

Danny's Dose

dannysdose.com

Car headrest covers include a pocket for emergency and medical information cards featured on Danny's Dose website.

### Automobile Alert Decals

Danny's Dose

dannysdose.com

Decals to be placed on cars in case of emergency. General medical alert and bleeding disorder specific decals available.

## PODCASTS



### BloodStream™ Podcast

Believe Limited

bloodstreampod.com

Provides 360-degree look at the bleeding

disorder community: news, updates, community contributions. Includes "Ask the Expert" to answer questions from community members; BloodLine, a nonfiction storytelling podcast. Hosted by Patrick James Lynch.

### B2B Podcast: B's in a Pod

Pfizer Inc.

hemophiliavillage.com



Podcast series: patients, caregivers, friends in the hemophilia B community discuss advocacy groups, sibling relationships, self-infusion, camp, ER visits, dating, marriage.

## VON WILLEBRAND DISEASE (VWD)

### A Guide to Living with von Willebrand Disease

Laureen A. Kelley and Paul Clement  
LA Kelley Communications, Inc.  
2002, 2006, 2012

#### New edition coming in 2021!

Free to families and patients  
kelleycom.com

Fourth edition of the world's first book on the world's most commonly inherited bleeding disorder. Topics include learning to cope with VWD, inheritance, the medical system, treatment, women's issues, health insurance. Complete resource guide and real-life stories. Sponsored by CSL Behring.

### The Hemophilia, von Willebrand Disease & Platelet Disorders Handbook

Hemophilia of Georgia  
hog.org

Comprehensive online guide to living with a bleeding disorder; written from the perspective of the person with a bleeding disorder, but also for family members.

### VWD Connect Foundation

vwdconnect.org



Formed to serve the bleeding disorder community, the foundation focuses on severe VWD. Provides education and connection for patients and families; supports research to benefit the VWD community.

### National Outreach for von Willebrand (NOW)

Arizona Hemophilia Association  
arizonahemophilia.org

National educational conference for individuals and families living with VWD. Info on new medical advances, tools to better manage VWD, sharing with others. Travel expenses paid. Funded by a grant from CSL Behring.







## MY FIRST FACTOR Series

Shannon Brush

Illustrated by Brooke Henson

LA Kelley Communications, Inc.

2008–2015

kelleycom.com

hardcover or e-book download

World's first books for toddlers with hemophilia. Series of colorful, chunky books just right for small hands. Available as 10-book gift set or individually. Sponsored by Bayer HealthCare. Ages 18 mo.–4 yr.

### ► Words

One-word concepts about family and hemophilia.

### ► Week

Regular infusions help a toddler stay active.

### ► Fitness

Yoga, playing, laughing, and good food keep a toddler healthy.

### ► HTC

Who does a toddler meet at the HTC?

### ► Hemophilia

What is hemophilia? Bruises, "owie," factor!

### ► Infusions

What are the steps in an infusion? A first look for toddlers.

### ► Joints

Let's name all our joints! How do they work?

### ► Camp

What will hemophilia camp be like, when you get older?

### ► Self-Infusion

When you start growing up, you can do all sorts of things by yourself!

### ► Safety

How do I stay safe? Ways a child learns to protect himself.

### ► Mis primeras palabras del Factor

A toddler's first book about hemophilia in Spanish.

### ► Coloring Book

Download a copy at [kelleycom.com](http://kelleycom.com). Illustrations from previous *My First Factor* books help keep your toddler busy and happy.

## Joshua: The Knight of the Red Snake

Lauren A. Kelley

LA Kelley Communications, Inc.

1995

kelleycom.com

Empowering story about a preschooler with hemophilia.

Illustrated large-text format ends on a note of joy and confidence.

Sponsored by CSL Behring. Ages 3–7.



## Must You Always Be a Boy?

Lauren A. Kelley

LA Kelley Communications, Inc.

1991

kelleycom.com

Four illustrated, rhyming tales explore adult reactions to bleeds, overprotective parents, sibling rivalry, classroom bullies.

Sponsored by CSL Behring. Ages 3–8.



## Alexis: The Prince Who Had Hemophilia

Lauren A. Kelley

Illustrated by Paul Chirvase

LA Kelley Communications, Inc.

