Parent Empowerment Newsletter

LA Kelley Communications, Inc.

PEN

30 years anniversary

inside

Leadership at a Crossroads

What's the future for the bleeding disorder community?

Laurie Kelley

Our bleeding disorder community has awakened to an enviable—and somewhat uncomfortable—position at the dawn of 2020. We've achieved many successes over the past 15 years: continued access to treatment choices, collaboration with payers, social media presence, stronger global presence, advances in factor product development, novel therapies, and soon, the promise of gene therapy.

Yet we also find ourselves questioning what the future will be for our nonprofit organizations, their constituent involvement, and their leadership. In a remarkable coincidence, the leaders of our two main national organizations have resigned within six months of one another. Kimberly Haugstad, executive director of Hemophilia Federation of America (HFA), resigned in August 2019. Val Bias, CEO of National Hemophilia Foundation (NHF), resigned in December. Both served for over 10 years during a time of serious insurance threats to our community and rapid product innovation.

While unusual, a simultaneous change in leadership wouldn't be worrisome, except that we are on the cusp of massive, community-wide changes yet again. A novel therapy—Hemlibra®—is attracting customers away from traditional factor therapies. And the revenues from those therapies have traditionally supplied the funding our community needs. We wonder: Who will lead now? Who will steer our community and navigate the coming tumultuous waters? Do we even need two national organizations? Has the time come to consolidate? To answer these questions, we need to look at our community's history and future through the lens of leadership.

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welcome

I read a wonderful parable once. A traveler came upon three men working at a construction site. All three were doing the same job, but when the traveler asked the men what the job was, the answers varied. The first man said, "Breaking rocks." The second said, "Earning a living." The third said, "Helping to build a cathedral."

Leadership is more than just working hard. It's more than a job. It's creating a pathway to a vision. A vision is an ideal picture of the way things will be in the future. It's a guiding light, a beacon. And its picture should be captivating enough so that others will be compelled to follow. An effective leader must have a vision of where to lead people. And a plan of action. A Japanese proverb says, "Vision without action is a dream. Action without vision is a nightmare."

The bleeding disorder community has a fascinating leadership past, reviewed in this issue of PEN, that can serve as a model for other groups, businesses, and nonprofits. Our community has had great leadership success, fueled by the huge loss of life we suffered in the 1980s and '90s. And we've learned that leadership takes even more than vision: it takes communication, character, commitment, and collaboration.

In 2020, we're at a leadership crossroads. It's a time to reevaluate where our leadership has been—that stunning leadership that pulled us through our worst crisis—and where we're headed now. With two main national organizations, many chapters, and independent nonprofits, all facing huge changes in the funding landscape, the road ahead won't be easy. But finding the right leader or leaders can make traveling smoother. And those leaders must start with vision.

One exceptional leader in industry, Walt Disney, died before he could see the opening of Disney World in Orlando. On opening day, a reporter allegedly said to Roy Disney, Walt's nephew, "Too bad Walt isn't here to see this." To which Roy replied, "He did see it. That's why it's here." That's the power of vision, which our new generation of leaders will use to light the way to the next decade.

Laurie Kelley

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."

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Felicia Carbajal

Why I Choose Cannabis

The cannabis plant has been deeply engrained in American history since our country's inception. Commonly called medical marijuana and hemp, *cannabis sativa* has been used in everything from textiles and paper to medicines and spiritual tools. Although cannabis has been viewed as harmful or illegal, it has the potential to combat our nation's opioid crisis, repair some of the harm caused by the war on drugs, and offer Americans a natural alternative to pharmaceutical drugs.

Why do I believe so strongly that cannabis can do all of this? It's pretty simple: cannabis has been a wonder drug for treating my chronic pain from a spinal cord injury over a decade ago. It has also revolutionized the treatment of my depression, anxiety, and PTSD, and has numerous therapeutic effects when used in its various forms.

After my last back surgery, a microdiscectomy, I took Vicodin to manage the pain, but this would alter my mood and only mask the pain momentarily. I wasn't myself, and the pain always came back. My medical team said this would be my life. Between the epidural injections and physical therapy sessions, I began researching alternatives.

I tried everything to reduce my pain. I bought gadgets like seat and hand-held massagers, a laser acupuncture pen, and electrostimulation devices. I tried countless complementary therapies like chiropractic treatments and acupuncture. Up to that point, cannabis was last on my list of options. Fortunately, I was introduced to a world-renowned medical professional who was working with patients on low-dose cannabis options with controlled intake of THC. His team gave me a bottle of tincture to try. Three days later, I was pain-free, no longer needing Vicodin, and smiling.

This introduction marked the beginning of my journey with cannabis. I knew the power of sharing my story, and became even more intrigued by the potential of this plant as I weaned myself off a cocktail of anxiety, depression, and pain meds that caused more harm than good. I knew I had to keep learning.

First, I researched the legal history of cannabis. I grew up a DARE[1] evangelist during the 1980s and was unaware of cannabis's history in the US dating back over a century. Hemp was a valuable crop in the American colonies, used for a variety of purposes, including paper and rope. Eventually, it entered American pharmacopeia as cannabis and became a tool for advancing conservative agendas. Today, more than half the country has some form of regulated cannabis, and a majority of states allow the sale and transportation of hemp-derived products.

Next I explored the science of cannabis. I had friends who'd been diagnosed with HIV and AIDS and knew that this was their medicine, but I didn't understand why or how. I dove deep into the research and discovered the endocannabinoid system (ECS).[2]

The ECS is a network of neurotransmitters and receptors that work round-the-clock to help keep the body in homeostasis. Found throughout the bodies of mammals and other vertebrates, the ECS responds to the presence or deficiency of cannabinoids, which can be *endogenous* (produced within the organism) or *exogenous* (produced externally).

Endocannabinoids are produced internally and regulate the function of just about every physiological system within the body. *Phytocannabinoids* are endocannabinoids derived from plants, including but not limited to cannabis.

Naturally, I went to my physician and began asking about the ECS. To my surprise, she knew little about it. I shared some links from the National Institutes of Health (NIH)[3] and other research bodies[4] about current clinical trials and research.

I'm fortunate to live in a state with regulated cannabis, which means I have access to clean, tested cannabis products—a privilege I don't take lightly. I have the opportunity to explore other cannabinoids, including THCA (tetrahydrocannabinolic acid) and THCV (tetrahydrocannabivari) in conjunction with

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1. Drug Abuse Resistance Education 2. www.uclahealth.org/cannabis/human-endocannabinoid-system 3. nccih.nih.gov/health/marijuana 4. www.cancer.gov/about-cancer/treatment/cam/patient/cannabis-pdq

Paul Clement

Show Me the Numbers!

Hemophilia Is More Common Than We Thought

Hemophilia is a rare disorder, often estimated at 20,000 people in the US, or 1 in 5,000 male births. But based on recent work by researchers affiliated with the World Federation of Hemophilia (WFH), hemophilia, while still rare, is more common than we thought. Why and how did those numbers change?

Past Estimates of Hemophilia Cases

How do we estimate the prevalence of hemophilia? "Prevalence" means the total number of identified cases of hemophilia in a population, divided by the total number of males in that population at a given time. For more than 20 years, the most widely used estimate has been based on research from the US Centers for Disease Control and Prevention (CDC).[1] In 1993, the CDC established the hemophilia surveillance system (HSS) in six states. HHS was designed to identify cases of hemophilia and extract data from medical records. The CDC team identified 2,743 cases of hemophilia, estimated to include about 20% of the US hemophilia population. From this data, the age-adjusted prevalence of hemophilia in all six states in 1994 was 1 in 7,462 males.

Although prevalence tells us how many people are living with a disorder or disease, it doesn't tell us how many people were born with a disorder or contracted a disease after birth: this is called "incidence." Along with estimating the prevalence of hemophilia, the CDC researchers also looked at the incidence of hemophilia from 1982 to 1991. They calculated the mean incidence to be 1 in 5,032 live male births.[2] This last number has been the most widely used estimate for the incidence of hemophilia—and the most trusted—until now.

Incidence and prevalence are two different kinds of measurements. When used together, they can tell us how many people developed a disease and how many recovered. For example, the common cold may have a high incidence and a very low prevalence (meaning that many people developed the disease, but almost all recovered). Or in hemophilia, the incidence of inhibitors in hemophilia A may be as high as 30% (and about 3% for hemophilia B). But because many of these inhibitors are transient (temporary), and because others can be eliminated through immune tolerance therapy (ITT), the prevalence of inhibitors in hemophilia A (the number of people living with an inhibitor) is about 5% to 7%—a lot lower than the incidence.

In the case of lifelong genetic disorders that people are born with, the difference between the two numbers—incidence and prevalence—indicates not how many recovered, but how many died. To differentiate between disorders you're born with and those you can "catch," many researchers prefer the term "prevalence at birth" (or "birth prevalence") over the term incidence.

