PEN's Insurance Pulse
Inspiring Advocacy

Inside

From Food to Factor: The Road to New Therapies in Hemophilia

Wendy Owens

Hemophilia treatment has come a long way in the past 100 years. A century ago, if you lived in Chicago, for example, you may have seen hematologist Dr. Gordon G. Burdick. At that time, Dr. Burdick approved the use of lime salts in treating hemophilia. He and other hematologists of his era also used gelatin "to increase coagulability of the blood."[1] Today, gelatin is used in all manner of jiggly desserts and to strengthen nails; lime salt (aka calcium chloride) is used in sports drinks to balance electrolytes. With the rise of the pharmaceutical industry and its scientific advancements, doctors now have many non-food-related options to treat hemophilia, and so do you.

1. "The Medical Treatment of Hemorrhage," *Medical Standard* 37:1 (Jan. 1914).

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Welcome

PEN'S INSURANCE PULSE

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An article I published in the November 2004 issue of PEN, titled "The Coming Storm," predicted much of the insurance and pharmaceutical industry change we are now seeing. I could call an updated version of this article "The Winds of Change"! Looking back over the past 30 years, I believe we are seeing more change now, at high velocity. In just the past 18 months, we've seen new factor VIII and IX products launched; a revolutionary treatment, Hemlibra®, which isn't even a factor product; gene therapy successes in clinical studies on the rise; products like Monoclate®-P and Helixate® FS no longer being manufactured; Baxter spinning off its biotech division, which became Baxalta; then Baxalta being bought rapidly by Shire; Shire now considered for takeover by Takeda; Biogen selling off its hemophilia division, which became Bioverativ; and Bioverativ being bought by Sanofi. Is your head spinning yet?

In our feature article, Wendy Owens makes sense of one key question: Given all the product changes, and especially new products like Hemlibra, how will the insurance industry react? How will it handle higher prices as it struggles to keep costs down? Try to understand the issues involved, while you consult with your hemophilia treatment center (HTC) team about new products and whether they'll be covered under your current insurance policy.

Read our Community Forum, where experts debate how payers will cover the new products and subsequent price changes. And in our column My Life, learn how one mom and her medical team fought the good fight, and won—when an insurer tried to monkey with lowering her three sons' dosage by 65%.

What's the best way to maintain access to healthcare and affordability? Read. Engage your HTC team, your state hemophilia organization, and our national organizations. And be prepared! The winds of change can easily shift to hurricane force in this uncertain and political healthcare landscape. Luckily, we have resources available and a community of experts ready to assist.

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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The Cost of Working with a Bleeding Disorder

Shelby Smoak

One of life's biggest transitions is growing into adulthood and entering the job market. You may embrace the idea of growing up and moving out, but you may fail to appreciate the cost of living. Transitioning becomes even more stressful for you as someone with a bleeding disorder, because the need for health insurance must be (forgive the pun!) factored into career choice. Preparing a budget estimate for living on your own and understanding a potential employer's insurance policy are top priorities to make your transition into the job market and independence as successful as possible. To estimate the cost of living, let's use national financial averages to provide strong baseline figures.

Start by considering what everyone transitioning into the job market must consider—minimum living expenses. Americans spend almost 62% of their income on three things: housing, transportation, and food.[1] Though it varies depending on where you live, the average rent for a one-bedroom apartment is $1,040 a month, or $12,480 a year.[2] Transportation costs can vary too, but except in dense cities, you'll probably need a car for commuting, with a car payment around $400 a month or $4,800 a year. The national car insurance average is $76 a month, $912 a year.[3] Food will be about 12% of your budget and, while this is flexible if you survive on a steady diet of Ramen noodles, an average monthly cost for food is $583 or about $7,000 a year.[4]

Now let's add utilities and phone expenses. Although the cost of utilities may vary depending on the season, the average electricity cost is $110 a month or $1,320 a year.[5] And the average cost of mobile phone service is around $50 a month, or $600 a year.[6]

All this adds up to $27,112, a budget baseline for successfully striking out on your own in an average town. This figure is probably higher than what you thought you'd spend on necessities for a year. Some costs can be tweaked and will vary with your own budget, but the math doesn't lie. It's expensive to live on your own.

Luckily, the average salary for young adults entering the job market provides this earning capacity: people with a high school education typically earn $31,800; with a college degree, this jumps to $50,000.[7] But these figures fail to address the biggest expense—healthcare! The most important consideration in your job search isn't salary. It's health insurance.