2018

kelleycom.com

paperback or e-book

True story of Alexis, youngest child of Tsar Nicholas II, and how hemophilia influenced events leading to the Russian revolution. Age 8 and older.



## Just A Boy

Ziva Mann

Illustrated by

Tatjana Mai-Wyss

LA Kelley Communications, Inc. 2016

kelleycom.com

paperback or e-book

Whimsical storybook about an imaginative boy with hemophilia who learns to control his pain as he transitions to needlesticks. Using his imagination, and with his mother's help, he conquers his fears. Ages 3–6.

## The Great Inhibinator

Chris Perretti Barnes

Diplomat Specialty Infusion Group 2011

diplomatpharmacy.com



Colorful story about Nate, a boy who has an inhibitor, that centers on creating a Halloween costume. Ages 4–7.

## I Am Nate!

Chris Perretti Barnes

Diplomat Specialty Infusion Group 2011

diplomatpharmacy.com



Story about Nate, a boy who explains how having hemophilia affects his life. Ages 4–7.

## It's Always About Nate!

Chris Perretti Barnes

Diplomat Specialty Infusion Group 2012

diplomatpharmacy.com



Explores how Nate's sisters are affected by his hemophilia. Bleeding episodes can throw a wrench into family plans, especially when they require more than just a home infusion. In the end, Nate's sisters realize that a little compassion can go a long way.

# PERIODICALS

All periodical subscriptions listed here are free.



## Parent Empowerment Newsletter (PEN)

LA Kelley Communications, Inc.  
kelleycom.com

*Quarterly.* Oldest bleeding disorder newsletter in US produced and edited by a parent of a child with hemophilia. In-depth medical, scientific, consumer, parenting articles and news to empower parents and patients as educated consumers.

## OneVoice

Save One Life  
saveonelife.net



*Bimonthly.* E-zine reports on partner organizations, camps, activities funded by Save One Life, the international nonprofit founded by Laurie Kelley that provides sponsorships to children with bleeding disorders in impoverished countries.



## Factor Nine News

Coalition for Hemophilia B  
hemob.org

*Quarterly.* Easy-to-read scientific info, research, community events related to hemophilia B.



## Dateline Federation

Hemophilia Federation of America (HEA)  
hemophiliafed.org

*Quarterly.* Healthcare info and news about bleeding disorders, government, healthcare events, clinical studies, innovative programs.



## Lifelines for Health

Comprehensive Health Education Services (CHES)  
comphealthed.com

First national publication for people with inhibitors; educational, inspirational tool for families and healthcare providers.



## The Source

Plasma Protein Therapeutics Association (PPTA)  
pptaglobal.org • PDF download only

*Quarterly.* Info about the global plasma protein therapeutics industry. Interviews with key leaders; articles on safety and innovation; stories about patients and plasma donors; US and European legislative and regulatory topics.



## The Edge

Genentech  
genentechhemophilia.com

*Biannual.* Stories from the community about lifestyles, including challenges overcome. News from Genentech, and more.



## HemAware

National Hemophilia Foundation (NHF)  
hemaware.org

*Quarterly.* magazine of NHF. Articles on medical research and treatment; families and children; community events; people making a difference.

## Connected to what matters.

Through personal education and support, we help people living with hemophilia live life as they envision.



Let's connect.  
rareblooddisorders.com  
@HemophiliaCoRes  
1-855-SGZHEME

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MAT-US-2019890-v1.0-09/2020

SANOFI GENZYME

# DIGITAL GAMES, APPS & MEDIA

## MOVIES & VIDEOS



### Let's Talk Mental Health

*Believe Ltd.* 2020

[believeitld.com](http://believeitld.com)

Film destigmatizes conversations on mental health and rare disease; five members of the US bleeding disorder community open their hearts and lives to show how we can gain strength through struggle, and that perhaps we aren't so different after all. Intended to spark conversation, increase awareness, decrease stigma. Website provides easy-to-use links, resources, and tools for connection, screening, receiving support. Produced in partnership with Mental Health Matters Too. Supported by Sanofi Genzyme.

### Bombardier Blood

2019

[bombardierblood.com](http://bombardierblood.com)

iTunes, Amazon Prime



Documentary on the extraordinary story of mountaineer Chris Bombardier, who has hemophilia B, and his quest to conquer

the Seven Summits. Explores Chris's motivation for undertaking death-defying climbs, and follows his journey to Nepal to summit Mt. Everest to raise awareness of the disparity in hemophilia care in developing countries.

