Celebrating Our 10th Anniversary

September 2019

# PEN's Insurance Pulse

Inspiring Advocacy

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# Novel Therapies and Factor: Keeping the Old with the Gold

**Wendy Owens**

Change. It's happening all around us. And it's happening at a rapid pace with hemophilia treatment. The US Food and Drug Administration approved 19 new hemophilia treatment products in the past seven years, and 31 more are in the pipeline.[1] New clotting factor concentrate products (factor) and novel nonfactor therapies continue to join the hemophilia treatment marketplace. Other novel nonfactor therapies, like RNAi (RNA interference therapy) and various gene therapy products, appear to be working well in clinical trials and may soon join the growing list of treatment options.

Despite all the change, one thing remains the same, at least for now: You still need clotting factor concentrate, or bypassing agents if you have inhibitors, to treat bleeds.

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1. "Emerging Therapies Currently Undergoing Clinical Trials," *Dateline Federation*, Special Issue 2018–19: 29–39.

# Welcome


## PEN'S INSURANCE PULSE

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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 Pulse articles, when we refer to a person with hemophilia, we may alternately use "he" or "she," or just "he."

I can always tell when there's a new factor product on the market: some people switch to the newest product right away, and start shipping their unwanted products to Project SHARE, for us to donate overseas. This year already, we've seen an uptick in donations. In this issue of Pulse, feature writer Wendy Owens explains why you might want to hang on to your factor just a bit longer, and how insurance companies may view having two brands of therapies.

Other people are still struggling to get any kind of treatment. Meet Milora Morley, a young woman in the health-care field. I saw her mention on Facebook what a hard time she was having getting any doctor to believe that she has hemophilia. So I contacted her to learn more. What a story! Then, by chance, I bumped into her at Hemophilia Federation of America's annual symposium in San Diego, not long after we first corresponded. Not only is Milo scientifically minded, she's a great writer with a fascinating story. Read about it in "My Life."

And be sure to read all of Pulse, because we cover a broad range of topics in insurance. It's our 10th anniversary of bringing you useful information and strategies to overcome insurance challenges. Rest assured that your national and state bleeding disorder organizations are working hard, getting ready to defend you against anything that might remove access to therapies or increase cost. Make sure you're working hard for these same goals too!

*Laurie*

PEN's Insurance Pulse is a newsletter for families and patients affected by bleeding disorders. It is published by LA Kelley Communications, Inc., a worldwide provider of educational resources for the bleeding disorder community. Pulse focuses on insurance, coverage and reimbursement policies, trends, family profiles, and expert opinions.

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Laurie Kelley

# Prepare for BIG Changes


When we use the word "transitions" in hemophilia, we normally think about our child's life cycle (or our own) and how it impacts healthcare: Children transitioning into adulthood at age 18 need to see an adult hematologist, not a pediatric hematologist. Adults finishing college or trade school need to transition into the workforce. Adults at age 26 need to transition off their parents' health insurance. We may have joint issues as we age.

But we can also view transitions as something happening to hemophilia treatment, because that has a life cycle too. And right now, hemophilia treatment is undergoing massive, rapid change. New products, novel therapies, and gene therapy…can the US market of roughly 20,000 with hemophilia support all the products? Will insurance cover them all?

## A Look Back: Our Infancy

If we accept that hemophilia treatment has a life cycle, let's look back at its infancy and development. Early treatment for hemophilia involved whole blood transfusion and later, human plasma. Fresh frozen plasma (FFP) allowed treatment to be stored and then thawed when needed. Whole blood and FFP were both only marginally effective at controlling a bleed, due to the low level of factor VIII and large volumes that needed to be infused. Then, in the mid-1960s, came cryoprecipitate: rich in factor VIII, and created from thawed human plasma. Its higher factor VIII level in a smaller volume made administration easier, faster, and more effective.

A breakthrough came with commercial clotting factor concentrate in 1968, made from donated human blood or blood plasma. This new plasma-derived concentrate was freeze-dried and reconstituted with water when needed. Factor in a bottle! Now treatment could be done at home or away, freeing patients to travel and live more normal lives. Infusion time was tremendously shortened, meaning treatment could stop bleeding faster, reducing pain and helping to prevent joint disease. But these early factor concentrates were not treated to inactivate viruses. Tragically, in the late 1970s and early 1980s, contamination of the nation's blood supply from donors infected with HIV, hepatitis C, and other viruses meant that clotting factor was also contaminated. Thousands of people who had hemophilia and used factor concentrates were infected and died.

In the mid-1980s, a new stage in the life cycle of hemophilia treatment emerged: the creation of factor that was virally inactivated. Viral inactivation processes, such as heat or solvent/detergent wash, could destroy the fragile HIV. Today, all clotting factor is considered safe.

The deaths of so many led to another groundbreaking stage in the life cycle of hemophilia treatment: recombinant clotting factor. Recombinant factor is produced in a lab, using human genes that are inserted into genetically altered mammalian cells. In 1992, the first recombinant factor VIII clotting factor product (Recombinate, manufactured by Baxter) was approved by the FDA. In 1997, the first recombinant factor IX product (BeneFIX®, manufactured by Genetics Institute) entered the market. And now? There are now about 20 recombinant factor products on the market to treat hemophilia. We've come a long way.