Finding a job with health insurance is a win, but there are things to consider. First, you'll need to understand the out-of-pocket costs related to premiums, deductibles, copays, tiers, and coinsurance. Our community has many resources to help you understand, but these costs will be a burden. Second, you'll need to know the difference between "fully insured" and "self-insured." A company that is fully insured offers advantages over companies that self-insure. Fully insured is the standard insurance model, in which the employer pays a monthly premium to the insurer, who then handles the claims and billing. Most small businesses are fully insured, as are most larger companies. But the premiums are guaranteed only for the policy year, and each year the insurer will usually renegotiate the premium based on factors including the cost of claims submitted by a company's employees. High-cost

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1. Alex Morrell and Skye Gould, "A Close Look at Americans' Food Budget Shows an Obvious Place to Save Money," Business Insider, Feb. 17, 2017, businessinsider.com. 2. "America's 2017 Rental Market in Review," ABODO, Jan. 3, 2018, abodo.com. 3. "Average Auto Loan Interest Rates: 2018 Facts and Figures," ValuePenguin, valuepenguin.com. 4. Morrell and Gould, "A Close Look." 5. "Average Monthly Electric Bill by State–Updated Data," NAHB: Eye on Housing, Mar. 12, 2015, eyeonhousing.org. 6. "Cell Phone Plan Cost," Cost Helper Electronics, electronics.costhelper.com. 7. "Annual Earnings of Young Adults," National Center for Education Statistics, Apr. 2018, nces.ed.gov.

From Food to Factor *from cover*

Today, there are 31 different FDA-approved therapies on the US market for hemophilia. In addition to the FDA-approved therapies, as of this writing, there are 14 new factor therapies and three "novel" therapies, other than gene therapy, in some stage of drug development for treating hemophilia. The novel therapies use mechanisms other than factor replacement to treat and prevent bleeds, and some can be administered subcutaneously.[2] Once approved, one of the new therapies may be right for you; but there are steps you, and others, must take for you to have ready access to them through your health insurance plan.

Start with what and who you know

If you want to use a new factor replacement product or novel therapy, talk to your hematologist to see if it's right for you. Do some research beforehand, so you're prepared for the discussion. Biopharmaceutical companies with new therapies in clinical trials, or with ones newly approved by the FDA, will have data and information on these therapies available on their websites. A heads-up: what you find on such sites will be data and information these companies legally can provide, like data from their clinical trials. These websites will not provide medical advice, but will give you a basic idea of how a treatment may work and how it performed in clinical trials.

Based on the advice of your hematologist, if you decide to switch to a new therapy, accessing it through your health insurance policy's prescription drug plan is your next step—or hurdle, in some cases. You'll need to find out if your health insurance will cover the new therapy. Remember, just because a new therapy receives FDA approval doesn't mean it's immediately available to you via your insurance plan. Your health insurer needs to be sure that any new FDA-approved therapy is safe, works as well or better than other therapies used to treat the same disease or condition, and is cost-effective. With some exceptions, health insurance companies cover a full range of—but not all currently available—FDA-approved clotting factor products for treating hemophilia. But there is no guarantee that new factor replacement products and novel therapies will enjoy the same coverage range as current clotting factor products.

According to Jennifer Luddy, director of corporate communications at Express Scripts, the largest pharmacy benefits manager (PBM) in the US, "There are a variety of agents to address the various needs of patients with hemophilia on our formulary, based on type of hemophilia, past therapy, and presence of inhibitors." But will Express Scripts and other PBMs and insurers cover new factor therapies and novel therapies once they are approved? That remains a question to be answered only by an evaluation process to which all new drugs are subjected.

To cover or not to cover?

FDA approval of a new medication or therapy does not mean that it will be covered by all health plans. Health insurance companies and PBMs are the gatekeepers for a drug's availability to patients. Let's take PBMs as an example. PBMs are massive, multibillion-dollar companies that manage drug costs for their clients. PBMs' drug formularies include FDA-approved medications and therapies available only by prescription. The drugs on a PBM's formulary, or list, are covered by insurance plans that offer prescription drug benefits. To make this list, an FDA-approved medication or therapy must be evaluated for clinical appropriateness first—how safe it is and how well it works—and cost second.

This evaluation process uses a combination of data on a drug's effectiveness and treatment value to reach a decision about whether to put that drug on a formulary. "It's a clinical-first process that relies on the recommendations of an independent group of physicians and pharmacists before cost considerations," Luddy says. "All medications are subject to this process."

Before the drug reaches a formulary, a panel of independent experts called a pharmacy and therapeutics (P&T) committee looks at the clinical appropriateness of a new drug. The P&T committee reviews drugs for all conditions, including hemophilia. P&T committees can include nurses, doctors, pharmacists, and

2. As of this writing, the two novel therapies in clinical trials are Alnylam's fitusiran (ALN-AT3SC), an RNA interference (RNAi) therapy, in phase I/II of clinical trials; and Hemlibra® (emicizumab-kxwh), a bispecific monoclonal antibody, in phase III of clinical trials and granted priority review by the FDA for use by adults and adolescents 12 and older with hemophilia A without factor VIII inhibitors.