In a perfect world, where everyone with hemophilia is diagnosed and has top-notch care, and no one is harmed by having hemophilia,

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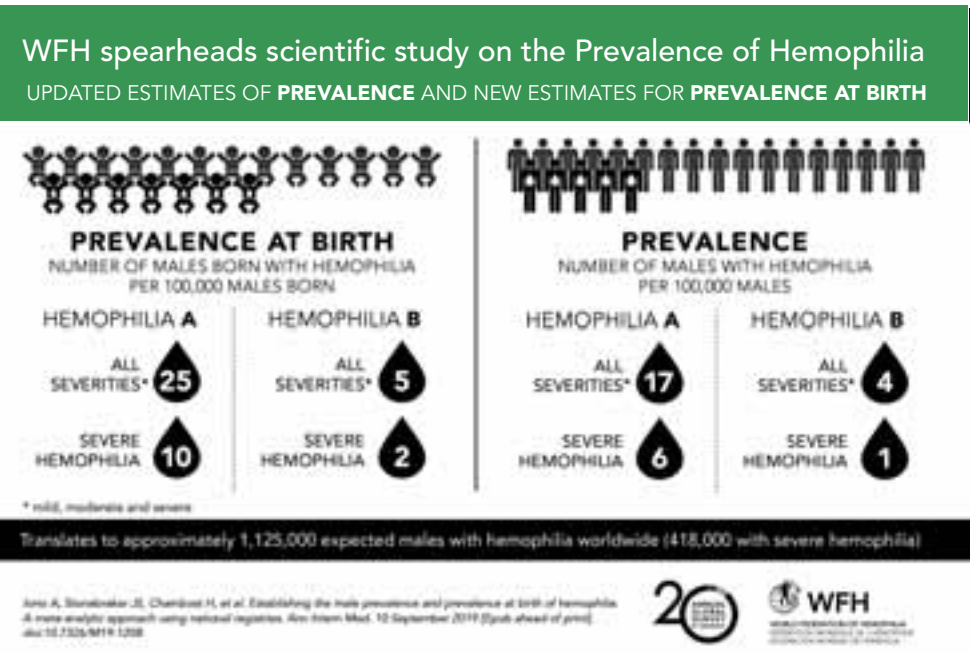

1. J. M. Soucie, B. Evatt, D. Jackson, and the Hemophilia Surveillance System Project Investigators, "Occurrence of Hemophilia in the United States," *American Journal of Hematology* 59 (1998): 288–94. 2. At the time, too few women were diagnosed with hemophilia, so they weren't included in the study.

richard's review

Linda Weaver's Studio

Richard J. Atwood

Television Doctors Treating Hemophilia

In the 1960s, highly rated primetime medical dramas contributed to the rising popularity of network television viewing. Between 1950 and 1980, there were 55 drama primetime series involving physicians on the three major networks (ABC, CBS, NBC). Many of these series had medical advisors on the set; plus, they had endorsements from medical societies. Viewers had high expectations for the accurate depiction of any medical problems, all embedded within a compelling storyline.

These television doctor half-hour or hour-long dramas eerily followed the same script-writing formula: the cast included an older physician and a younger physician, surrounded by an assortment of minor characters. Rather than foster a mentoring relationship, the older and younger doctors experienced dramatic conflict: for example, experienced versus neophyte, or humane versus scientific. The medical dramas also provided some mystery: Could the physicians correctly diagnose and successfully treat the difficult medical condition introduced each week?

Fictional physicians, portrayed by famous actors, treated patients with hemophilia in a few of these evening broadcasts. A critical look back at some of these television doctor dramas offers another perspective on the history of bleeding disorders.

Dr. Kildare

Frederick Schiller Faust, writing as Max Brand, introduced the fictional Dr. James Kildare in a *Cosmopolitan* short story in 1936. Subsequent serialized short stories about Dr. Kildare appeared in pulp magazines. Paramount released a Dr. Kildare movie in 1937. Then Metro-Goldwyn-Mayer bought the rights to the popular Kildare character, and released nine Dr. Kildare movies and six Dr. Gillespie movies between 1938 and 1947. Lew Ayres starred in the Kildare role, with Lionel Barrymore as the older Dr. Gillespie. Ayres and Barrymore continued their roles in the *The Story of Dr. Kildare* radio broadcast on the MGM network for 80 episodes from 1949 to 1951.

Radio was still a popular medium when the "Mysterious Hemophiliac Patient" episode was broadcast on May 18, 1951. At Blair Hospital, Dr. Kildare and Dr. Gillespie evaluate an anonymous patient who is found to have pyloric cancer, along with undisclosed hemophilia. The patient is an abdicated king from a small European country. Presurgical treatment includes ovarian extract capsules for two weeks, plus an intramuscular injection of 30 cc of fresh blood serum. During surgery, the patient receives direct blood transfusions. The hemophilia proves to be a greater problem than the cancer, but the patient successfully recovers from the surgery. This treatment for hemophilia was more appropriate during the 1930s, yet inappropriate in the 1950s when more effective blood products, such as fresh frozen plasma or Fraction I, were used.

Richard Chamberlain (left) and Raymond Massey

Richard Chamberlain as Jimmy Kildare, and Raymond Massey as Leonard Gillespie revived the *Dr. Kildare* series on television from 1961 to 1966. In 190 episodes during five seasons, NBC aired the award-winning MGM television *Dr. Kildare* series on Thursday nights. Apparently this popular Dr. Kildare never treated a patient with hemophilia.

Medic

NBC broadcast the award-winning television medical drama *Medic* for 59 episodes over two seasons, from 1954 to 1956. The 30-minute program was filmed in black and white. It was the first television medical drama to focus on medical procedures. Richard Boone starred as Dr. Konrad Styner. In "A Time to Be Alive," the episode broadcast on January 31, 1955, 11-year-old Davey Stinson falls off his bike and bumps his knee. Due to his hemophilia, Davey spends two weeks in the hospital, where he receives four pints of blood and a leg brace. Recovering at home, Davey falls and cuts himself with broken glass. Back in the hospital, Davey receives two units of antihemophilic plasma

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Leadership Redefined

Laurie Kelley

The bleeding disorder community is filled with admirable leaders. Though I often say that no one is a born leader, leadership can be birthed. In our community, leadership arose from the cataclysmic devastation when HIV was discovered in the blood supply in the 1980s and infected about half of our community. This horrific event birthed many leaders, whose legacies stand today.

When we think of those leaders—like Corey Dubin, Dana Kuhn, Val Bias, Jeanne White-Ginder, and Louise Ray—we think of certain leadership qualities: vision, authority, courage, commitment. These are leaders who made history and secured a safer future for us all.

Yet today, when you ask regular moms and dads and hemophilia patients about leadership, a slightly different picture emerges, highlighting different qualities. It helps to know more about what our community members seek today in leadership—who they admire, and what they expect from the leadership of tomorrow.

Types of Leadership

There are many types of leadership, but I usually peg leaders as one of three types: positional, situational, and transformational.

In a nutshell, positional leaders are usually elected, appointed, or inherited: think president, CEO, executive director, chief, even king or queen.

Situational leaders may or may not be positional leaders, but they rise to the forefront when a problem, event, or situation calls for a leader. Think first responder, rescuer, or anyone who steps forward to take action, like Lech Walesa of Poland in 1980, or US Army Major Hugh Thompson, Jr., during the Vietnam War. Or even Ripley in *Alien*!

Transformational leaders influence a community, industry, group, or era to make massive and lasting changes: think Jesus, Gandhi, Walt Disney, or Steve Jobs.

When asked about leaders, people often name leaders who fall into those three categories. Characteristics of these types of leaders? Powerful, authoritative, visionary, action-oriented, decisive, committed, communicative, innovative, influential, even charismatic. Do bleeding disorder families see leadership the same way?

The Softer Side of Leadership

Rather than describe leaders in our community as figureheads with power and influence, parents and patients associate softer qualities with leaders these days. These softer qualities include traits that make the "followers," or beneficiaries of leadership, feel more secure and feel their needs are being met. We polled parents and patients on Facebook to see what leadership qualities they look for most in the bleeding disorder community. And top among these qualities is *listening*.

Eva Bagay replied, "For me, a good quality of leadership is to have an ear to listen, to communicate. To protect their member." Krissy Pirehpour-Miller agreed. "Leadership is the ability to not just listen but truly *hear* you and your concerns. A leader inspires others and is someone who advocates passionately."

Dr. Anupama Pattiyeri of India added that a leader must be "a patient listener and motivator—someone who focuses on solutions and not on problems alone." Katelyn Popowich-Evans and Alicia Morado both put listening as their first sign of good leadership.

Alicia Morado

Wayne Cook remarked that leadership means "the ability to listen before deciding. Having ties to the community and leading with integrity, honesty and compassion." Karla Brown of Texas agreed; for her, leadership means "commitment to our community combined with a personal connection."