## The Family Expands: Crying for Attention

Why so many products? There are some things to know about the factor market. First, the US is the largest market. Not in population—that would be China. But in dollars. Estimates are $4.6 billion annually.[1] This lucrative market attracts competitors. But factor products are protected by patents. So to avoid patent infringement, changes or improvements are made

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1. Mark Terry, "A Look at Hemophilia Drug Prices and the Market," biospace.com, July 3, 2018 (accessed June 2, 2019).

## Novel Therapies *from cover*

This means you could use a novel therapy for prophylaxis to prevent bleeds, or even receive gene therapy that might bring you to a mild hemophilia factor level. But when a bleed happens, you'll need factor! Remember the old song about making new friends but keeping the old? Because "one is silver and the other gold." Let's make new therapies, but keep the old—because for now, the old therapies remain the gold standard for treating bleeds. Gold isn't cheap, and neither is treating hemophilia. The big question is this: Will health insurers pay for the duo of a novel treatment for prophylaxis plus the factor you may need to treat bleeds?

### New Pairings, New Access Concerns

Health insurance providers are looking hard at the benefits of paying for two medications to treat hemophilia now, versus just one. "We are absolutely experiencing more concerns from insurance companies about patients being on a treatment plan that could be three times the cost of factor alone," says Moniqa Cadriel, hemophilia program specialist at the Hemophilia and Thrombosis Treatment Center at UC San Diego Health. "This shift of treating prophylactically with a novel therapy and having factor in case of bleeds has insurers asking 'why.' So, they require even more justification and a detailed explanation of why a patient needs both."

Before you can switch to a novel therapy/factor combination as your treatment regime, insurers may ask your hematologist to explain what added value this treatment duo would give your health outcomes. Some insurers require that patients get pre-approved before they can fill their prescription for the novel therapy/factor combo. The process of receiving approval from your insurance provider before being able to access a prescribed drug is called *prior authorization*. Basically, this means your hematologist must supply written justification for why you need a treatment, and your insurance company must agree that this value is worth the expense.

### Data Drive Decisions

"Prior authorization can be easy or daunting because of the cost of a novel therapy plus factor, instead of factor alone," says Dr. Stacy Croteau, pediatric hematologist and clinical researcher at Dana-Farber/Boston Children's Cancer and Blood Disorders Center. "Providers must justify the need for keeping factor at home, and they need to have the data to support why it is better for a patient to use a novel therapy/factor combination rather than factor alone." Some of this data comes from clinical trials and other studies demonstrating that one drug works better than others and is safe. This data also comes from you. Your hematologist and team will make the case to your health insurer that a novel therapy is right for you; but to maintain access to the novel therapy/factor duo, you must stick to your prescribed treatment regime and keep a record of treatments.

"Patients need to log their treatment, no matter what kind, in case they face insurance-related access issues, which are case-by-case and wholly dependent on each patient's medical situation," Cadriel says. She admits that there is no perfect advice for guaranteeing that an insurer will continue to support your use of novel therapy/factor as co-treatments after giving initial approval. "When a patient is healthy, they are healthy for a reason; and that reason is that they are receiving the right care and treatment," explains Cadriel. "Insurance companies should not try to be providers, but patients need to show the value of their novel treatment and factor combination so their doctor can let the insurer know the combination makes a big difference to their life."

If you use a novel therapy, sticking to your prescribed treatment regime and logging your treatments is one way to demonstrate the value of that therapy, especially when your logs show it's preventing bleeds. For your hematologist, your log lets her monitor adherence to the treatment schedule, so she can make changes to improve your outcomes when necessary. For a health insurer, your log can demonstrate how a therapy is working and whether it improves your outcomes—this could mean reducing trips to the emergency room and allowing you to increase physical activity. Such improvements are good for you and can reduce your healthcare costs, making your insurer happy.

### Factor at Home

Once your insurer has approved your prescribed treatment regime of a novel therapy/factor, you can get the dosing you need. Here's the next question: How much factor must you have on hand in case you have a bleed? The answer: Much less than you needed before if you used factor for prophylaxis. "Patients will find themselves keeping much less factor at home, and this may be an uncomfortable feeling for some," says Croteau. "It will seem an odd deviation from the norm, but eventually they will become comfortable with this, even patients with inhibitors."

How many doses of factor should you have on hand? National Hemophilia Foundation's (NHF) Medical and Scientific Advisory Council (MASAC) recommends that patients using factor "who infrequently infuse [have] doses available at home to allow…for care in an emergency, as

local healthcare facilities cannot be relied upon to stock the appropriate replacement products for these patients."[2]

George Stone of Lake Frederick, Virginia, used to keep almost 50 vials of factor at home for regular prophylaxis infusions, but things have changed now that he's on a novel therapy. "Previously, I was ordering 48 vials of factor per 90 days for prophy treatment three times per week," says Stone. "I don't anticipate there would be a problem having the factor I might need on hand with adequate hematologist justification."

Dr. Annette Von Drygalski is director of the Hemophilia and Thrombosis Treatment Center at UC San Diego Health. She advises, "When you are on a novel therapy, you need to be pragmatic and use common sense. You need to be in regular contact with your HTC [hemophilia treatment center] and hematologist to figure out together how much factor to keep on hand. There still is much we don't know about using new therapies, and until we do, we need to work together to see how it all plays out. This is the most important part."

Croteau looks at a patient's phenotype, how close the patient is to an HTC, and how quickly a specialty pharmacy can get factor to that patient. Croteau explains, "The goal is to get patients what they need right when they need it."