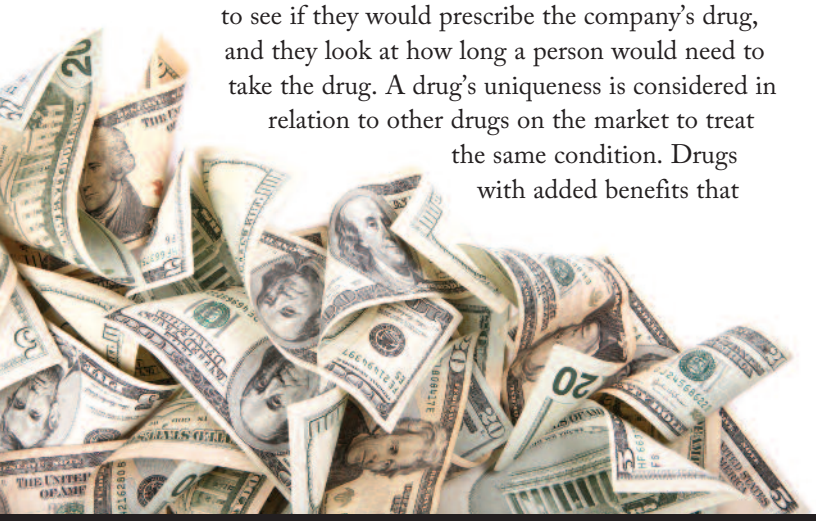
other clinical experts. The P&T committee for the PBM CVS Caremark, for example, is composed of 22 independent healthcare providers, including practicing pharmacists and physicians.[3] The P&T committee reviews safety data, clinical trial results, doctor recommendations, and FDA-approved prescribing information developed by the pharmaceutical company making the drug.

The P&T committee is *not* interested in the cost of a new drug. Instead, the committee's goal is to determine whether a new drug is safe for use, and whether it performs better than other drugs currently available to treat the same condition. Each decision by a P&T committee for each drug is either an "include," "exclude," or "optional for inclusion" in a formulary. According to Express Scripts, 15% of the drugs on its formulary receive an "include" designation from the P&T committee, with the other 85% being "optional for inclusion." Fewer than 1% of drugs available in the US receive an "exclude" designation from the Express Scripts P&T committee.[4]

Show me the money

If a P&T committee advises an insurance company or PBM to add a drug to its formulary as "include" or "optional for inclusion," then it's the job of the insurer or PBM and the pharmaceutical company providing the drug to arrive at a price. According to various studies, the average cost to manufacturers for developing a new drug is $648 million to $2.7 billion, a cost that often depends on what disease or disorder the drug will treat.[5] So how do insurers and pharmaceutical companies, who would like to recover the research and development cost of the drug as well as earn a profit, agree on what a drug should cost insurers?

The answer is simple. Pharmaceutical companies price a drug for as much as the market is willing to pay for it. These companies do their homework and look at the price of drugs similar to theirs. They talk to doctors like your hematologist to see if they would prescribe the company's drug, and they look at how long a person would need to take the drug. A drug's uniqueness is considered in relation to other drugs on the market to treat the same condition. Drugs with added benefits that may have a big impact on a patient population and lower healthcare cost overall can be priced higher.



For example, if a new drug has the potential (or has proven through clinical trials) to change the current practice of medicine used to treat the conditions the drug targets, it could be more expensive, like the hepatitis C treatment Sovaldi®, which is a cure. A drug also is special if it can prevent the need for certain medical treatments or the necessity for surgeries or other procedures. Drugs that can cut down on expensive surgeries, hospital trips, and doctor visits for patients are often priced higher because of the savings they offer customers and insurers on these types of medical expenses. Drug companies also issue higher prices for drugs that can extend or even save lives. To help their chances of getting a drug on an insurance company's or PBM's drug formulary, pharmaceutical companies may negotiate drug rebates to ensure that the drug ends up as a "preferred drug" on a formulary. Preferred drugs cost insurers and patients less, so patients are more likely to choose them. Another way drug manufacturers balance revenue generation is by pricing drugs low at first, and then increasing the price at steady intervals.

What? More obstacles to new drugs?

So to recap, here are the hurdles a new factor therapy or novel therapy must clear to reach you, the patient: (1) the treatment must receive FDA approval; (2) a P&T committee must give the drug a thumbs-up to "include," or thumbs-sideways for "optional for inclusion" on a formulary; (3) an insurer or PBM and the drug company must negotiate a price to be paid for the new drug. Now you can get that drug, right? Well, not exactly. You can face your own obstacles to receiving a new therapy, and these may be put in place by the same thumbs-up or thumbs-sideways decision group—the P&T committee.