Tina Battillo noted that listening, combined with empathy, insight, and integrity, gives a leader the ability to understand life from a patient's or caregiver's point of view; the ability to understand important values of the community; and "the strength to stand up for those values." In other words, parents and patients view the traditional forms of leadership as less important than "servant" leadership.

Tina Battillo

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Leadership Types

There are many definitions of leadership. John Maxwell, famed author on leadership, says that leadership is "influence, nothing more, nothing less."[1] Well, yes and no. Leadership in the bleeding disorder community certainly means much more: it has been a figurehead in peaceful times, then tested through trial by fire. A better definition, for us, might be that leadership is the power to influence others to act toward a vision for the common good.

The successes of the bleeding disorder community have become a model to be followed by other chronic disorder communities. Throughout their history, bleeding disorder advocacy organizations have shown different types of leadership. To understand what type of leadership created our success, and what may be needed for the future, it helps to break down leadership into three basic types.

Positional leadership is appointed, earned, or inherited. The president of a country, CEO of a company, principal of a school, chair of a board of directors, pastor of a church, even king or queen, are examples of positional leadership. In the bleeding disorder community, a positional leader is the CEO of a nonprofit, pharmaceutical company, or specialty pharmacy; or the director of a hemophilia treatment center (HTC).

Situational leadership happens during an extraordinary event that often elevates someone suddenly to a leadership position that usually requires immediate action, sometimes at great risk. Malala Yousafzai, the young Pakistani who was shot while standing up for girls' right to be educated, is an excellent example of this. Situational leaders may or may not be positional leaders. Former New York City Mayor Rudy Giuliani was both. Though under fire for his role in the current administration, he was once hailed as a great situational leader in the aftermath of 9/11. In our bleeding disorder community, Louise Ray, mother of Ricky Ray, became a situational leader when her home was firebombed by neighbors in 1987 who wanted her and her three HIV-positive sons to move.

Transformational leadership is perhaps the most powerful of all. This leadership can begin through position or situation, but it usually creates lasting change, outliving its founder. A transformational leader's influence is sweeping: it changes history, alters social systems, affects culture, and impacts people's beliefs. Examples include most religious leaders, like Buddha, Jesus, or Mohammad; business leaders like Bill Gates or Thomas Edison; social leaders like Mother Teresa or Florence Nightingale; and political leaders like Abraham Lincoln or Nelson Mandela. In our global bleeding disorder community, Brian O'Mahony of Ireland, former president of the World Federation of Hemophilia (WFH), completely changed the scope, operations, and mindset of the WFH in the late 1990s, helping to make it the extraordinary nonprofit it is today.

The bleeding disorder community has shown all three styles of leadership, mostly due to its unique and tragic history. In many ways, its history has mirrored the three styles of leadership in stages, from positional to situational to transformational.

A Tale of Two Organizations

Our history starts with positional leadership and a vast vision. The Hemophilia Foundation (later changed to National Hemophilia Foundation) was founded in 1948 by Robert Lee Henry, a Long Island attorney whose son had hemophilia. Henry was inspired by an article he read about the suffering of a young boy with hemophilia who was not as fortunate as his own son. Henry's vision was to provide grants and donations for research and clinical study of bleeding disorders; to publish information related to bleeding disorder prevention and treatment; to provide medical scholarships; and to provide funds for affected families.

For decades, NHF was the only national voice for the bleeding disorder community and naturally became the advocacy leader. While treatment using whole blood advanced to fresh frozen plasma and then to cryoprecipitate, NHF monitored treatment research. A revolutionary breakthrough came in the late 1960s with the development of commercial factor concentrate, made from large pools of human blood plasma. This meant a dramatic change in lifestyle and improved quality of life for patients with hemophilia. Factor concentrate also signaled the end of being tethered to HTCs for constant treatment. And it eventually led to a leadership crisis.

Pharmaceutical funding was vital to NHF's survival. No one worried about this potential conflict of interest until a mysterious illness began affecting patients with hemophilia—an illness later called *acquired immunodeficiency syndrome* (AIDS). By the time the human immunodeficiency virus (HIV) was identified in 1984 as the cause of AIDS, about half the nation's estimated 20,000 people with hemophilia had already been infected, including about 90% of patients with severe hemophilia. In 1983, a potential conflict of interest started when NHF's leadership sent patients a letter recommending that they continue using plasma-derived factor concentrate until more was known about the situation. By the time scientists determined that HIV was in the nation's blood supply—and blood products—thousands of patients were infected with HIV from using contaminated plasma-derived blood-clotting products.

A situational leadership crisis developed. NHF's perceived lapse in leadership early in the AIDS crisis led to a desperate, raging outcry in the community from those who had lost loved ones to AIDS. And this led to the birth of two more national organizations. In the spring of 1993 in Alexandria, Virginia,

1. John Maxwell, *The 21 Irrefutable Laws of Leadership* (Nashville: Thomas Nelson, 2007), 13 (Kindle version).

15 people formerly affiliated with NHF met. Among them were patients Corey Dubin and Jonathan Wadleigh, as well as Jan Hamilton, a parent of a child with hemophilia. After the meeting, Corey and Jonathan traveled the country and met with various chapters to discuss their concerns about NHF leadership.

Situational leaders were born. Jonathan Wadleigh became founder and president of the hemophilia AIDS activist group Committee of Ten Thousand (COTT), which then became a vigilant watchdog of the nation's blood supply. And Jan Hamilton helped form a new nonprofit, the Hemophilia Federation of America. "At that first meeting" in Virginia, she says, "we decided there was a need for another national organization that would focus on family, and not on blood safety and technology. NHF seemed to focus on HTCs, the medical side, whereas we focused on community development and chapter development. We got patients themselves and families directly involved."

At first, HFA refused pharmaceutical donations as its funding source, believing that this would lead to conflict of interest and had possibly contributed to disastrous decision-making at NHF. So HFA accepted funding mostly from specialty pharmacies, whose revenue, of course, comes from pharmaceutical companies. HFA was more grassroots than NHF; it focused on providing direct services to families, and not research. HFA's first-year budget was $30,000 at a time when NHF's was in the millions.

Both NHF and HFA worked diligently in the 1990s to get compensation for families affected by HIV. Both emerged as powerful lobbying entities, and secured passage and funding for the Ricky Ray Hemophilia Relief Fund Act.[2]

Despite a common tragic past, the two nonprofits operated with some animosity for years. Eventually, as the HIV crisis passed, HFA's funding began shifting away from specialty pharmacies. In the early 2000s, HFA began to accept direct pharmaceutical funding. Both NHF and HFA agreed to collaborate, particularly on patient advocacy issues. It took until January 14, 2003, for NHF's CEO and executive director Richard Hellner to visit Jan Hamilton in Louisiana—the first time the NHF voluntarily visited HFA in a spirit of cooperation.

The outcome of the situational leadership of the 1990s? Today, both organizations are highly regarded and effective advocates for the bleeding disorder community. They are different organizations, with differing infrastructures, focus, resources, and most of all, differing visions. NHF is still viewed as primarily a medical and research-oriented nonprofit. HFA is viewed primarily as a grassroots nonprofit that benefits families more directly with assistance programs and education.

Both are funded through a combination of individual philanthropy and corporate sponsorships, government support, advertising revenue, and proceeds from special events. The majority of funding for both comes directly or indirectly from pharmaceutical companies. NHF is worth about $21 million, with a paid staff of over 80, and headquartered in New York City. HFA is a $5 million organization, with a paid staff of 20, and headquartered in Washington, DC.


Jan Hamilton

"Both organizations are similar in that both seek a cure, but I still feel that HFA's annual meeting is like a family reunion," notes Jan Hamilton. "Our information is imparted differently. We're aimed at families, and try to make information more understandable."

For example, NHF's Medical and Scientific Advisory Committee (MASAC) is the preeminent hemophilia medical authority in the world. MASAC defines policies and issues statements for treatment recommendations and standards of care. Its published advisories are sent worldwide. And HFA's Helping Hands program is a crisis management fund that alleviates daily life challenges by providing funds to prevent eviction, home repossession, or utility cut-off. Helping Hands was instrumental in supplying immediate assistance to families in hurricane-ravaged Puerto Rico in 2017, a great example of HFA's situational leadership.


Michelle Kim

More and more, given the changing needs of the community, these two nonprofits seek ways to collaborate. Michelle Kim, Esq., executive director of the Southern California Hemophilia Foundation, says, "I've been impressed with the way HFA and NHF partnered when they filed a joint amicus brief in the 5th Circuit Court of Appeals to combat the repeal of the ACA [Affordable Care Act], and other joint statements with regards to factor recalls. Their combined voice is powerful in the advocacy space."