Von Drygalski agrees. "Patients need a safety net; they need to be prepared in case they do have a bleed, and this means having factor on hand. Depending on the patient, I recommend having two to five doses of factor at home in case of a bleed."

In the future, insurers may look closely at the number of factor doses patients keep at home, especially if these backup doses expire because novel therapies are effectively preventing bleeds over a long period. But Von Drygalski doesn't think expired factor is a bad thing: "If the doses you have on hand expire, they expire," she says. "You hope you don't need them; and you should not feel badly about it. Yes, there is a cost to having factor around for treating a bleed should you have one, but it is better to have a few doses on hand in case of an emergency."

## Inventory Management

Managing your factor inventory when you use a novel therapy differs from using factor for prophylaxis. While it sits on a shelf, your factor's expiration clock is ticking. It's important to keep track of using or not using factor, in case your insurance company asks. According to Cadriel, "Insurers are not providing a set limit for the number of factor doses a patient can have at home yet, but they can ask whether patients have had factor expire at home."

MASAC guidelines encourage patients and family members to "track expiration dates of [clotting factor concentrate] on a monthly basis. Doses that are about to expire should be utilized first to prevent waste."[3]

Note your factor expiration dates in a location you check regularly, or set a reminder on your phone a few weeks or days before your factor is set to expire. Also, talk to your hematologist and other hemophilia healthcare providers, and ask them how to manage your factor inventory. "Factor inventory management will require different support and education," notes Croteau. "The factor inventory management regime for patients using a novel therapy will be more like it is for mild hemophilia patients who need to check factor expiration dates if they keep some on hand."

Inventory management doesn't end with watching factor expiration dates. If you need to use your factor, make sure you have unexpired ancillary infusion products available, too. "All infusion products have an expiration date," says Alex Dube, senior sales manager at Save Rite Medical. "If ancillary infusion products are properly stored, they should not degrade, but over time their sterility can become compromised. The loss of sterility is why they have an expiration date."

Look for the manufacturer's expiration dates on saline, tubing, needles, and other ancillary products, and be sure to regularly refresh your supply.

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2. "MASAC Recommendations Regarding Doses of Clotting Factor Concentrate in the Home," June 7, 2016. 3. "MASAC Recommendations."

## Ask the Expert

Laurie Kelley

# Your Health: It's Worth Fighting For

### Q I feel like I'm not really getting what I need from my healthcare coverage. What should I do if I've already received a denial of service or medication?

Here are examples of denials that may happen:

- Your health insurance company won't let you be seen at a hemophilia treatment center (HTC) for comprehensive care because the HTC isn't "in-network."
- You can't go to an HTC because you're in a health maintenance organization (HMO). Your managed care company doesn't have any physical therapists in-network experienced with bleeding disorders.
- Your HTC doctor prescribed a brand of factor replacement therapy, and your insurance company won't approve it.

What can you do about these denials?

Typically, people tell me they've just accepted the insurance company's decision and denial. But you need to be proactive. Did you or your healthcare provider ask the insurance company to reconsider? Did you file a complaint? You have a right to challenge the decision. You'll likely get help from your provider, and you may enlist the help of your local hemophilia organization. Consider asking for an appeal even if you have Medicaid. Sometimes, people with Medicaid feel they are getting "free" healthcare and don't want to rock the boat. *No matter what type of healthcare coverage you have, you have the right to ask the company to reconsider.*

### Q How about a few ideas to get me started on an appeal?

Here are some ideas on getting what you need:

- Insurance companies are in the business of providing care that leads to good medical outcomes for their members. If you and your healthcare providers can show why something is medically necessary, your chances of getting the insurance company to cover it will increase.
- If an HTC isn't in-network, ask the insurance company to make an exception. When you call the number on the back of your insurance card, be prepared to tell them why you need this service. You'll probably have to ask the HTC or provider to write a letter to the insurance company. If the insurance company still says no, you can file an appeal.
- If your insurance company doesn't agree with the treatment plan that you and your healthcare provider have agreed on, you may need a letter of medical necessity. For example, if your treatment plan includes a prescription for a different factor replacement therapy than you currently use, a prior authorization may be required. If the insurance company still doesn't approve it, you can appeal that decision.
- You'll need to follow the processes for complaints and appeals that your insurance company requires, so keep good records of phone calls and correspondence (emails, letters, forms).
- You can get the details on the company's process by calling the number on the back of your insurance card or going to the insurer's website.

Bottom line: If you and your healthcare team think that you're not getting medically necessary treatment approved, you've got to speak up!

Milora Morley, MPH

# Hemophilia: A Woman's Journey to Effective Treatment

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

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

I remember I encouraged my brother to be positive and live his best life. Hemophilia was not the end of the world! Now, here I am years later, feeling discouraged and defeated, as if hemophilia were indeed the end of the world.

My personal journey has been difficult. Witnessing my brother's experience made me hope that I'd also get the attention and treatment I need to move forward. But I was wrong. As much as health professionals may know about hemophilia, it seems that they know it only as it pertains to males. In my experience, the specialists aren't always educated about this bleeding disorder, or maybe they truly don't want to believe that a woman can have hemophilia.

## A Surprise Diagnosis

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

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

My brother wrapped up his final semester as a senior, and I moved to Los Angeles, California, in October 2017. In November my brother called, asking me to contact his nurse. When I called her, she explained that they had been trying to reach me for the last few months. I was in a bit of shock as she told me that I have a factor level of 38%—mild hemophilia A—and that I should register with a local hemophilia treatment center (HTC).