### Challenge Accepted

*Genentech*

[genentechhemophilia.com](http://genentechhemophilia.com)

Web series dedicated to challenging ideas about living with a bleeding disorder, including



overcoming barriers and misperceptions, and avoiding excuses for not accomplishing dreams. Hosted by Justin Willman.

### Stop the Bleeding!

*Believe Digital*

[stbhemmo.com](http://stbhemmo.com) • YouTube



Award-winning comedic web series about a dysfunctional

nonprofit organization serving the bleeding disorder community.

## APPS

### HemMobile®

*Pfizer Inc.*

iPhone users: download on App Store

Android users: download on Google

Helps patients keep track of bleeds and infusions; log infusions quickly; record bleeds precisely; generate reports to share with the treatment team; monitor factor supply.

### HemMobile® Striiv® Wearable

*Pfizer Inc.*

[hemmobilewearable.com](http://hemmobilewearable.com)

Striiv Wearable paired with HemMobile offers activity tracking to monitor daily

activity, heart rate, number of steps. Tracking intensity of activity means better discussions with the medical team about choosing activities, staying prepared, and managing hemophilia.

## GAMES

### Hemocraft

*Pfizer Inc.*

[hemocraftquest.com](http://hemocraftquest.com)



Virtual world designed for people with hemophilia,

ages 8–16. Slay the Ender Dragon; craft components of an infusion kit; monitor the “factor bar” to prepare for adventure; share game strategy with friends and family to explain what it's like to manage hemophilia.

### HemoAction Online Game

*World Federation of Hemophilia (WFH)*

English, Spanish, French

[hemoaction.org](http://hemoaction.org)

Adventure game teaches children about hemophilia: how to prevent bleeds and manage hemophilia; the clotting process, types of bleeds, factor infusions, suitable physical activities.

## MUSIC

### My First Factor Song

*Lyrics by Carri Nease*

[kelleycom.com](http://kelleycom.com)

MP3 download

Sing along and teach through song! To the tune of “Alouette,” teach your toddler with hemophilia about bumps, bruises, “boo-boos,” factor.



### Blood Vibrations

[bloodvibrations.bandcamp.com](http://bloodvibrations.bandcamp.com)

Ongoing grassroots project collects music created by people with bleeding disorders. Provides forum for creativity,

expression, sharing, learning, wellness. Musical experience required.





# ONLINE RESOURCES

## BleedingDisorders.com

*Takeda*

Explores Takeda's scholarship opportunities, community events, articles, advice, tips; patients can connect with insurance resources and their Takeda rep or HTC.

## Hemophilia Village

*Pfizer Inc.*

hemophiliavillage.com  
Provides info about programs and resources to assist the hemophilia community, including Constructive Conversations.

## Hello Tools: School Guide

*Takeda*

bleedingdisorders.com  
Website contains presentation and downloadable PDFs: workbooks and toolkits give patients and supporters info to get started; school resource guide supports academic success, development, and well-being of students with bleeding disorders.

## Living with Hemophilia®

*Bayer HealthCare*

livingwithhemophilia.com  
Info on clotting factors, genetics, pain management, medical treatment for patients and caregivers. Website features

Bayer Leadership U, Living Fit! and "Living with Hemophilia on Your Own Terms" video series explaining hemophilia terminology.

## HemeWork

*Genentech*

hemework.com

Program designed by and for the community to support career development through inspirational community stories, tailored resources. Includes collection of career resources for patients and caregivers to learn more about workplace rights; finding assistance when transitioning to college or a new job.

## Factor My Way™

*Octapharma*

factormyway.com

New website helps hemophilia patients: free trial program for Octapharma therapies; access to factor for patients who lose coverage; copay assistance; insurance assistance; patient educators; nurse educators; social media sites for community connection; guest speakers; patient stories.