The Changing Needs of Our Community

Both NHF and HFA were founded as community leadership organizations to address specific needs: one emerged as a positional leader to address research and treatment needs; the other as a situational leader to address the sudden needs of a traumatized community. As both continue to address the needs of the community, how have those needs changed over time? Understanding the changes might give us clues about the future of both organizations.

When NHF was founded, there was simply a need for a national patient organization, to give patients a voice. When HFA emerged, it gave voice to a segment that was ignored, and also addressed direct needs of families. Now, with excellent treatments available, with many bleeding disorder nonprofits

2. The Ricky Ray Hemophilia Relief Fund Act of 1998 established the Ricky Ray Hemophilia Relief Fund Program designed to provide compassionate payments to patients with blood-clotting disorders who contracted HIV through the use of antihemophilic factor administered between July 1, 1982, and December 31, 1987.

available to serve, and with global social media connections, our community's needs have changed.

Michelle Kim believes the primary need is to always ensure access to medication, which means access to insurance. "I am ever horrified," she says, "that insurance and access are guided by which state you live in." She notes that additional needs we must prepare for are "an aging bleeding disorders population, specifically regarding cardiovascular risk and senior living. We need to do a better job reaching out to communities of color, and those with cultural and language barriers and those living in rural areas. Finally, there has been a lot of discussion recently with regard to mental health. We have tools to identify if someone is having mental health issues, but we lack resources to provide relief."

Jan Hamilton also cites several changing needs. "I'm very concerned for continuing or bettering relationships with FDA and CDC,[3] so that if we ever are faced with another pathogen, we are able to address it immediately and with the cooperation of government. We need community members to sit on government advisory boards."

Jeremy Griffin, executive director of the New York City Chapter, cites HTC long-term viability; mental health and [opiate] addiction concerns; and research to improve standards of care guidelines.

Jeremy Griffin

Val Bias, former CEO of NHF, notes, "Transition to gene therapy will be important; patients will be cautious, and it won't happen overnight. We need to educate patients about gene therapy. And gene therapy, once available, will affect the HTCs with 340B programs.[4] Patients will still need a strong treatment network. A reduction in clotting factor units may impact HTCs and chapters. The 340B operating revenue is built on clotting factor unit sales. Patients who use a lot of factor, like those with severe hemophilia and inhibitors, will gravitate to novel therapies. Using less factor and more novel technology will hit HTCs financially."

Val Bias

We must take change by the hand, or rest assuredly, change will take us by the throat.

—Winston Churchill

An Earthquake Changes the Landscape

Indeed, novel therapies and gene therapy, both considered desirable and revolutionary, will permanently alter the bleeding disorder landscape—leading to a massive shift in funding for both HTCs and nonprofits. This has already begun to happen, as some patients shift from factor concentrate to Hemlibra. With most community-support funding traditionally coming from factor manufacturers like Aptevo, Bayer, CSL Behring, Octapharma, Pfizer, Sanofi-Genzyme, and Takeda, newcomers like Genentech, Biomarin, uniQure, and Spark will cause a seismic shift. We'll see some companies fading away, others being acquired, products being consolidated, and only a few manufacturers left to dominate the market in bleeding disorders. These changes will cause a scramble for funding.

Rich Pezzillo

Rich Pezzillo, person with hemophilia and executive director of New England Hemophilia Association, agrees. "Diversification of funds is paramount. Many of the bleeding disorder advocacy organizations rely on industry support. To sustain our mission, we must diversify funding through outside-of-industry funding, donor retention, increased fundraising, major gift donations, and new methods on how to better share our story with the general public. The public only hears that treatment is expensive, and there is no cure. And I hear from colleagues that fundraiser results are decreasing due to treatment advances because we are losing our story, but it doesn't have to be that way."

Jan Hamilton adds, "Funding is under threat. Chapters are getting less funding from manufacturers. Specialty pharmacies provide some, [but] less and less each year. Chapters need to do more fundraising on their own. I heard over the last few years that chapters thought about funding shortages that 'this too shall pass,' and funding will go back to the way it was. But the number of home care companies and specialty pharmacies is so small now." More scrutiny and tighter control by insurance companies left bleeding disorder specialty pharmacies reeling; with squeezed profit margins, many specialty pharmacies faced the inevitable—selling their businesses. Large pharmacies have continued to buy smaller ones in a feeding frenzy, and the numerous acquisitions and consolidations mean fewer sources for soliciting donations.

Rich Pezzillo takes it a step further than just funding concerns. "Speaking from the local, state level, if we don't change the funding landscape, we will become threats to one another—eating at each other for lack of funding." Jeremy Griffin agrees. "This

3. US Food and Drug Administration (FDA) and Centers for Disease Control (CDC). 4. About 80% of the approximately 144 US HTCs generate additional revenue by selling factor. They participate in a federal drug discount program called the 340B Drug Pricing Program. Section 340B of the Public Health Service Act (PHS) was enacted in 1992 as part of the Veteran's Health Care Act and is managed by the Health Resources and Services Administration (HRSA), the same agency that manages the HTC hemophilia grants. The 340B program requires pharmaceutical manufacturers who participate in Medicaid programs to discount prices for covered outpatient drugs purchased by specified government-supported facilities, or "covered entities." The discounted price is called PHS pricing or 340B pricing. These covered entities can then resell the drugs to patients at a markup, keeping the revenues to help support services and programs.

decline in national funding is likely to increase competition between the two main organizations and potentially further divide the community and create increased redundancy."

Does It Take Two?

Given the concern about shortages of funding due to novel therapies and the promise of gene therapy, is there a need now for two national organizations? The original goals—providing a national voice, backing research into therapies, and focusing on direct patient needs—seem to have been largely met. If funding is shrinking, why have two expensive organizations for a hemophilia population of roughly 20,000 people?

Thoughtfully, Jeremy Griffin compares the two organizations before commenting on whether they should merge. "Our community is lucky to have two incredible national organizations working to improve care and quality of life for people with bleeding disorders. Both organizations offer significant value and each has their niche. NHF has a history of driving research, setting treatment guidelines, ensuring access to care, leading policy initiatives, and improving resources for local chapters. NHF is also different from HFA because it directly involves the treatment community at its meetings with sessions for doctors, social workers, physical therapists, and nurses."

Griffin continues, "HFA also has an incredible history and a rich family feeling for an organization. It was built by the people for the people. HFA is known for its direct patient assistance and creating incredible programs for chapters to expand education across the country. More recently HFA has taken a larger leadership role in policy, research, and preserving our collective history."

Jan Hamilton believes there is a need for both organizations. "NHF is huge. HFA is growing. I can't imagine one organization so big that it handles every issue well. A combined organization would lose the personal touch. HFA was founded for the patient and their family. I don't see a merger as success."

Val Bias agrees. "How do you merge into one when one is five times bigger than the other? That's an acquisition. If both merged now, HFA would cease to exist." Michelle Kim adds, "I practice merges and acquisition law, and there is always a deep concern about the intersection of corporate cultures of merging organizations. This would be a challenge for the new CEO, given the differences between NHF's and HFA's culture."

Val argues, "There is a place for HFA, and what they do and how they do it. I'm biased because I come from the HIV era, where it was better to have two voices. If something happens and you have only one point of view, does that serve the community as whole?"


Brian Andrew

The idea of having two voices is essential to the bleeding disorder community. Brian Andrew, chair of NHF's board of directors, adds, "The important thing to think about is that we're a community-based group of organizations. It's a large enough community where there are different voices and different opinions about what's important. Not surprisingly, these voices differ from time to time. What's important is when there's an opportunity to have a louder voice, two organizations is fine, if the message is consistent, like to policy makers."

Rich Pezzillo says, "As a chapter leader, there is value as being a member of both. Both provide tools and skills needed for local organizations to be successful. NHF provides a lot of support for chapters, for board retreats, staff development, grant opportunities, advocacy collation and building grants. And NHF has MASAC—we rely heavily on MASAC recommendations. NHF hosts Washington Days, the largest advocacy forum. HFA does a great job with family support. It provides programs like Dads in Action and Blood Brotherhood. For advocacy, both have done an incredible job of working together around product safety concerns, and legislative action alerts."

Jeremy Griffin wonders, "If we were to build the perfect solution from scratch today, would we build it as two organizations? Probably not. It's hard to say the perfect route forward. We need a community working group that will do a thorough, independent analysis of the value of having a merged organization versus two independent organizations. All stakeholders need to be heard. The reality is that we have two national organizations that provide tremendous value, each with their subspecialties. It is also a reality that as both organizations have independently evolved, more of their programs have begun to overlap or at times even compete. Now don't get me wrong, I still believe both organizations offer unique things to the community, but there is definitely some redundancy. With funding changes and decreasing budgets it will be important to be more strategic about the future."