I asked her what hemophilia looks like in women. I reflected on my past and current health issues, particularly my prolonged menstrual bleeding. At age 16, I began experiencing irregular menstrual bleeding due to abnormal hormone levels, but by age 22 my periods became more irregular and very prolonged. My menstrual cycle would stop for a day or two and start all over again; this went on for months. Doctors diagnosed me with pre-polycystic ovarian syndrome (PCOS), and started me on birth control right away.

Before my hemophilia diagnosis, my ob/gyn refused to take me off birth control. We agreed that once I reached my weight goal, she would take me off so we could address the underlying issue of what was causing my PCOS or prolonged bleeding. I lost nearly 90 pounds to combat PCOS, and achieved my weight goal, only to have the doctor change her mind and tell me that I should just stay on birth control.

I felt defeated. I eventually decided to stop birth control and explore my own options. By this time, I had already relocated to LA and had just received my hemophilia A diagnosis, but I was also having prolonged bleeding. I thought it best to start the journey with the mindset that maybe this bleeding was not just PCOS but also influenced by my new hemophilia diagnosis. So I connected with my PCP and decided to get back on birth control while I figured this all out.

*Names have been changed due to pending legal action.

*continued on page 13*

# Community Forum

Laurie Kelley

Since we started publishing PEN's Insurance Pulse in 2009, we used "Community Forum" to ask opinion leaders in the hemophilia community about their thoughts on trends in insurance. But parents and patients are opinion leaders, too. For this issue, we asked community members on Facebook: What is your number-one worry about health insurance these days? What trends have you noticed that have you concerned? The results fell into five main categories.

## 1. Out-of-Pocket Costs

Out-of-pocket costs are the money consumers must pay to buy or keep insurance. These costs can include copays, coinsurance, deductibles, and premiums.

Stacey Mollinet writes, "Our son's max out-of-pocket was reached with the first shipment of factor in January, and it's a big amount. It's been $5,000–$8,000, depending on our insurance plan over the past several years. Although our factor company currently pays our copays, I'm worried that our insurance company will stop allowing a third party to cover the copays for us."

Stacey Mollinet

Susan Fanning writes, "What kills our family is copays. It's $75 per child per visit to see a specialist, and $150 to get my kids to their hematologist. It's $35 per kid per visit for therapy, which includes physical and psychological therapies. Let's not talk glasses—a never-ending money pit. My kids aren't getting all the services they need, because I don't have enough nickels to rub together. Coming up with the full amount of cash upfront until the deductible kicks in is difficult. I'm disabled and my husband works hard, but has minimum benefits."

Nathan Loots agrees. "My out-of-pocket as gone from $6,000 to $9,000 annually. Our biggest stress is that the max will keep rising." And for Jennifer Smookler-Davis, it's the monthly premium: "The overall monthly premium cost of insurance to get a good plan with good coverage keeps rising. My monthly premium has increased over 40%."

Bottom line? Learn what out-of-pocket costs are covered by your insurance plan. If you're using a bottom tier "catastrophic" ACA [Affordable Care Act] bronze plan, put aside at least $7,900 annually per person, or $15,800 for a family, to pay for out-of-pocket costs like copays, coinsurance, and premiums.[1]

Nathan Loots and family

## 2. Accumulator Adjustment Programs

Some nonprofit organizations, as well as factor manufacturers themselves, offer up to $12,000 annually to help pay for medication out-of-pocket costs for qualified individuals.[2] Unfortunately, some insurance companies have stepped in to limit how those funds are used. Under a "copay accumulator adjustment program," your copay assistance is applied toward the cost of your medication, but not toward your annual out-of-pocket deductible. Often, patients are unaware of this change until several months have passed and they're hit with thousands in copay costs that weren't covered by their copay cards. Insurance companies use accumulator adjustment programs to influence consumers to buy lower-cost generic drugs; but for many diseases and disorders, including hemophilia, there are no generic drugs.

Rachel Neyland was personally affected by accumulator adjustment. "The insurance company took the entire $12,000 copay assistance I got for factor, and I still had to pay $10,000 for my deductible. So they took $22,000 instead of only my $10,000 deductible."

Lauren Killgore had the same experience. "Our copay coupon only helped us with the cost of my husband's medication for one month. His copay is over $6,300 every time his prescription is filled, and the copay coupon always satisfied the deductible until our insurance adopted the accumulator adjustor program. Now the copay coupon is only applied to the copay for the medication fill. The accumulator adjustor does not allow third-party payments to satisfy the deductible anymore. While we were in the process of getting everything figured out with this new program, he developed a target joint and now has joint damage because of a bleed that resolved itself—without factor due to the insurance hold."

Hope Thacker adds, "On July 1, 2019, our pharmaceutical copay increased to $1,300 per child per month. Without the help of a third-party copay assistance acquired by our employer, there's no way we would be able to make the monthly copay payments for our children's medication."

Hope Thacker and family

1. An ACA plan has an out-of-pocket maximum of no more than $7,900 in 2019. If you have more than one person in your plan, the combined family out-of-pocket max can't exceed $15,800, although the plan must have an embedded individual out-of-pocket max that can't exceed $7,900. Normally, only the lowest-cost plans reach these maximums. People with chronic disorders should opt for a gold or platinum plan, which costs more upfront but has lower copays. 2. For a listing of nonprofit patient assistance programs that can help with copays, visit https://panfoundation.org/index.php/en/patients/patient-resources/co-payment-assistance-organizations.