CVS Caremark says, "The physician always makes the ultimate prescribing determination as to the most appropriate course of therapy."[6] But beware. It is a P&T committee that reviews and approves all utilization management (UM) criteria for a drug. UM criteria is a set of techniques used by insurers to manage costs. UM criteria may include prior authorization, step therapy, and quantity limits outside of FDA-approved labeling, which specifies dosing sizes and frequency.[7] What this means is

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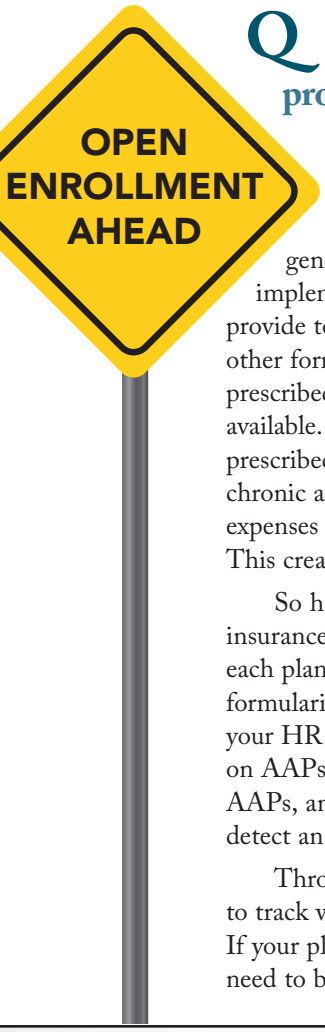
3. Formulary Development and Management at CVS Caremark, https://www.caremark.com/portal/asset/FormDevMgmt.pdf (accessed Mar. 5, 2018). 4. "How We Build a Formulary," Express Scripts. Available at lab.express-scripts.com (accessed Mar. 21, 2018). 5. Mathew Herper, "The Cost of Developing Drugs Is Insane. That Paper That Says Otherwise Is Insanely Bad," *Forbes*, Oct. 17, 2017. Available at www.forbes.com (accessed Mar. 21, 2018). 6. Formulary Development and Management at CVS Caremark (accessed Mar. 10, 2018). 7. As part of a drug approval by the FDA, the drug's manufacturer must specify dosing sizes and frequency. Treatments are prescribed based on these specifications, but if a doctor wants different dosing for a patient, prior authorization may be needed.

Ask the Expert

Kim Isenberg
Vice President, Policy and Advocacy, Hemophilia Federation of America (HFA)



Q I'm hearing more and more about accumulator adjuster programs (AAPs). As we move into open enrollment this fall, how do I find out if my health insurance plan has an AAP?

A Accumulator adjuster programs are part of a strategy to drive patients to a generic drug in order to contain costs that pharmacy benefit managers (PBMs) are implementing around the country. AAPs are part of the benefit design that PBMs provide to health insurance plans, and apply to patients who use drug copay cards and other forms of manufacturer copay assistance. AAPs are still applied to patients who are prescribed specialty products such as clotting factor even though there are no generics available. Under an AAP, a PBM accepts the manufacturer copay assistance for out-of-pocket costs associated with a prescribed drug, but then *doesn't credit that amount toward the patient's overall deductible*. This means that patients with chronic and expensive disorders will still be required to personally pay deductibles, copays, and other out-of-pocket expenses up to the yearly out-of-pocket maximum, even as the health plan draws down the full amount of the copay card. This creates a huge financial burden for patients and their families.

So how do you find out if your health insurance plan has an AAP? Many employers provide a choice of health insurance plans during open enrollment. Prior to enrolling in your 2019 health insurance plan, review the policies for each plan offered and all plan documents. Make sure you have copies of the summary of benefits and coverage, drug formularies, and provider network. Most of this information is available online. Fully understand your options. Ask your HR department or call the insurance plan directly if you think you need more guidance or can't find information on AAPs in your plan options—and keep pressing for clear answers. Note that there is no industry standard name for AAPs, and some plans use euphemistic titles such as "Out of Pocket Protection Program." This can make it hard to detect an AAP in your plan. Finally, don't wait until the last minute to enroll. Start researching as early as you can.

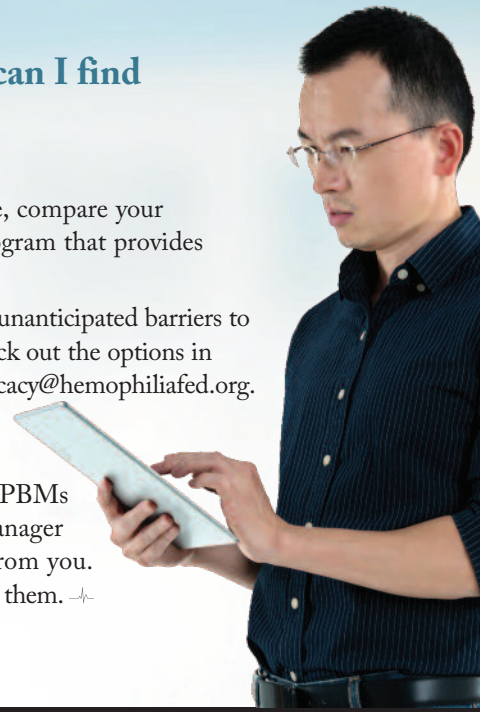
Throughout your plan year, closely watch your Explanation of Benefits (EOB) notifications. You should be able to track whether the copay assistance payment is being applied to your deductible and/or out-of-pocket maximum. If your plan has an AAP, this means you'll be billed for your copay after your copay assistance is depleted. You may need to budget for that unanticipated out-of-pocket cost or seek additional financial assistance.