Rich Pezzillo agrees. "I'd love to see an assessment of programs, to weed out any overlap and say to the community, here are the programs, NHF does this one, and HFA does another. We could do a needs assessment in five areas: chapter support, funding, advocacy, research, and programming. What do both organizations do well, where is there overlap, and where can we work nationally or locally together? We must be forward-thinking now. As a community we are going to be forced to be reactive, and now, today, we have an opportunity to be proactive."

Michelle Kim says, "One of my favorite quotes is by Abraham Lincoln—'A house divided against itself cannot stand.' I hope we can learn to all work together to create a unified, impactful and influential group for our rare disease community."

Type of Leadership Needed?

Regardless of whether both national organizations stay separate or merge, a new leadership style may be needed for the future. Not just positional, not occasionally situational, but perhaps completely transformational, to make permanent and wide-ranging changes to our beliefs, our culture, and our vision.

Val Bias stresses the importance of listening to constituents—families, the medical community, chapters, and industry. "In my early years as CEO, I traveled 200 days each year to build relationships and have visibility. Staff were used to an executive director being in the office all the time. You can't lead from a distance. You need to talk to people, be with them, care for them, and listen to their stories."

Brian Andrew adds, "We must take into account different constituencies. Historically, we had an arm's-length relationship with payers, but we had to engage them more, to understand what they think. Same thing with policy makers; same with pharmaceutical companies. We need to listen at different levels, and use the information gathered to envision what things might look like in three to five years."

Listening was mentioned by all leaders interviewed here, as a required skill for the next leaders. Michelle Kim believes, "Because my job can be challenging, with many different stakeholders with differing opinions, I try to follow three principles: (1) Have a vision for the organization which is guided by our mission statement; (2) Lead by humility and an attitude of servitude; and (3) Create a culture of integrity both in the office among the staff and board and within the community. I would love to see a leader who is a team and community builder, and a great listener who can unify the community while considering the vast needs and differences among all of us."

Jeremy Griffin has a broad view of our community's leadership and where it might be headed. "Technology has been the biggest driver in how our leadership has changed. Old models valued hierarchical structures to define clear decision-making roles. Now technology allows power structures to be more decentralized and data to drive the process. Leaders of the past needed to know all the answers; now leaders need to listen and synthesize the answers. There is a much greater need for leaders to be consensus builders, and able to make adjustments rapidly as the pace of change quickens. The most successful leaders are the ones that can sift through these vast data sources and feedback loops to recognize patterns, identify emerging needs, and build responsive solutions."

If there continue to be two organizations, Rich Pezzillo adds, "Collaborative leadership is essential for any organization. Working together on key pillars, like advocacy, only strengthens a community. Going forward, through this changing landscape, it's vital that both organizations are forward-thinking, and have new CEOs who collaborate, listen and adapt to evolving needs."

Our leaders' most-admired leaders

Jan Hamilton →	David Ford, endowed chair, University of Alabama
Val Bias →	Malcolm X, Abraham Lincoln
Brian Andrew →	Admiral James Stavridis
Michelle Kim →	Maya Angelou
Rich Pezzillo →	Martin Luther King, Jr.
Jeremy Griffin →	Thomas Jefferson

You never change things by fighting the existing. To change something, build a new model that makes the existing model obsolete.

—Buckminster Fuller

The Future Belongs to the Young

One of the tenets of effective leadership is to develop leaders, so that the vision and mission live on long after the founder and current leaders are gone. For the bleeding disorder community, this means engaging youth in programs, community fundraising, and decision-making, and directly mentoring them. There is a threat to our future leadership now, as we have become more successful. With effective home treatment and responsive advocacy, youth may have drifted from the community to lead independent lives, just as we had always hoped. In doing so, they stop visiting their HTC, local chapter events, and national meetings.

It's long been recognized that youth need to be engaged and mentored. NHF established the National Young Leaders Institute (NYLI), a two-year program for community members aged 18–25. Val Bias believes it's an essential program for the community's future. Brian Andrew adds, "The graduates of NYLI are amazing! They are articulate, passionate, and understand the issues."

Rich Pezzillo was such a graduate and now is a community leader. "NYLI is a great program. I would not have imagined myself running a chapter, but by getting involved as a teen, it's allowed me to appreciate the value of leadership. NYLI taught me more than any textbook. We need to invest in teens, and empower their parents to know that even with treatment advantages there is still a need for them to be active." Rich was

also a graduate of Bayer's Leadership U,[5] a comprehensive and paid leadership internship for the bleeding disorder community with proven results producing future leaders.

Val Bias offers another solution: approach kids when they're young. Val was director of a camp in Northern California for young people with bleeding disorders. "When I experienced that older kids at camp didn't want to learn leadership, we started with 12-year-olds." He advises continuing youth leadership going forward. "Make camps the draw point, and maybe do three youth program summits in the summer."

Michelle Kim agrees, adding, "Whenever possible, we need to give our youth an opportunity to be heard and to be responsible regardless of whether they succeed or fail. They will not know about their potential unless they are given an opportunity, and programs such as Washington Days, local organization leadership opportunities, and involvement can help foster these leaders."

Jan Hamilton wonders, "If there were better or more frequent leadership workshops around the country, at the chapter and national level, and graduates could be on a master list of those who could be drafted for certain positions, that might be a way to develop leadership."

Rich Pezzillo adds, "We need some sort of summit of leaders of chapter, industry, and advocates. We all have the same mission, and we need to envision what our future could be."

Vital to the future is remembering the past. Leaders must be able to share the unique and devastating story of hemophilia and HIV, and of victory and a future of promise. Rich says that a pressing need is storytelling: "In an evolving landscape, we as community must learn how to effectively share our story. Our story doesn't change because we have new advancements. Storytelling is essential for success. We can't lose our history, we can't lose where we are now, and we can't lose where we are going."

Jeremy Griffin summarizes the crossroads of leadership, where we stand now. "The landscape for our community is changing rapidly; this holds immense possibility and risk at the same time. The highest current need I believe is a bold, visionary leader to navigate these rapid currents. Our two largest national organizations are in transition, and both need leaders to unify our vision for the future." A transformational leader can carry the bleeding disorder community's past into our present, respect and listen to its many constituents now, and assess changing needs. Such a leader will be visible, caring, and strategic, to map our future with a new vision into a new world.

5. Visit www.livingwithhemophilia.com to learn more.

Headliners

MANUFACTURER

Particle Problem

Particles were found in some production batches of Hemlibra®, which consist of the drug protein substance and silicone oil, a nontoxic, organic polymer included in all IV and injected medicines. Genentech alerted health authorities in March 2019: US FDA, European Medicines Agency (EMA), Swissmedic, Health Canada, and Japan's Ministry for Health, Labour and Welfare (MHLW) all agreed that the benefit/risk profile of Hemlibra remains unchanged, and have supported the continued distribution of Hemlibra to patients. National Hemophilia Foundation's Medical and Scientific Advisory Committee (NHF's MASAC) issued its own statement recommending no change in prescribing or using Hemlibra. **Why this matters:** Particle contaminants in injectable drugs could potentially harm patients, but the risk from subcutaneous administration is considered lower compared to intravenous injectables.

For info: hemlibra.com

Immune Tolerance with a Twist

US clinicians at Emory University reported successful use of a novel protocol ("Atlanta Protocol") combining immune tolerance induction (ITI) with Hemlibra prophylaxis in seven pediatric patients with severe hemophilia A and inhibitors. Using ITI with either recombinant or plasma-derived factor VIII products and Hemlibra achieved a negative inhibitor titer in three patients, and a normal factor VIII recovery (≥66%) in two patients. Four patients experienced a total of nine bleeding events, but there were no thrombotic events in any patient. **Why this matters:** ITI while on Hemlibra prophylaxis is a realistic approach in pediatric patients with inhibitors, according to researchers.

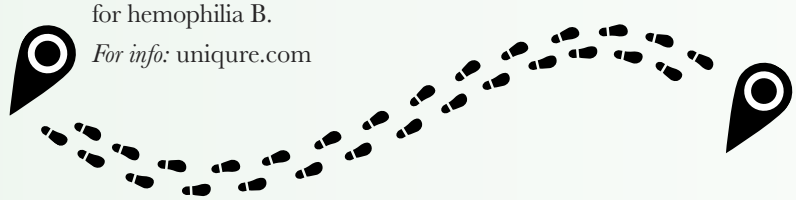
For info: Haemophilia, September 2019

SCIENCE

One Step Closer

UniQure achieved planned enrollment of 56 patients in its HOPE-B phase III trial of etranacogene dezaparvovec (AMT-061), an investigational adeno-associated virus (AAV5)–based gene therapy incorporating the company's patent-protected Padua variant of the factor IX gene for treating patients with severe and moderately severe hemophilia B. AMT-061 has been granted Breakthrough Therapy designation by the FDA. Interim results of uniQure's ongoing phase IIb study showed that a single administration of AMT-061 resulted in sustained increases in factor IX levels up to 54% of normal, with a mean factor IX level of 45% of normal. During this period, no phase IIb study patient reported any bleeding events or required any infusion of factor IX replacement therapy. And no patient experienced any material loss of factor IX activity. **Why this matters:** UniQure's successful phase III trial brings us one step closer to a gene therapy for hemophilia B.