## 3. Payers That Don't Understand Hemophilia

"I'm an educator in Kentucky," says Dana Jones-Brady, "and we used to have Humana. They accepted the fact that my kids had hemophilia B. They never questioned anything, and always paid our factor bills. When we switched to Anthem a few years later, something definitely changed. Our first emergency issue with one of the boys was rather pricey. They requested open records and a letter to 'prove' my kid had hemophilia B. I was so upset. I was so annoyed at the fact they would believe a parent would lie about this. It was a big ordeal. Luckily, since then we haven't had any issues."

"I have Anthem, too," chimes in Nathan Loots, "and they are good once they get the doctor's orders. I had Cigna for four years, and they are awful. It was the worst four years of my life. I would spend 30 to 40 hours on the phone with them monthly disputing the claims about pre-existing conditions, and them denying it. All three of my boys have hemophilia A. However, I would always receive our medicines. When [the] pre-existing [condition exclusion period] went away with ACA, we by chance switched to Anthem Blue Cross, and they have been amazing—no issue at all. Once they got the records they needed, they didn't question anything. They even called our supplier to fix an issue with their billing side."

Hope Thacker also has three boys, all with severe hemophilia A. "While we've always been able to acquire treatment and medications, working through the mess that is insurance today has been so stressful. Every company is different, their level of understanding of hemophilia is different, coverage is different (like copays vs. major medical), and allowances for emergencies are very stressful."

## 4. Specialty Pharmacies Closing

Specialty pharmacies (SPs) purchase factor either directly from the manufacturers in large quantities, or through specialty drug distributors, who also buy in bulk from manufacturers. Then SPs deliver factor to the patients. The top 10 SPs are multibillion-dollar companies that control 80% of the market share of specialty drugs. Beginning in the 1980s, the profitable hemophilia market produced small SPs dedicated only to hemophilia, often employing or even founded by people with hemophilia. And people could choose the SP they wanted, based on referrals from friends or SP reps at conferences. But since 2005, to control costs, insurance companies have been designating a preferred SP or offering a choice of two or three. Smaller SPs have faced narrower profit margins, and have often been dropped from major contracts. Larger companies have been buying smaller SPs. The few remaining small SPs will find it hard to survive.

In some cases, hemophilia patients may lose their jobs at SPs, especially as their children age out of their insurance plan.

## 5. The Future

In a tense and explosive political climate, and amid ongoing efforts to undermine or repeal the ACA and its protections for people with chronic disorders, no one knows what the future will be for health insurance. "Every day I worry that I'll have to choose between paying our rent, buying groceries, or paying for factor," says Jessica Smith. "Worrying about running out of factor and not being able to afford more is always on my mind. What if the laws change? What if no insurance company will cover my son? The 'what ifs' are always there."

"Agreed!" exclaims Tommy Townsend. "Luckily, our personal specific insurance situation is great right now—no major problems in my son's almost nine-year life. I am more concerned about the big picture, such as national changes to healthcare laws that may come as political administrations continually change. The idea of my son's pre-existing condition affecting his future access to care scares me."

Bonnie Culver fears the constant changing of insurance carriers from year to year. "Needing to start over with pre-authorizations. Fighting with the new company over whether it's a pharmacy benefit or a medical benefit. Then fighting over having a choice in products and a choice in suppliers. By the time you get it all worked out, you're forced to switch to a new insurance company and start all over again."

And what about new designations in diagnosis, and new therapies? Shellye Horowitz notes, "As a woman who only recently received the correct diagnosis from my HTC so I can receive the factor I need, I worry future access to therapies will be taken away by insurance companies who don't believe women actually get hemophilia."

Shellye Horowitz


The best defense is a good offense. Contact your local bleeding disorder chapter to get involved and educated. Visit the websites of National Hemophilia Foundation (NHF) and Hemophilia Federation of America (HFA) to learn more. And forward your insurance concerns, complaints, and victories to HFA, whose hotline, Project Calls, may be able to help. The story you share may in turn help others.

**HFA: www.hemophiliafed.org**
**NHF: www.hemophilia.org**

# Tracks & Trends


## Cheers, ACA

March 23, 2019, was the 9th anniversary of the Affordable Care Act's passage. From 2010 to 2017, the ACA reduced healthcare spending by $2.3 trillion. In 2017 alone, health expenditures were $650 billion lower than projected, keeping healthcare spending under 18% of GDP—a tad over where it was in 2010. The ACA expanded health coverage to more than 20 million previously uninsured Americans.

*Source: www.statnews.com*


## The Uninsured

The number of Americans without health insurance has increased by 7 million since 2016. In 2017, 9.1% of Americans had no health insurance: according to a Gallup Poll, the most common reasons were that healthcare was unaffordable or people didn't feel they needed it. Other reasons: many Americans thought the ACA was repealed; the Trump administration has allowed some states to require low-income residents to work to gain health coverage; funding for ACA enrollment and outreach has been cut, leaving fewer people aware of their health insurance options.

*Source: www.gallup.cpm*

## Did You Know?

**1.5%**

of all Google searches are health-related.