Q My health insurance plan is provided by my employer, so how can I find resources to help with the financial hardship an AAP creates?

A Before enrolling in your employer's health insurance plan, explore your options. For example, compare your employer's plan to your spouse's plan. Or find out if your state has a chronic disease assistance program that provides assistance with out-of-pocket costs.

AAPs can leave people who live with expensive chronic conditions, like bleeding disorders, with unanticipated barriers to treatment when an individual or a family can't pay the out-of-pocket cost. If this happens to you, check out the options in HFA's Resource Library: Navigating Patient Assistance Programs.[1] Or contact HFA directly at advocacy@hemophiliafed.org. You may also want to see if your specialty pharmacy provider can suggest any sources of assistance. A new patient assistance fund[2] may be able to help with expenses if you're faced with an AAP.

HFA and National Hemophilia Foundation (NHF) are working to educate health plans and PBMs about the dangers posed by AAPs. If you have received a letter from your employer or benefits manager stating that your copay cards will no longer be applied to your deductible, HFA needs to hear from you. Please share your story with Project CALLS.[3] Collecting data about these issues is the only way to fight them.



1. www.hemophiliafed.org (search "navigating patient assistance programs"). 2. panfoundation.org/index.php/en/about-us/media-room/patient-access-network-foundation-opens-new-hemophilia-patient-assistance-fund (accessed July 17, 2018). 3. www.hemophiliafed.org (search "Project CALLS").

Dosage Denied! Where Does Insurance's Power End?

Laurie Kelley

Samantha* lives in Montana, our fourth-largest state but with one of the lowest populations, at 1.05 million. This means that families with hemophilia have an extra burden of traveling long distances to a hemophilia treatment center (HTC). Samantha's three sons with severe hemophilia, ages 18, 13, and 11, were used to the 11-hour drive to Denver's renowned HTC and a 7-hour drive to the closest hemophilia hematologist.

What they were not used to was a sudden denial by their insurance company of their hematologist-approved factor dosage.

On January 1, 2016, Samantha switched to a new insurance company, Assurance*, which was registered with the state exchange, or Marketplace. Six months later, Assurance made a curious request. The company asked Samantha to provide the weights of each of her three sons. This seemed odd, because Samantha always provided weights when she ordered factor, but she complied. Then her hematologist and specialty pharmacy received the same request from Assurance. The hematologist sent the weights, along with a three-month prescription. But Samantha was baffled.

The reason for the company's request soon became clear. On July 11, 2016, Assurance sent a letter to Samantha's specialty pharmacy and hematologist explaining that the boys were using factor outside the recommendations of the product insert (PI). The PI is the set of instructions and medical information provided by the manufacturer inside each package of factor. The insurance company claimed that by using more factor per dose than was recommended, the family was acting outside of FDA regulations.

Shortly after, Assurance sent Samantha a letter declaring it would only authorize the average dose according to the PI, contradicting the prescription of the boys' hematologist, and cutting the hematologist-ordered doses by 65%.

This letter shocked Samantha. "I reached out to Assurance via email to ask what was going on," she recalls. "The company replied, said the hematologist had been notified, and I needed to contact the hematologist and have her reissue a script at a lower dosage. The insurance company kept me out of loop as much as possible."

But Samantha was smart: she made notes on every phone call and documented everything.

Then Assurance sent a one-page letter about the dosage change to the hematologist and specialty pharmacy. Although Samantha requested a copy, she didn't receive one; her specialty pharmacy finally forwarded her a copy.

The letter stated that Assurance had an external review board investigate the case, but deceptively, the insurance company requested only a review of the PI's *average dosing*, neglecting to send the bleed history of each boy and the recommendations of the hematologist! The letter did not verify who this review board was, or what outside consulting company was used and whether it had included hematologists knowledgeable about hemophilia.

Samantha knew a fight was coming. Luckily, she was prepared. Her hematologist had recommended using an amount higher than the dose shown on the PI. Each boy's dose was well documented. "We have 150 pages of documentation showing why each of our sons is on doses higher than the PI," says Samantha. "Their half-lives, their recovery studies were all done consistently. So the scientific and medical facts were there."

The changes recommended by Assurance were shocking: Samantha's youngest son had been prescribed 130 IU/kg of recombinant factor IX, three times a week. Assurance changed his dose to 80 IU/kg twice a week, a decrease of 67% per month. Another son was prescribed 150 IU/kg, three times a week. Assurance decreased this by 64.5%, down to 80 IU/kg, twice a week.

Samantha notes, "My boys are very athletic and extremely toned; there's no obesity. They are big boys. My youngest now weighs 184 lbs and is almost six feet tall. They needed the dosage prescribed by their hematologist."

Meanwhile, Samantha's world became more stressful. "Raising three boys, fighting this battle, it was overwhelming!" Within one week of the lower-dosage change, all three boys had bleeds, flu-like symptoms, aching joints, and headaches. "My oldest son told me everything aches inside him. My middle son took a shower late one night.