For info: uniqure.com



A Sub-Q IX?

The first two subjects have completed dosing in a phase IIb study of dalcinonacog alfa (DalcA), an investigational next-generation subcutaneously administered recombinant factor IX therapy for severe hemophilia B. Catalyst Biosciences reported circulating factor IX levels exceeded the desired goal of >12% with no detectable anti-drug antibodies. Patient enrollment is ongoing, and Catalyst anticipates reporting final data in the first half of 2020. The trial is expected to enroll up to six subjects, who will receive a single intravenous dose followed by daily subcutaneous doses of DalcA for 28 days. **Why this matters:** This subcutaneous factor IX, when administered daily, boosted factor levels to 12% to 30%, significantly reducing the chance of spontaneous joint bleeds.

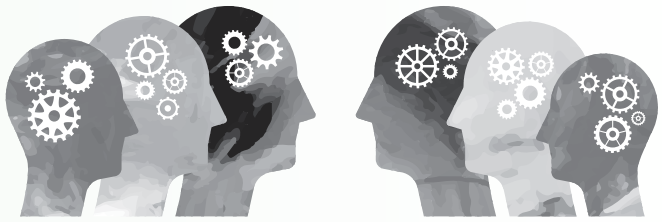
For info: catalystbiosciences.com

GLOBAL

Flying Factor

California-based Zipline will increase the number of remote hospitals and health centers it supplies with blood delivered by unmanned drone craft from 25 to 100 facilities in Rwanda by 2020. Zipline's drones can fly over 99 miles at 62 mph, carrying 3.7 lbs. Drones drop the blood or other medical supplies by parachute. Since the company's inception, Zipline drones have made about 16,000 flights to supply blood and medications to Rwandan health facilities. **Why this matters:** Drones may one day deliver factor to patients in rural areas of developing countries.

For info: flyzipline.com



First Gene Editing for Hemophilia?

Novo Nordisk and Bluebird Bio began a three-year research collaboration to develop next-generation in vivo genome editing treatments for genetic diseases, including hemophilia. The two companies will focus on identifying a gene therapy candidate to help people with hemophilia A permanently end factor replacement therapy. The companies will use Bluebird Bio's proprietary mRNA-based "megaTAL" technology, designed to provide a highly specific, efficient way to silence, edit, or insert genetic components. **Why this matters:** Many factor manufacturers, under pressure as Hemlibra continues to capture market share, are entering gene therapy trials for possible products to sell in the future.

For info: bluebirdbio.com

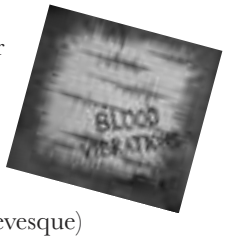
Daily Sub-Q in Trials

A phase II clinical trial evaluating prophylactic use of Catalyst Biosciences' subcutaneous factor VIIa variant marzeptacog alfa (activated) (MarzAA) significantly reduced the annualized bleeding rate (ABR) in patients with hemophilia A or B with inhibitors. Daily subcutaneous administration of MarzAA for 50 days at each patient's final dose significantly reduced the mean six-month ABR from 19.8 to 1.6. The median ABR was reduced to zero during treatment, with seven of nine subjects experiencing no bleeds, either traumatic or spontaneous, at their final dose level. There was no evidence of antibody or inhibitor formation. MarzAA has been granted Orphan Drug designation by the FDA for routine prophylaxis to prevent bleeding episodes in patients with hemophilia A or B with inhibitors. **Why this matters:** If this drug makes it to market, it will be another sub-q option for people with hemophilia and inhibitors.

For info: catalystbiosciences.com

PATIENT PROGRAMS

Picking Up Good Vibrations



The talented musicians at Blood Vibrations have released another album! The album debuted at the Blood Vibrations Listening Center as part of the Blood Work exhibition by FOLX (run by community member Justin Levesque) during NHF's Bleeding Disorders Conference in Anaheim in October. Anyone is welcome to submit a song for an upcoming album. Blood Vibrations does not request or accept funding, and doesn't charge artists to submit work or stream the music. **Why this matters:** Art and music are personal ways that people with bleeding disorders can express their experiences and feelings about their condition.

For info: bloodvibrations.bandcamp.com

MyBDC

My Bleeding Disorder Community (MyBDC) is a registry of de-identified aggregate data on patients with bleeding disorders. "De-identified" means that personal identifying information has been removed. MyBDC is managed by NHF. About 350 people are enrolled, including 165 affected people. To enroll, patients must take a baseline survey once, and then update surveys yearly, helping researchers look for bleeding patterns. MyBDC will help identify the community's research priorities and encourage participants to become active partners in their healthcare. **Why this matters:** This community-powered registry will help researchers understand what it really means to live with a bleeding disorder and how current treatments, therapies, and policies affect the community.

For info: hemophilia.org

SOUNDBITES

- A recent survey showed that 80% of patients don't know what gene therapy is. Check out **hemdifferently.com**, sponsored by Biomarin, to learn about gene therapy.
- Aptevo is introducing a new **3,000 IU vial size** of Ixinity (recombinant factor IX), adding to its assay sizes in 250, 500, 1,000, 1,500, and 2,000 strengths.
- Of 586 severe hemophilia A patients at the **German HTC in Bonn**, those with a family history of inhibitor development had an almost six times higher risk of developing inhibitors. Altogether, 19% of Bonn patients with severe hemophilia A had developed inhibitors.
- **Sigilon Therapeutics** received Orphan Drug designation for SIG-001, a novel encapsulated cell-based therapy to treat hemophilia A. Sigilon expects to begin clinical trials in the first half of 2020.
- The FDA approved **Octapharma's** wilate (plasma-derived von Willebrand factor/coagulation factor VIII complex) in adults and adolescents with hemophilia A for routine prophylaxis and on-demand treatment of bleeding.
- Israel's emergency medical service, Magen David Asom (MDA), is building the world's **first underground blood bank** that is safe from missiles, chemical and biological attacks, and earthquakes.
- Including recombinant versions of therapeutic plasma proteins, the 2018 US plasma proteins market totaled just over **$16.2 billion**, up 10.0% from 2017, according to a new report from the Marketing Research Bureau.
- Over an 18-month survey period, the use of **extended half-life (EHL)** factor VIII and factor IX concentrates increased from 10% of patients to 22% of patients at US hemophilia treatment centers affiliated with the American Thrombosis and Hemostasis Network (ATHN).
- Two patients who received a single infusion of FLT180a, an investigational adeno-associated virus hemophilia B gene therapy from **Freeline Therapeutics**, had a rise in factor IX levels to 40%, which remained stable for over a year.

New Mental Health Services for Bleeding Disorders

Wholeness Integrated Solutions (WIS) provides motivational and educational services and presentations to individuals, patient groups, couples, families, and professionals. Participants can learn about and discuss topics central to the eight integrated categories of whole living, under the biological, psychological, social, spiritual, nutritional, financial, educational, and environmental pillars of integrated wholeness. As participants learn, they develop new understandings and also tools that they can immediately apply.

Founder Matthew D. Barkdull, a person with hemophilia, is a licensed marriage and family therapist, certified medical family therapist, certified WholeFIT coach, and licensed financial adviser. He holds a master's of science, and an MBA with emphasis in strategic management. Matt has 20 years of experience in the social service, mental health, medical health, patient advocacy, and training/motivational speaking industries.

For info: 385-434-8136 or barkdullmd.wholeness@gmail.com

As I See It... from page 3

terpenes, aromatic plant essences found abundantly in cannabis that can provide therapeutic relief and enhance the efficacy of other compounds when combined (the "entourage effect").

For those who are new to cannabis or who live in unregulated areas, take this chance to educate yourself. Check out sites like Project CBD,[5] GreenFlower Media,[6] and Leafly,[7] and dive into the data. Go to the NIH website[8] and type "cannabis" along with your condition to review the research. The reality is that cannabis is personalized medicine, and the one-size approach won't work for everyone.

My biggest recommendation: When you explore cannabis, be safe. The cannabis industry is in its infancy; with recent reports of cannabis-induced health concerns, it's imperative to purchase from a licensed, regulated producer. You should be able to view the lab test results of any product you buy, so you know exactly what you're putting in your body. There are lots of options everywhere, including websites like Amazon, so be mindful! And if possible, test what you're using.

5. www.projectcbd.org 6. green-flower.com 7. www.leafly.com 8. nih.gov

Finally, make sure you're aware of the laws and regulations in your area. This is essential as more and more states regulate.