● ● ● FOR MORE INFORMATION ON ORDERING FEATURED PRODUCTS, ● ● ●  
PLEASE VISIT THE RESOURCES SECTION OF OUR WEBSITE AT KELLEYCOM.COM

### As I See It... from page 3

number of physicians involved with hemophilia on social media in a professional capacity is increasing, championed by the likes of Professor Mike Makris in Sheffield, UK, who has become an influencer in his own right. Professor Makris believes, "Information is no longer a privilege and the time when patients are more up to date and better informed than their doctors is already here."<sup>7</sup>

Feeling empowered in decision-making about one's health can play an important role in supporting people as they seek positive health behavior and lifestyle change. Yet, a high level of patient participation and engagement is essential. Preliminary studies have shown that social media interventions lead to some positive effects on the health of people living with chronic diseases, including promoting self-care and self-confidence, as well as offering psychosocial benefits, but these results are limited.<sup>4,6</sup> Similarly, the reporting in hemophilia is scarce, although a recent attempt was made to increase awareness of von Willebrand disease by targeting women in their reproductive years on social media, and inviting them to participate in an online self-assessment tool to recognize abnormal bleeding symptoms.<sup>8</sup>

Although social media is now viewed as a universal communication channel, there is a risk of reducing health information access for those who are not technologically connected. About

22% of the UK's population lack basic digital skills,<sup>9</sup> and 31% of rural US households are still without access to broadband internet.<sup>10</sup> The pandemic stands to make the impacts of digital exclusion worse for the millions of people affected, and the socioeconomic disadvantaged will be hit the hardest. What's more, engaging with eHealth (for example, health information from electronic sources) requires a skill set, or literacy, of its own to appraise and apply the knowledge gained in addressing and solving a health problem.<sup>4</sup>

Providers who design social media interventions or campaigns must be mindful of the different population segments in the patient community to ensure equity of access to educational opportunities, and not just target those who are more socially mobile and tech- and eHealth-literate. There is also still a strong need to examine not only how to tailor and deliver more effective and responsive patient education through social media, but also how to assess its impact on patient health outcomes, especially in the "new normal."<sup>9</sup>

*Laurence Woollard is founder and director of On The Pulse, an independent consultancy partnering with global healthcare providers and multi-agencies to drive patient education and choice in hemophilia and rare diseases. Laurence has hemophilia and can be reached at @TheWoollard on Twitter.*

7. M. Makris, "Twitter and Haemophilia," *Haemophilia* 26, no. 2 (2020): 181–82. 8. E. Reynen, et al., "Let's Talk Period! Preliminary Results of an Online Bleeding Awareness Knowledge Translation Project and Bleeding Assessment Tool Promoted on Social Media," *Haemophilia* 23 (2017): e282–86. 9. H. Holmes, et al., "Pay the Wi-fi or Feed the Children": Coronavirus Has Intensified the UK's Digital Divide," University of Cambridge (2020), [www.cam.ac.uk/stories/digitaldivide](http://www.cam.ac.uk/stories/digitaldivide). 10. A. Ramsetty, et al., "Impact of the Digital Divide in the Age of COVID-19," *Journal of the American Medical Informatics Association* 27, no. 7 (2020): 1147–48.

### gene therapy

#### Gene Therapy Denied...For Now

In a surprise decision, the US FDA delayed approval of BioMarin's factor VIII gene therapy product, Roctavian (formerly Valrox/ BMN 270). The FDA requested two years of safety and efficacy data on the 134 patients enrolled in the company's ongoing BMN 270-301 phase III clinical trial, to provide substantial evidence of a lasting effect based on annualized bleeding rate (ABR) in adults with severe factor VIII deficiency.

**Why this matters:** The last patient enrolled in the trial will complete two years of follow-up in November 2021, so a decision by the FDA probably won't happen until 2022.

*For info:* biomarin.com

### A Significantly Longer Half-life Factor VIII

Sanofi published positive final results from the phase I/IIa study of its novel recombinant factor VIII molecule, BIVV001 (rFVIII-Fc-VWF-XTEN), in people with severe hemophilia A. When compared to the 4-to-6 times longer half-life of extended half-life factor IX products, the 1.5-to-2 times longer half-life of extended half-life factor VIII products is disappointing. The shorter half-life of factor VIII is mainly due to interaction with von Willebrand factor (VWF). In BIVV001, Sanofi has fused three different molecules to a recombinant factor VIII protein: an Fc molecule (which extends half-life by instructing cells that ingest the factor to "recycle" factor instead of breaking it down); part of a VWF molecule (which bypasses the short half-life associated with natural VWF); and two XTENs (parts of protein molecules to further extend the half-life). In clinical trials, BIVV001 has been shown to have a half-life of 3 to 4 times that of standard half-life factor VIII. BIVV001 is now in phase III clinical trials. **Why this matters:** If BIVV001 produces positive phase III results and reaches the market, it will have the longest half-life of any factor VIII product, possibly allowing people with severe hemophilia A on prophylaxis to infuse once a week instead of two or three times a week.