*Names have been changed due to pending legal action.

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Community Forum

How will payers respond to coverage of new therapies?

Community forum contributors represent unique perspectives and areas of expertise in the bleeding disorder community

Elizabeth Stoltz
Advocacy & Policy Consultant
National Hemophilia Foundation (NHF)

My crystal ball is a bit foggy, but here are my predictions:

First, payers (insurance companies, state Medicaid programs, self-insured employers, and others) will probably pay for new therapies, including gene therapy, *but* the payers will very likely have prior authorization criteria that define which patients can access these products.

Second, new therapies probably won't be available immediately after FDA approval. Why? Gene therapy products will probably be expensive. Payers will want time to understand how they work and for whom they will be appropriate. For example, one gene therapy clinical trial excludes patients with inhibitors. Another excludes patients with HIV. If a product hasn't been tested in a segment of patients, then payers and physicians probably won't use it for those patients; it's a matter of safety.

Now, let's go back to cost: Payers don't have endless buckets of money. A commercial insurance company's "income" is made up of the monthly premiums, copays, coinsurances, and deductibles that we pay. Its "expenses" are the medical claims paid to providers (including doctors, hospitals, pharmacies, and labs) *and* everything else it takes to run the business (employee salaries, building rent, and so on). See the boxes above for a quick fictional example.

In 2017, XYZ insurance company's income was $50 million from its members, which include 10 people with hemophilia.

XYZ paid out $45 million for claims and $3 million for business expenses, leaving a profit of $2 million. Let's say of the $45 million, hemophilia represents expenses of $6 million.

In 2018, XYZ expects the same $50 million income and $3 million business expenses. Pretend that a hemophilia gene therapy gets FDA approval, and the cost is $1 million per patient. If five of XYZ's members are eligible for gene therapy, that would cost the insurance company $5 million. Subtract out the usual expenses for five hemophilia patients, say at $3 million annually, and XYZ would have no profit. Any business would be challenged to have no profit, and potential loss, year after year.

What does this mean to our community? Everyone who is eligible for any expensive therapy may not be able to get it immediately.

What can our community members do if they want to receive a new therapy?

- Talk to your hemophilia treatment center (HTC) team or hematologist. They can help you understand if a particular product might work for you.
- If you and your medical team decide to move forward, they will have to justify to your payer why this is the right therapy for you. This takes time, and your insurance company may or may not approve it.
- Be prepared to help your medical team make the case for you. That might include extra tests, *accurate* factor logs, and other documentation.

Remember that insurance companies are *not* the enemy! To get the best care, you and your healthcare team need to work with them, not against them.

NHF has initiatives in place to educate payers on bleeding disorders and also to enhance the relationship between these payers and HTCs. As a community, we need to be responsible stewards of healthcare dollars while getting excellent care. Working together, we can make bleeding disorders as small a part of our lives as possible.

Joe Pugliese
President and CEO
Hemophilia Alliance

I don't think there's a real distinction between how payers are going to approach the marketplace based on the type of provider you are—340B, HTC, or specialty pharmacy. Payers are most concerned about the rising cost of therapy, and probably less concerned about who they are reimbursing. And they're spending more money! Confounding the issue is that increasingly, private insurers own their own specialty pharmacies, and there's a poor alignment of incentives. For instance, OptumRx is a very profitable division of UnitedHealthcare, the largest healthcare company in the world. Optum benefits from higher pricing of factor. Say you make a 25% margin. On $1 a unit, you make only 25 cents; but if the price is $2 a unit, then you make 50 cents—twice the profit. It'll be interesting to see how the internal conflict of interest for payers plays itself out.

Having numerous product options creates concern and some confusion among clinicians and patients. If you've been well managed on a specific product for the last 10 or 20 years, you're probably most comfortable staying on what you know works for you; that's true of both clinicians and patients. But clearly, clinicians to date have embraced new therapies, and there are always early adopters of any new product. Increasingly, with more therapies, the clinician has to consider the best way to manage each patient. So there are personal and health considerations as well as cost issues. For example, Hemlibra®, the new hemophilia A prophylactic therapy for people with inhibitors, is a great advancement; but it comes with potential challenges, and clinicians are cautious about making changes until they are comfortable they are doing no harm.

Regarding gene therapy, everything I've heard suggests that we'll have some sort of staggered payments based on a minimum level of expression of factor levels over a period of time. Let's say the proposed cost for gene therapy treatment is $1 million. Nobody wants to pay $1 million up front, and then find out two years later that they have zero expression of factor. It may be that you have to maintain an expression level of not less than 20% over five years. To be fair to the payer, who wants to pay $1 million if 12 months later, there's no increase in factor levels, or if the insured changes insurance companies?

But it really becomes this question: Who do you contract with? Are you contracting with an HTC? Working directly with the gene therapy company? If so, then in the case of an HTC, is there revenue potential for the HTC in the gene therapy model? The patient will still need follow-up, and breakthrough bleeds will need treatment. I don't think that piece has been worked out yet.