After years of taking opioids, I'm finally free. Cannabis can improve the quality of your life, too. Understand and explore the possibilities of cannabinoid therapies. Together we can fight the stigma and perception surrounding cannabis, save countless lives from opioid-related overdoses and deaths, and heal the harm from the war on drugs by voting for sensible drug policy.

Felicia Carbajal is a values-based community organizer, social entrepreneur, change-maker and innovator in the cannabis industry. Based in Los Angeles, the cannabis capital of the nation, Felicia has over two decades of experience in California's cannabis market. Felicia has worked with world-renowned cannabis medical professionals, has consulted numerous cannabis brands, and is a trusted resource for multiple patient and consumer communities. Currently Felicia is executive director of the Social Impact Center: felicia@thesocialimpactcenter.org.

Inhibitor Insights... from page 4

then the prevalence at birth and prevalence estimates would be very close. In reality, prevalence is always lower than prevalence at birth. In wealthy countries, where care is more available, the difference between prevalence and prevalence at birth is smaller than in poorer countries. And the poorer the country, the greater this difference becomes. So the difference between these two numbers can be used as a marker for the level of healthcare of a country: the closer they are, the better the level of healthcare.

New Research

What has changed? We now have new data, which will undoubtedly replace the prevalence estimate from the 1998 CDC study. In September 2019, researchers working with the Data and Demographics Committee of the WFH published their work on the prevalence, prevalence at birth, and life expectancy disadvantage of people with hemophilia.[3,4]

The committee's work uses data from several large national patient registries containing high-quality data on many thousands of patients; this gives a more accurate estimate of the prevalence of hemophilia than is possible with smaller studies, such as the 1998 CDC study. Data came from the national patient registries of Australia, Canada, France, Italy, New Zealand, and the UK. Data from US registries—the CDC's Universal Data Collection (UDC) and newer Community Counts surveillance project—was not used because these studies cover only patients seen at hemophilia treatment centers (estimated at less than 70% of US hemophilia patients). Also, the CDC uses a low cutoff—30% factor level—in its definition of mild hemophilia; this would result in a lower prevalence of mild hemophilia as compared to prevalence in other countries.[5]

Show Me the Numbers!

So what did WFH researchers find? They determined that prevalence at birth, per 100,000 males, is 24.6 cases for all severities of hemophilia A; 9.5 cases for severe hemophilia A; 5.0 cases for all severities of hemophilia B; and 1.5 cases for severe hemophilia B (see infographic). Combining the prevalence of all severities of hemophilia A and B gives a total of 29.6 cases per 100,000 males, or 1 in 3,378 male births. This makes hemophilia *one-third more common* than we'd thought.

It also means that many more people are born with hemophilia worldwide than we'd thought. The WFH's previous estimate was about 400,000 people worldwide with hemophilia. We now know that this estimate is too low. The WFH researchers now estimate that 1,125,000 males have hemophilia worldwide, including about 418,000 with severe hemophilia.

Sadly, fewer than 200,000 patients have been identified globally; most patients have not been diagnosed. And most people with hemophilia have a "life expectancy disadvantage"—they tend to die younger than their peers without hemophilia.

3. A. Iorio, J. S. Stonebraker, H. Chambost, et al., for the Data and Demographics Committee of the World Federation of Hemophilia, "Establishing the Prevalence and Prevalence at Birth of Hemophilia in Males: A Meta-analytic Approach Using National Registries," *Annals of Intern Medicine* 171 (2019): 540–46. 4. The WFH has a copy of its October 9, 2019, webinar on the study available for download: https://news.wfh.org/wfh-holds-webinar-titled-updated-estimates-of-prevalence-and-prevalence-at-birth-of-hemophilia/ 5. Most of the world defines mild hemophilia as being 6% to 40% factor level, while NHF and HFA use a range of 6% to 49% in their definition of mild hemophilia.

This means that although there *should* be 1,125,000 males worldwide with hemophilia, there are really many fewer, because of premature deaths.

> *Prevalence at birth* (similar to incidence) means the number of people born with hemophilia.
>
> *Prevalence* tells us how many people with hemophilia are still alive at a certain point in time. For hemophilia, prevalence is always lower than prevalence at birth.

The difference between these two estimates is an indicator of the level of healthcare in a country. And that's directly tied to income: high-income countries have a higher prevalence of hemophilia, meaning better access to care and fewer people with hemophilia dying. But even in wealthy countries where patients have access to treatments, there's a major life-expectancy disadvantage, or difference between how many men with hemophilia should be alive and how many are actually alive: 30% premature deaths for hemophilia A, 37% for severe hemophilia A, 24% for hemophilia B, and 27% for severe hemophilia B.[6,7] In lower-income countries where there is little or no treatment for bleeds, the life-expectancy gap is dramatic: 64%, 77%, and 93% premature deaths for countries with upper-middle, lower-middle, and low incomes, respectively.

From Information to Insights

These new numbers for prevalence and prevalence at birth are the first step in determining the "burden of disease"—that is, the cumulative effect of a disease, including health outcomes such as morbidity (medical problems caused by having a disease) and mortality (deaths); social aspects; and costs to society. The new prevalence numbers were based on data from wealthy countries where high-quality healthcare is available, but there is wide variation in the reported prevalence of hemophilia between wealthy and poorer countries. This indicates two things: (1) the inability of healthcare systems in low-income countries to diagnose hemophilia; and (2) how little access to care these people in poorer countries have (meaning more premature deaths). This shows the huge healthcare challenges faced by developing countries.

These new, powerful numbers can also be used strategically by the WFH and other advocacy groups to lobby governments to provide treatment and services for people who lack them. The numbers give a snapshot of the progress a country makes to reduce its people's suffering. Carly Fiorina, former chief executive officer of Hewlett Packard, once wrote, "The goal is to turn data into information and information into insight."[8] We can use our new data to improve the lives of the known 200,000 with hemophilia, and eventually to identify the suspected 1 million and more with hemophilia.

6. These premature deaths in high-income countries are due mainly to HIV/AIDS and hepatitis C infection, as well as uncontrolled bleeds, particularly in people with inhibitors. 7. The life-expectancy disadvantage was calculated by dividing the prevalence by the prevalence at birth, and subtracting from one. 8. hp.com/hpinfo/execteam/speeches/fiorina/04openworld.html

Richard's Review... from page 5

and six units of fresh blood. His physicians worry that Davey might develop antibodies and "become refractory" (now called having inhibitors) to the fresh blood and plasma. Meantime, Davey's untested sister worries that she might be a hemophilia carrier. Davey recovers and becomes an honor student. This hemophilia treatment was appropriate for 1954, though the amount of blood given is worrisome for circulatory overload.

Ben Casey

Vince Edwards

In direct competition with *Dr. Kildare*, ABC broadcast 153 episodes of the award-winning *Ben Casey* series on Wednesday evenings during five seasons, from 1961 to 1966. Vince Edwards played Dr. Ben Casey, while Sam Jaffe was the older Dr. David Zorba. Unfortunately, Dr. Casey never treated a patient with hemophilia. Yet National Hemophilia Foundation chose Vince Edwards—often mistaken by the public to be a real doctor—as their celebrity spokesperson.

Marcus Welby, M.D.

ABC aired the award-winning *Marcus Welby, M.D.* for 169 episodes on Tuesdays over seven seasons, from 1969 to 1976. The hour-long popular program, rated number 1 in its second season, was filmed in color. The American Academy of General Practice endorsed the series. Robert Young starred as Dr. Marcus Welby, while James Brolin played the younger Dr. Steve Kiley. In "The Daredevil Gesture" episode, broadcast March 17, 1970, 17-year-old Larry Bellows is a high school senior who doesn't want his classmates to know that he has hemophilia. Larry is hospitalized about four times a year for his hemophilia, and treated with AHF (antihemophilic factor) injections. His mother, suffering from guilt, is overprotective. His sister, though never carrier tested, refuses to ever have any children. Larry defies his mother to attend a science club hike into the California canyons. He hemorrhages into his left knee while assisting a classmate with a broken ankle. Dr. Kiley rushes to the canyon with concentrate and plasma to treat Larry, who finally admits to his classmates that he has hemophilia. This episode focuses on the psychological

dilemma of an adolescent with hemophilia who strives for normalcy by overcompensating with reckless behavior.

All bleeding disorders are considered rare. The popular medical dramas broadcast on network television from 1950 to 1980 rarely used hemophilia as an interesting disease of the week. This is surprising, knowing how the misconception of bleeding to death due to hemophilia has so often been used as a plot enhancer by fiction writers. The fictional doctors on television acted more like role models, often in dramatic tension with other doctors, rather than as cooperative medical mentors to each other. Their sometimes inappropriate hemophilia treatments highlight the fact that the bleeding disorder community needed better treatment, namely through government-funded comprehensive care at hemophilia treatment centers staffed by a multidisciplinary team of medical specialists. Regrettably, televised medical dramas never showed the important advancement in hemophilia treatment that arrived in the 1970s.