*For info:* "BIVV001 Fusion Protein as Factor VIII Replacement Therapy for Hemophilia A," [www.nejm.org](http://www.nejm.org)

## Novel Therapies for Oral Administration

Atomwise, a San Francisco-based company, uses artificial intelligence (AI) technology for small molecule drug discovery. It will partner with GC Pharma (formerly Green Cross) to develop novel hemophilia therapies, including drug options that can be taken orally. Atomwise will screen targets for small molecules using its proprietary AtomNet AI drug discovery platform.

**Why this matters:** The AtomNet platform can screen 16 billion compounds for potential molecules in less than two days, dramatically accelerating the early drug discovery process, which otherwise can take months or years to complete.

*For info:* atomwise.com

## First National HTC Survey

The first national survey of patients seen at US hemophilia treatment centers (HTCs) reports that of 5,006 respondents, 94.2% to 97.9% say they are "always" or "usually" satisfied with overall HTC care, regardless of patient gender, age, race, ethnicity, language, diagnosis, severity, region, or frequency of HTC contact. Nationally, 26.4% rated insurance and 21.2% rated language as "always" or "usually" being problems when getting HTC services. **Why this matters:** Positive patient satisfaction with healthcare is associated with treatment compliance and better outcomes. But overall satisfaction with HTCs was unknown until now.

*For info:* "Patient Satisfaction with US Hemophilia Treatment Center Care, Teams and Services," [onlinelibrary.wiley.com](http://onlinelibrary.wiley.com)

## global

### His Boots Were Made for Hiking!

William Addison, a teen with hemophilia, made history this summer as the first known person with hemophilia to hike the entire Appalachian Trail (AT). He hiked 2,200 miles and raised almost \$27,000 for Save One Life! William now has only Mt. Katahdin in Maine to summit, to officially complete the AT. Listen to the Bloodstream podcast about his journey.

**Why this matters:** William, like many other physically active people with hemophilia, has successfully pushed the boundaries of what patients can accomplish with proper care and support.

*For info:* [bloodstreammedia.com/episodes/bloodstream-the-people-have-spoken-about-copays-and-sobbing-for-save-one-life](http://bloodstreammedia.com/episodes/bloodstream-the-people-have-spoken-about-copays-and-sobbing-for-save-one-life)



## Nuwiq Unique: New Indication



The US Food and Drug Administration (FDA) approved updated prescribing information (PI) for Nuwiq®, Octapharma's human cell line–derived recombinant factor VIII (rFVIII). Nuwiq is approved for preventing and treating bleeding in people with hemophilia A. Updated PI includes immunogenicity data from the NuProtect study in previously untreated patients (PUPs), the largest prospective study of a single rFVIII product in PUPs. **Why this matters:** Human cell lines can produce recombinant factor that's more similar to its natural counterpart, compared to factor produced by animal cell lines. This reduces the risk of inhibitor development—key in treatment decisions for PUPs.

For info: octapharma-us.com



## Quick and Simple Diagnostics

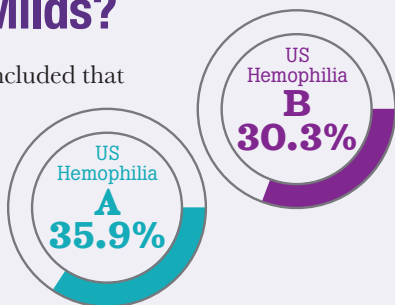
National Institutes of Health (NIH) awarded DNA Medicine Institute (DMI) a \$1.5 million grant to advance a quick, easy way of monitoring factor VIII and Hemlibra® blood levels in people with hemophilia A, especially in developing countries. DMI, an affiliate of rHEALTH, is developing a device that analyzes a blood sample taken by finger prick, within 15 minutes. **Why this matters:** The device prepares and analyzes a blood sample and sends results to an app via Bluetooth. Results can be shared directly with a patient's healthcare provider, reducing the need for in-person visits.