Clinicians don't make treatment choices based on the cost of care. They make decisions based on what they think is the best clinical option for their patients. This is why newer products have enjoyed a high level of success. Clearly, they're more expensive than older therapies. But the real question is, what are the insurance companies going to do? We're now finding hemophilia lumped in with all sorts of other diseases that now have really expensive therapies. We used to be the only ones who had a disorder with an expensive solution. Increasingly, there are more and more diseases with expensive solutions. That's what I think is really driving the focus on how you try to manage this cost.

Michael Bradley
Vice President of Business Development
The Access Group

Currently, public and private insurance companies are trying to figure out what the future of healthcare will look like while at the same time dealing with the high cost of drugs, especially specialty drugs, such as clotting factor concentrate.

The hemophilia marketplace has seen significant changes over the past several years. In the US, there are now more than 16 factor therapies for hemophilia A and 11 for hemophilia B. Late last year, the FDA approved the first non-factor therapy for prophylaxis in people with hemophilia A and inhibitors. Other therapies are waiting in the wings, and it's assumed that within the next five years, gene therapy will become a reality.

When determining insurance coverage for factor, a payer will assess the safety of the product (is it safe to infuse, and does it have side effects?), the clinical benefit (does it work?), and ultimately, the price (how much does it cost per unit, per patient, per year?).

For non-factor therapies, payers will need to fully understand the clinical benefits and to determine safety. Payers will also need to determine the cost, which can be a bit tricky since these products can't be directly compared to other factor products on a per-unit basis. As with any new therapy, it can take a while for an insurance company to agree to include the product in their formulary as a covered benefit.

The development of gene therapies represents a new frontier in medicine, with the potential to help many patients with serious or even fatal conditions. This state-of-the-art therapy may offer

continued on page 15

Tracks & Trends

Good News!

Patient Services, Inc. (PSI), a nonprofit dedicated to providing health insurance premium support, paid $92.6 million in direct program services in 2017, including support to bleeding disorder patients. An average of $4,795 was paid to individual patients in 2017.

PSI Annual Report 2017

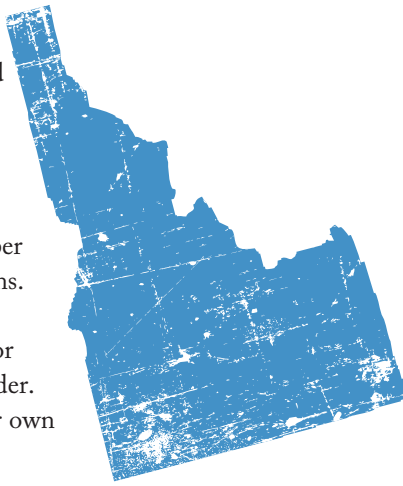
Bad News!

Centers for Medicare and Medicaid Services (CMS) has blocked payments from PSI and similar nonprofits, and has been sued over this ruling.

Oh no, Idaho

An executive order by Gov. Butch Otter allows Idaho to offer state-based health plans (SBPs) that are exempt from many of the Affordable Care Act (ACA) protections for people with pre-existing conditions—like hemophilia. SBPs will provide a cheaper alternative to the ACA-compliant plans. They will not provide essential health benefit standards or cover treatment for diabetes, HIV/AIDS, or bipolar disorder. Idaho is one of 17 states that run their own Marketplace exchange.