## Yes, the ACA Is Still Here

Kaiser Family Foundation found that 17% of Americans believe the ACA has been repealed; 14% aren't sure. The ACA is still standing law. Republicans weren't able to repeal "Obamacare" when they controlled both houses of Congress, and despite their intense efforts to repeal the law, it continues largely in place.

*Source: www.kff.org*


## Age 65? Some Facts About Medicare

Medicare is not based on affordability. If you are age 65 or older, and if you qualify for or are receiving Social Security benefits, you may be eligible. Medicare is not free, but the premium is much lower than for standard health insurance. Medicare covers up to 80% of what private insurers do; it doesn't cover all costs. Medicare has four parts:

- Part A: Hospital/hospice insurance
- Part B: Medical insurance
- Part C: Medicare Advantage plans
- Part D: Prescription drug plans

*Source: medalerthelp.org*

## Do-It-Yourself

Buying health insurance on your own? Here are options for purchasing a policy:

- Your state's health insurance Marketplace at healthcare.gov
- Health insurance company
- Websites like insure.com offering health insurance quotes from multiple carriers
- Health insurance agent

## HFA Takes Action

On December 14, 2018, a Texas judge ruled the entire ACA unconstitutional. (This ruling has no immediate impact on the ACA.) On April 1, 2019, Hemophilia Federation of America joined with 16 other patient groups to file an *amicus curiae* ("friend of the court") brief to appeal the Texas district court decision. The brief argues that affordable, accessible healthcare is essential to managing chronic disease; that the ACA improved access to healthcare for millions; and that the decision ignored Congress's policy-based choice to preserve the ACA.

*www.hemophiliafed.org*

## The High Price of Health

Americans pay more for medical costs than do citizens in other nations, despite our medical care ranking last against 10 other high-income countries. The Commonwealth Fund's research showed that while Americans pay the highest healthcare costs, they also have the lowest healthcare outcomes. Mortality rates in the US are higher than in comparable countries, and although the mortality rate has steadily decreased over the last few decades, we continue to lag behind in reducing mortality.

*Source: www.commonwealthfund.org*

## Fall for Insurance!

The open-enrollment period for 2019 ACA plans was November 1 to December 15, 2018—a much shorter enrollment period than in years past! You can still get ACA health coverage if you qualify for a special enrollment period due to a life event like getting married, losing other coverage, or having a baby.


## Obamacare Carries On . . .

The ACA is still in effect in 2019. Although President Trump and other key members of government are trying to implement legislation to repeal the ACA and change the American health insurance industry, there have been no sweeping changes for health insurance in 2019.

## Help for Determining Healthcare Costs

The Health Insurance Marketplace Calculator by Kaiser Family Foundation was updated for 2019. It includes local info on health plans being sold through ACA Marketplaces during 2019's open enrollment period. With the calculator, US consumers can generate estimates of their health insurance premiums and what financial help may be available for Marketplace plans (based on household income, family size, ages of family members, zip code). The calculator also helps you see if you could be eligible for Medicaid. Spanish-language version available.

*Source: www.kff.org*

## No More Penalty?

The ACA included an individual mandate, requiring most people to have health insurance, enforced by a federal tax penalty (also an individual responsibility payment). While the health insurance requirement remains, the Tax Cuts and Jobs Act eliminated the tax penalty starting in 2019, so there's no longer a penalty for not having health insurance. Though the federal mandate is gone, several states have implemented their own mandates to have health insurance or pay a penalty through state tax returns.

**Novel Therapies** *from page 5*

## Practice, Practice, Practice?

For some patients, one of the notable benefits of currently available—or soon to be available—novel therapies is that they can be administered subcutaneously with a shot. When novel therapies work well, patients could go months or years without needing to treat a bleed. As a result, patients and caregivers may get out of practice infusing themselves or a loved one. Despite this possibility, some patients and caregivers aren't too worried, especially if they have a lifetime, or a child's lifetime, of experience. When asked if he's concerned about forgetting how to infuse because a novel therapy is working well for him, Stone says, "Sticking my veins is like riding a bicycle or swimming. You only need to learn once."

Genny Moore's nephew Christopher lives with her in Crest Hill, Illinois. She says, "With nearly 20 years of almost daily infusions, it's not a concern. If anything, the break that the veins are getting and the strength that Chris has been gaining with being more mobile will allow the veins to be accessed easier. But if he felt like he needed to, he would practice."

Carri Nease of Essex, Maryland, doesn't foresee any problem infusing her son with hemophilia who is on a novel therapy. "I also have hemophilia, and I treat with factor as needed," she says. "Honestly, as long as I can stick myself, I'll be fine if I need to poke a hole in someone else. So, I don't worry if he has a bleed bad enough that we need to do a dose. We'd be going in for assistance if I couldn't get a vein."

Still, if you're on a novel therapy now, or plan to be in the future, make sure that your insurance company will cover home nursing services in case you suffer trauma and need the help of a medical professional to infuse at home. "Infusion skills are going to evolve over time in previously treated patients," explains Croteau. "Patient and caregiver skills might get rusty, and people would need home nursing support for treatment of a serious bleed, not just a run-of-the-mill joint bleed, but treating a bleed caused by an injury."