For info: rHEALTH.com

## Missing Milds?

A recent study concluded that about 35.9% of US hemophilia A and 30.7% of hemophilia B patients, mostly mild severity, remain undiagnosed and unaccounted for in current prevalence estimates. The study model was developed from a literature review and other secondary data sources by Takeda USA and PharmaMetrics investigators. Results were presented at National Hemophilia Foundation's (NHF) virtual annual meeting in August. **Why this matters:** Investigators concluded that improvements in disease management and longer life expectancy have led to an increase in the prevalence of hemophilia; but lower diagnosis rates for people with mild and moderate forms suggest room for more improvement in diagnosis and case reporting.

For info: "Incidence and Prevalence of Diagnosed and Undiagnosed Hemophilia A and Hemophilia B in Select Countries," academy.isth.org



## soundbites

- CSL Behring's plasma-derived factor IX product **Mononine** is being discontinued, although current supplies will be available until at least 2021.
- CSL Behring and Ferring Pharmaceuticals are recalling lots of **Stimate Nasal Spray** (DDAVP, desmopressin) distributed after January 10, 2018.
- Sanofi has voluntarily paused dosing and enrollment in the ongoing **fitusiran** clinical studies to allow investigation of reports of nonfatal thrombotic events in patients in the trials.
- The first patient has been dosed in the **Pfizer** and **Sangamo** phase III AFFINE study of giroctocogene fitelparvovec (SB-525), an investigational hemophilia A gene therapy that uses recombinant adeno-associated viruses as vectors to deliver the factor VIII gene to liver cells without triggering significant immune responses.

Novo Nordisk and Genentech have warned healthcare professionals that their respective hemophilia treatments, Rebinyn® and Hemlibra, can interfere with some common lab tests that measure blood coagulation. This is a medical concern in patients with **COVID-19** who experience abnormal blood clotting as a result of the disease. Both companies have provided tables of blood tests commonly used when diagnosing COVID-19 coagulation problems. The tables identify tests that may be affected by Rebinyn or Hemlibra, and also suggest alternative blood tests that are not affected by the drugs.



or higher than 10,000 BU.

An inhibitor titer less than or equal to 5 BU ( $\leq 5$  BU) is considered a low-titer inhibitor. An inhibitor greater than 5 BU ( $> 5$  BU) is considered a high-titer inhibitor. If you have a low-titer inhibitor, you can still use standard factor to treat bleeds, although in higher doses to accommodate for some of the factor being neutralized by the inhibitor. If you have a high-titer inhibitor, standard factor concentrates are not effective because all the factor is quickly neutralized after an infusion.

In addition to the inhibitor titer, inhibitors are categorized by how the immune system responds to infused factor. For some people, the inhibitor titer stays more or less stable and doesn't rise after the patient is exposed to factor. If your child has an inhibitor titer  $\leq 5$  BU, and it remains at or below 5 BU for several days after an infusion, he is a low responder.

For others, when factor is infused, the immune system quickly ramps up inhibitor production in an effort to neutralize the infused factor. This results in an increase of the inhibitor titer within four to seven days of exposure to factor, peaking within one to three weeks. This ramping up of inhibitors after factor

exposure is an anamnestic response (meaning a memory or recall response). If, after exposure to factor, the inhibitor titer rises above 5 BU over a few days, then your child is classified as a high responder. High-responding inhibitors are more challenging to treat than low-responding inhibitors because normal factor concentrates are useless with high-titer, high-responding inhibitors. Treating bleeds with these inhibitors requires special factor concentrates called bypassing agents, such as FEIBA or NovoSeven.