YOU... from page 6

Servant Leadership

The style of leadership parents and patients most often mentioned is known as servant leadership. Servant leadership puts people's needs first. A leader must understand the needs of the followers or constituents, be there with them, even suffer with them. Indeed, a trait of servant leadership is compassion, which means to suffer or feel alongside someone. It follows that traits of servant leaders include listening, empathy, stewardship, awareness, healing, commitment, and community-building. Historically, servant leaders have been either positional, situational, or transformational leaders too, but have always put their people's needs first.

Raymond W. Stanhope agreed. "To be in service to the people you are leading—servant leadership" explains the style of leadership that works best today in our community.

Raymond W. Stanhope

Who are the servant leaders in the bleeding disorder community? Kathe Gusler cited Dr. Lisa Boggio and Dr. Michael Tarantino, of Illinois: "Both physicians are excellent leaders...They always take time to explain to patients and integrate therapies into everyday life. I am a better nurse for knowing them." Rayna Conetta mentioned Dr. Clifford Takemoto, currently at St. Jude's, "for leading a whole generation of patients through recombinant therapies by studying the medicine and checking the facts. All the while he has a wonderful bedside manner to both parents and children." Emma Miller-Clark added that Dr. Takemoto "would always make time to volunteer for the community. A true gem of a human being and a brilliant doctor."

Kelly Lynn Gonzalez, who was nominated by respondents as a servant leader herself, listed integrity, compassion, and honesty as important qualities for our community leaders. These characteristics don't require a leader to be in a position of authority.

Kelly Lynn Gonzalez

But positional leaders—those in authority—can be servant leaders as well. Christal Reed nominated Jeff and Rose Watkins. "They started a chapter here in Illinois and do so much to help the underserved members of our community!"

Likewise, Tiffany Branham Maness recommended Sue Martin, executive director of Hemophilia of South Carolina, as a servant leader. "She gives our organization her heart and soul. She has turned our local chapter into a source of hope, progression and compassion for our bleeding disorders community. And most importantly she drives people to educate themselves on advocacy, so we can advocate strongly and effectively for our children, and teach them how to become strong advocates for themselves. I love her to bits! She's turned what I was afraid would be a curse into a blessing, and we feel like superheroes that can handle anything because of her work!"

Perhaps no one models servant leadership quite like Jonathan James, a person with hemophilia who founded and runs Hope for Hemophilia, a nonprofit in Louisiana. Renee Michelle wrote, "This leader has the biggest heart in the world. He leads with so much compassion. Jonathan is not afraid to get in the trenches with anyone and hold their hand through a hardship. This guy gives all he has and then keeps giving...he leads from the heart. After spending time with Jonathan you walk away refreshed and feel like you can conquer the world because he is so positive and uplifting to his fellow bleeding disorder families."

Ray Stanhope warned that as great as servant leaders are, they must ensure that they are not indispensable. In other words, leaders must develop *other* leaders.

Mentoring

The great British wartime leader Winston Churchill was called the "Old Lion." I think of this when I think of the top leaders in our community who fought our war with HIV, and are now in their 60s. Mentoring a new generation of lions, then, is vital. Some mentoring happens at the top, with youth leadership programs from National Hemophilia Foundation (NHF), but mentoring can occur anywhere by any of our servant leaders.

Renee Michelle with Benjamin Denman

Renee Michelle nominated Benjamin Denman as a servant leader who mentors. "This guy has such a huge heart when it comes to our community. He wants to be so involved everywhere that he sometimes spreads himself too thin; but being there for his brothers and sisters is his passion. He has a list of guys he calls to check up on almost weekly. Benjamin has a heart to mentor the younger generation...to help people transition from teenagers to adults. He is loved and trusted by all because he is so real—and funny."

Two mothers mentioned their own sons as servant leaders who mentor younger followers. Dawn Butler Spikes wrote, "My oldest son, Grant Spikes, age 25, is passionate about empowering younger kids. He never missed a year of Camp Ailihpomeh (Texas) since age seven, and even after moving out of state for a while, came back to work as a counselor in summers. He is now the Camp Ailihpomeh director! He graduated from Texas A&M and was in the Corps of Cadets all four years. He was not about to let severe hemophilia A slow him down, and wants to pass that message on to other kids and parents." Servant leadership by example, combined with positional leadership, is powerful!

Kimberly Hennessy-Rosenfelt nominated her son Dakota J. Rosenfelt. She believes he has these leadership qualities, so important in servant leaders: integrity, honesty, loyalty, empathy, compassion, inspiration—and mentoring.

Unsung Heroes

Finally, Lisa-Marie Mathieu made a poignant suggestion: "I'd like to nominate all of the 'silent leaders'...parents who take the time, often in private, to welcome new members, offer our help, guidance, experience as parents with a baby with a bleeding disorder...Often done behind the scenes, but very helpful and appreciated nonetheless."

Sometimes the greatest leaders are the ones without the fanfare, tweets, photo ops, and popular achievements. They are the servant leaders who quietly and modestly, powerfully and permanently, change the world day by day, child by child. They are all of you—parents, patients, doctors, nurses—who dedicate their lives and careers to the bleeding disorder community.

inbox

I CAN'T THANK you enough for responding to me with your generosity and kindness, and for your book *Raising a Child with Hemophilia*. Your enthusiasm and optimism has a ripple effect on me and thus upon many others. Thank you so much for your willingness to share and help others. You might want to know how I heard about you. I got in touch with our Hurley Medical Center Pediatric Hemophilia Clinic, situated in our own backyard. They too have been so helpful in giving me various ways to educate myself, enabling me to be a strong anchor for our children with sensitivity as well as knowledge.

Shainie Weingarten
MICHIGAN

THANK YOU AGAIN for all you do, Laurie. I attended a woman's conference in Colorado years ago, and you spoke personally about communication. It will always be with me. You taught me more about interpersonal communication that day than any college course. I really appreciate your personal touch; it always goes a long way to be intimate with your audience. On top of that, your vigor for the hemophilia community is inspiring and I am grateful to have such a strong presence be a source in the tribe.

Heather Witkowski
TEXAS

I WENT TO UTAH Hemophilia Foundation's "Everybody's Here" event on November 14 and was very impressed by the documentary *Bombardier Blood*. Here is a link to the official trailer on YouTube. Please take a minute and check it out to find out more about hemophilia and what you can do to help: https://youtu.be/qgl7Ahlujs4

I was also very honored to finally meet Laurie Kelley in person! Her books helped me handle my son's diagnosis, and she has been educating and helping our community so much. She is one of my biggest heroes!

Joy Des
UTAH

THANK YOU SO much for the books you sent my son Luke! They meant so much to him, and he enjoyed reading them. I'm so happy he has books that he can keep for a lifetime. The work that you do is so important for the hemophilia community. I hope I can join you on a mission trip one day!

Erika Wilson
NORTH CAROLINA

I WANT TO thank you for the books. My kids all love the *My First Factor* gift book set! It really got my four-year-old daughter interested and wanting to learn more about what her baby brother has. She even had me look up hemophilia cartoons on YouTube! So thank you so much. What you do is truly amazing.

Kimberly Sherman
WYOMING

WHEN WE FIRST got our diagnosis, you reached out, sent us books, with no request for compensation, even though we live in Canada. There are no boundaries on your goodwill. By taking donated product to countries and patients that don't have access to not only products, but needles and accessories needed for treatment, you're reaching out to the hemophilia community at large, not just in your own country, or continent. You made me feel like this diagnosis isn't just something to deal with, but we're part of a larger community. Instead of seeing this as a health issue, I can look at it as a blessing. Of all the health issues we could have had, I'm grateful it's hemophilia, because we are such a supportive group. Because we have the resources we have. Because it seems everyone with this diagnosis actually cares about others, and just wants everyone to succeed in raising, developing, and generally just helping one another.

Amanda McIntoshi
CANADA

PEN, November 2019

THANK YOU FOR the article on gene therapy. My son Zach did a trial for gene therapy. He travels around the world for work and needed to be at the HTC weekly for almost two years. He is my warrior. He was in the trial for the first recombinant factors also. Gene therapy put him through a lot, but he insisted. It brought the factor levels up for a while, and gave us so much hope, but then they crashed at the end. So, gene therapy will never work for him again, but then Hemlibra came along. Thanks for all that you are doing!

Carol Shaw
NEW YORK

I REALLY ENJOYED your article "Prepare for Big Changes" in PEN. You gave me a good laugh when comparing all the pharma changes to "wild teen years" and "marriages and divorces." That was a good piece of writing: balanced, informative, and humorous! Thanks again for all that you do.

Ken Wohleking
TEXAS

JUST WANTED TO thank you for the great article "Prepare for Big Changes"! Great summary of the history of all the companies merging, divorcing, etc. I could never keep it straight!

Wes Michael
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