The need for access to home nursing support is critical for new patients whose caregivers have never had to infuse them. According to Croteau, for previously untreated patients who treat with a novel therapy, "It's hard to develop infusion skills, as they are more like mild patients in that they don't use factor on a regular basis. They have no opportunity to practice, so they would need a home infusion nurse, especially if they live far enough from their HTC that it would be a burden to travel there to receive infusions." If you are the caregiver of a child using a novel therapy, be sure that you have authorization from your insurance company for home nursing services as needed.

## Factor Abroad

Though your novel therapy may be preventing bleeds, your access to factor in case of a bleed remains essential—especially when you travel. "Patients on novel therapies should not be having breakthrough bleeds, but they should not feel a false sense of security either," says Von Drygalski. "They need to have factor on hand." This may mean you'll need to contact your insurance company for approval of additional doses for when you travel.

"I actually just had this conversation with my nephew since we flew from Illinois to California for HFA's [Hemophilia Federation of America] Symposium," Moore says. "Chris brought the one dose of his novel therapy that he is due for preventative treatment while we are on the trip, and one or two doses of factor in case of emergency."

Consult your hematologist or HTC staff before you travel, and get their suggestions for how much factor you should have on hand during your trip. "I discussed with my hematologist our travel plans for three weeks in South Africa, and we agreed that I only needed to take four vials of factor along with my weekly novel therapy dosing," says Stone. "That, with the understanding that I could have factor shipped overnight in an emergency, made me comfortable. It was enjoyable to not have to travel with a suitcase full of factor, and my wife benefited from more suitcase space to bring stuff back."

NHF recommends that when patients travel, they bring a factor prescription from their doctor that includes contact information along with a letter from their hematologist describing their condition and the medication they need.[4] In addition, the US Centers for Disease Control and Prevention (CDC) recommends that hemophilia patients clearly label all medicine and medical supplies, make a list of their hospital and doctor's contact information, and wear a medical identification emblem, particularly when traveling alone.[5]

Hemophilia is a factor-related disorder. Factor remains an essential and potentially lifesaving elixir, even for people using novel therapies. If you use a novel therapy, or plan to use one that's currently in the pipeline of new products, stick to your treatment regime and log your treatments. You still have hemophilia, and you still may need your old, "gold" friend factor to get you out of a tight spot with a bleed.

4. Elizabeth Thompson Beckley, "Traveling with Medication: Practical Advice to Help Plan Airplane Trips," *Hemaware*, January 1, 2007. 5. "Safe Travel with a Bleeding Disorder," https://www.cdc.gov/ncbddd/hemophilia/travel-safe.html.

My PCP sent a referral for me to be seen by the local HTC in LA. However, my insurance denied it, as well as the appeal, due to "lack of medical necessity." At this point, I didn't have many options, so I figured I should see an in-network specialist.

## "Women are just carriers"

On April 5, 2018, I had my first appointment with an oncologist who supposedly had experience treating patients with hemophilia. I told him about my strong family history and that I thought hemophilia could possibly be the real cause of the menstrual issues I'd been having, and not PCOS, as stated by past physicians.

Although he had received a copy of the official diagnosis, the oncologist told me that it's unlikely for women to have hemophilia, and that women are just carriers. He suggested I be tested by a lab that he trusted. He also suggested I have an ultrasound performed to look at my ovaries. I complied.

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

Feeling a bit frustrated, I asked, "And what about my menstrual cycle?" The doctor acted confused, as if I'd never talked to him about my irregularities and PCOS. He told me nothing could be done about my irregular bleeding. I asked him, "Aren't you alarmed that I have prolonged bleeding when off birth control, and isn't that a symptom of hemophilia in women?"

He then became defensive, and I became even more frustrated because two things became clear: First, the doctor hadn't listened to me during my first visit, due to his assumptions about women with hemophilia; nor had he properly prepared for my visit. When diagnosing me, he spoke as if we had never discussed my family history of hemophilia, or any of the things I'd brought up.

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

In November 2018, I switched insurance plans through a new employer and tried the process again. This journey to finding the underlying issue is important for me. My new PCP, who was also a hematologist, admitted that he didn't think he'd be the most suitable hematologist for me because he was out of practice in that specialty and not familiar with women with hemophilia. He asked me what I wanted to do, and I said I wanted to go to the local HTC. He agreed to send in a referral through our medical group.

Again, my referral to be seen by the HTC was denied by insurance because they believed they had specialists more than capable of providing me the care I needed in-house. I obliged once again, and decided to see a specialist they referred me to in-network. But this time I was a bit more optimistic, because the specialist was a woman and this was one of the best health systems in greater LA. There was no reason I wouldn't be able to find the care I needed…right?

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

## Breakthrough

I really wanted to work with this specialist to figure out the best options for me. A friend of mine connected me with the Women's Bleeding Disorder Coalition, which helped educate me about what hemophilia looks like in a woman.

I thought it would be a good idea to connect my new specialist to the coalition. Surprisingly, she agreed, and I thought, wow, this is great! They provided my specialist with more information on hemophilia in women. I was happy and excited to hear that my specialist had taken that step on my behalf.

But during a follow-up visit, I was taken aback when my new specialist (who I had been bragging about) made comments invalidating the information that was shared with her through the Women's Bleeding Disorder Coalition, because they weren't "medical professionals." I was crushed.

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

I have also been working with Hemophilia Foundation of Southern California to get access to the GHPP (Genetically Handicapped Persons

Program) insurance, which would cover my visits to an HTC. I am currently awaiting a decision and crossing my fingers for a positive outcome. Now, at 28, I just want answers. I just want to know what's the true culprit behind my prolonged menstrual bleeding, and what I can do to control it.