But there's one case where standard factor can be used to treat bleeds in high responders. In high responders, the immune system often produces fewer and fewer antibodies over time if it isn't exposed to factor. If someone hasn't been exposed to factor for several months, then the inhibitor titer may have decreased to a level low enough that normal factor concentrates may be used to treat bleeds successfully for a few days—that is, before the anamnestic response kicks in and the inhibitor titer increases again, making the factor ineffective. ☺

*In Part 2 of this series, learn about the risk factor for inhibitors, how common inhibitors are, how to treat bleeds when you have inhibitors, and how to eliminate inhibitors.*

#### *Richard's Review... from page 5*

- The Currence Brothers, *He's Coming Back to Earth Again* (Major Recording Studios, n.d.)
- *The Currence Brothers Sing and Play Gospel Grass* (Major Recording Studios, n.d.)
- The Currence Brothers, *Ballads and Instrumentals* (Major Recording Studios, 1978)
- The Currence Brothers, *Muddy Boggy Banjo Man* (Major Recording Studios, 1979)
- Woody Simmons, *All Smiles Tonight* (Elderberry Records, 1979)
- The Currence Brothers, *That's the Man I'm Looking For* (Augusta Records, 1981)
- *Clyde See with the Currence Brothers* (Augusta Records, 1983)

The Currence Brothers are known for their talent on the banjo and fiddle, not for having band members with hemophilia. Their hemophilia did not define them, and it never prevented them from making music. Of course, some accommodations were made—switching instruments due to elbow bleeds; not playing at some events or traveling as much as they wanted—but not at the expense of their artistry and passion. Today, other family members continue the Currence musical tradition. ☺

*To learn more, read profiles of the Currence Brothers and other West Virginia musicians in *Mountains of Music* (University of Illinois Press, 1999), edited by John Lilly.*



IN THIS TOGETHER  
Saturday, 7:18 pm  
Checking out a music  
festival with his girlfriend  
Marc, hemophilia A

Not an actual patient

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

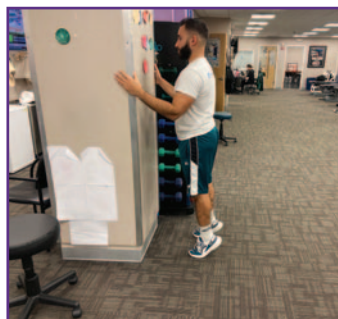
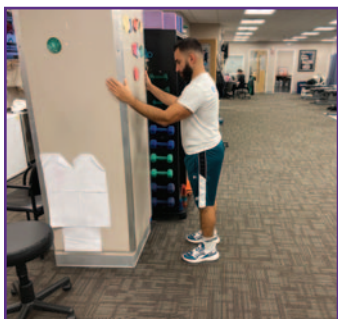
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## 6. Heel Raises



Stand facing a wall, with hands on the wall, toes pointed forward. Slowly raise your body up on tiptoes. Progress to not holding onto the wall, and further progress by standing on toes using both feet going up, and then lowering down on one. Start with 10–15 reps, and progress to 20–30 reps.

**Purpose:** Build strength through the calves, to improve strength during walking. Ankle bleeds are common, but ankle muscles are not commonly exercised. Having strong ankles will reduce strain that moves up through the knee, hip, and lower back.

## 7. Hip Flexor Stretch



Kneel on one knee, with the other at a 90-degree angle in front of you. Maintain upright posture, and keep your sternum/chest bone pointing upward. Slowly lean forward and feel the stretch through the groin. Begin by holding 15–20 seconds if you're under age 65, and holding 30–60 seconds if you're over 65, to help stretch the collagen. Progress by engaging the glutes to feel an increased stretch through the iliopsoas.

**Purpose:** Reduce strain on the quad and low back by improving mobility and flexibility of the hip flexor. Since most people are in a flexed posture and seated position for long periods, the iliopsoas tends to get super tight and is an area of increased muscle bleeds. Maintaining good range of motion will reduce the chance of target joints.

## 8. Hamstring Stretch



In a seated position, have your legs apart, and slowly lean toward one ankle. Begin by holding 15–20 seconds if you're under age 65, and 30–60 seconds if you're over 65 to stretch the muscle. Progress by reaching farther to improve the stretch.

**Purpose:** Reduce strain on the low back and knee joints by improving the mobility and flexibility of the hamstrings. If you spend a lot of time in a flexed posture and seated position, the hamstrings tend to get super tight. Maintaining good range of motion will reduce the chance of developing target joints.

A pandemic can make working out a challenge. These eight simple exercises focus on mobility, balance, and stability, and target all the major muscle groups. They can be performed at home without any equipment. I highly recommend performing these exercises daily, and after six to eight weeks, you'll see a major improvement. Exercise is vital if you have a bleeding disorder. The time to start is NOW! 🌱

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