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

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

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**Transitions** *from page 3*

to products, making them slightly different. Some of these changes have involved production processes; and the quest to remove unnecessary human and animal proteins gave us "generations" of recombinant factor products.

Now we have three recognized generations of factor products: *First-generation* products use animal blood proteins in the cell culture medium, and add human albumin, a blood protein, to the final formulation to stabilize the factor. *Second-generation* products stabilize the factor with sucrose, not albumin. *Third-generation* products use no added animal or human proteins, either during processing or in the final formulation.[2] Today, all factor products except for Recombinate and Kogenate® FS are third generation. A special shout-out to Octapharma's Nuwiq®, a recombinant factor VIII product that uses a human cell line instead of an animal cell line in its production process.

Recombinant products, by reducing the potential for viral transmission, are an improvement over past products. And yet first-generation products remain in use, as do plasma-derived products. So far, there seems to be a purpose and a place for all products.

## Growth Spurt!

But consumers want more. Researchers found ways to keep infused factor circulating in the body longer, by extending its half-life—meaning fewer infusions and less burdensome treatment. Eventually, extended half-life (EHL) factor was created. After a relatively calm 10 years in the marketplace with what is now called "standard half-life" or just "standard" factor, EHL factor came along, resulting in a big growth spurt in the life cycle of hemophilia treatment. Hailed as the next best thing, EHL factor was and still is heavily promoted. Biogen created the first two EHL products, which were soon followed by EHL products from Baxter (now Takeda), Novo Nordisk, Bayer, and CSL Behring.

The creation of EHL products seemed like the exciting, wild teen years for our community. Children with hemophilia lived even more normal lives, without infusing so often. Freedom!

## Family Feud

In the midst of this growth spurt and innovation, some major shifts were taking place in the hemophilia marketplace. Companies were separating, divorcing, getting married, taking on lovers, adopting other products. Baxter split off its biological division (which made its factor products) to become Baxalta. Genetics Institute was bought by Wyeth, which was then bought by Pfizer. In the early 2000s, Bayer had divested its plasma division, which became Talecris. Then Grifols bought Talecris (you can still see the primary color bar on the Koate®-DVI box, the same bar that appears on Kogenate FS). Biogen, soon after launching its groundbreaking EHL products, suddenly divested its hemophilia group, which became Bioverativ. Soon after the new sign was up at Bioverativ's headquarters, it was bought by Sanofi Genzyme. Baxalta must have been a very attractive mate, because soon after it was spun off by Baxter, it was snatched up by Shire, an Irish pharmaceutical company. Just when everyone was getting used to Irish brogues, Shire was purchased by the Japanese company Takeda, a pharma titan.

Perhaps no products have changed hands more than those of CSL Behring, starting in the 1980s, when it was Armour Pharmacuetical. And although CSL Behring has remained the parent company since 2007, it has recently abandoned some hemophilia products: Helixate® FS and Monoclate-P®. Its focus is now on EHL products only for hemophilia.

And this is the concern for the future life cycle of hemophilia treatment: Can the market sustain 20 recombinant products? Which products will be removed next from the marketplace? Will product change come from the

2. Recombinate is a first-generation recombinant product. Kogenate FS is a second-generation product. Advate, Adynovate, Afstyla, Alprolix, BeneFix, Eloctate, Idelvion, Ixinity, Jivi, Kovaltry, Novoeight, Nuwiq, Rebinyn, Rixubis, Vonvendi, and Xyntha are third-generation products.

consolidation of companies buying each other and reducing overlapping product lines? Will it come from flagging sales, if one product dominates the market? Or will product change come from a novel treatment—using no factor at all?

## Newest Child

One product getting a lot of attention these days is the newest addition to the family—Hemlibra®, a nonfactor antibody product that mimics the action of factor VIII in the body. It's used for prophylaxis in people with hemophilia A and inhibitors, and in those without inhibitors. The product's clinical success has many patients cheering on Facebook, some advocates cautioning consumers to wait and see, and some doctors already prescribing it for newborns. Hemlibra is called a "market disruptor" for a reason. It's created a whole new way to treat hemophilia: with weekly to monthly subcutaneous injections. And people with inhibitors are reporting no bleeds for weeks, even months in some cases. Will factor concentrate still be needed? Read Wendy Owens's feature article in this issue to learn the answer!

The life cycle of hemophilia treatment has entered a new phase, perhaps similar to young adulthood, where there are many options, and our community has been educated and prepared. We're ready for greater independence from this disorder, and ready to live life more on our own terms.

But like all young adults, we still need advice from our elders, from our blood brothers, from our physicians. And we need to do our own homework. We need to read about current products and new ones that will enter the marketplace. Do you feel informed enough to choose? Will your insurance cover all the new products? Will you switch to another product, if one day your insurance company no longer covers yours?

Stay in touch with the life cycle of hemophilia treatment: name changes of the corporate players, which products belong to whom, and especially, the manufacturer of the product you use. Decisions at the top—by government, by corporations, and by insurance companies—may eventually impact your choice of treatment.

We have more freedom and more choices than ever before, but we need to exercise responsibility, by learning all we can. Because one thing is for certain: we are only in the young adult stage of hemophilia therapy. Prepare for more growth and more change. It's coming.

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INSIDE:
Novel Therapies and Factor

September 2019

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