chirblog.org

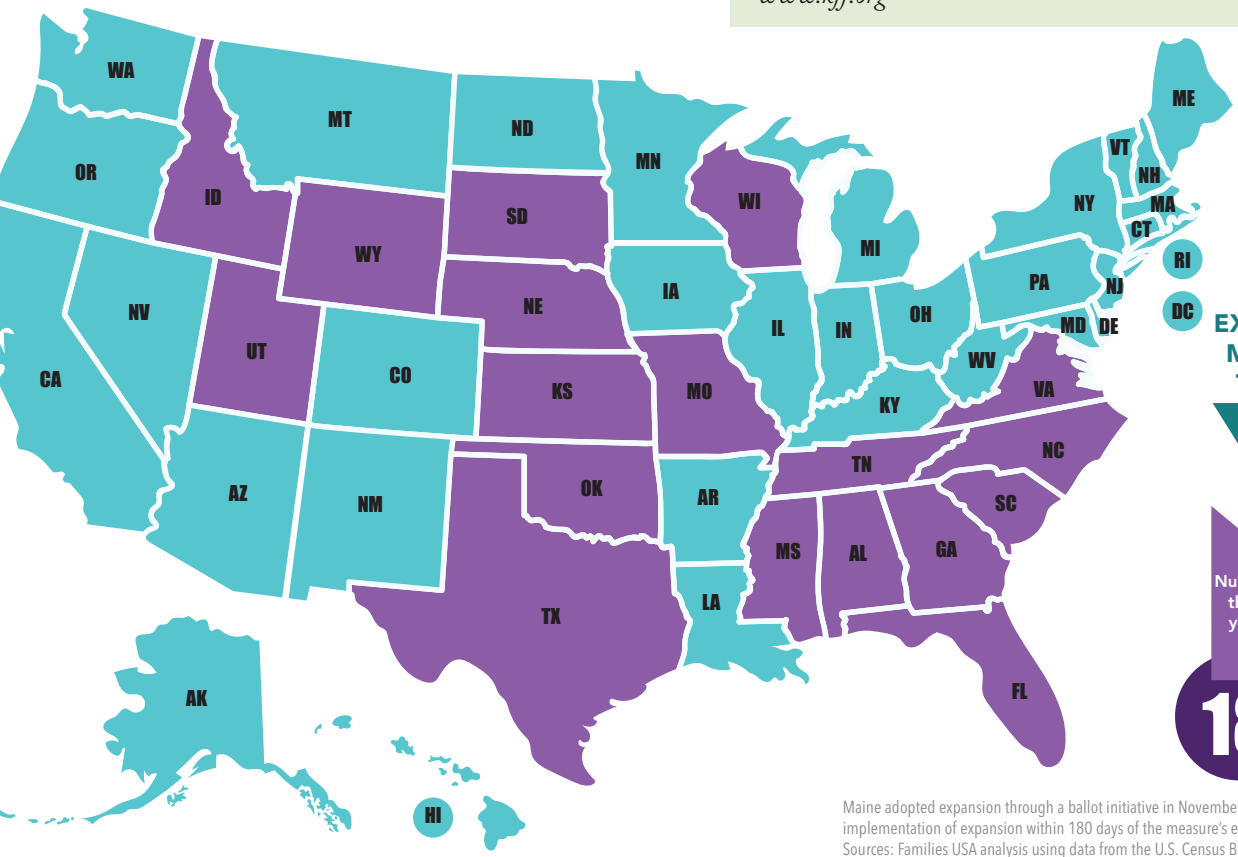


ACA Benefits Women

Since the Affordable Care Act was signed into law in 2010, the uninsured rate among women has dropped sharply, along with major increases in Medicaid in 33 states and in private insurance coverage. In 2013, Kaiser women's health survey found almost 1 in 5 non-elderly women were uninsured. By 2017, this dropped to 1 in 10.

www.kff.org

Which states expanded Medicaid under Obamacare?



33 STATES EXPANDING MEDICAID TO DATE

Number of states, including the District of Columbia, that have expanded Medicaid

18 STATES NOT CURRENTLY EXPANDING MEDICAID

Number of states that have not yet expanded Medicaid

Maine adopted expansion through a ballot initiative in November 2017. The ballot measure requires implementation of expansion within 180 days of the measure's effective date.
Sources: Families USA analysis using data from the U.S. Census Bureau American Community: Kaiser Family Foundation. State Health Facts. Medicaid Expansion Enrollment, January-March 2016, available online at kff.org.

familiesusa.org/product/50-state-look-medicaid-expansion

Who insures you?

For people with bleeding disorders, employer-based insurance covers about 56%, Medicaid 16.6%, Medicare 7.4%, Marketplace 10.1%, state-sponsored 2.7%, military 1.8%. Almost 5% didn't know how they are insured.

www.hemophiliafed.org

Did you know?

Centers for Medicare and Medicaid Services, in the Department of Health and Human Services (HHS), is responsible for implementing Medicare. Medicare helps pay for the healthcare needs of 59 million people, including adults over 65 and younger adults with permanent disabilities.

Eye-Popping Premiums

Based on insurers' requested premium increases, nine states are raising their premiums in 2019, ranging (before tax credits) from 7% to 36%: Maine, Maryland, New York, Oregon, Rhode Island, Vermont, Virginia, Washington, plus Washington DC.

www.kff.org

Fall for insurance!

The open-enrollment period for 2019 ACA plans is 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 2018. 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 2018.

Which side are you on?

According to Hemophilia Federation of America, 20% of families with bleeding disorders polled don't know if their factor is billed to the pharmacy side or medical side of their insurance plan.

www.hemophiliafed.org

From Food to Factor *from page 5*

that, after discussing pros, cons, and options of new therapies with you, your hematologist makes the prescribing determination, but you still might not have immediate access to a new therapy.

Prior authorization requires your doctor to tell your health insurer why you need a specific medication, and the health insurer has to agree that you need it. If your health insurer approves the requested authorization, the approval may be valid for only a set period of time and, when that time is up, may require reapproval. Prior authorization is one way insurers and PBMs try to keep costs down. Unfortunately, having to get prior authorization when a therapy is first prescribed, and reapproval at whatever time interval is required, can slow your access to treatment.[8] Find out whether a new therapy requires prior authorization before deciding to switch. Be prepared by having enough of your old product on hand to cover any delay in getting the new therapy.

Another barrier you may face is step therapy, sometimes called "fail first." Step therapy requires a patient to try and fail on typically less expensive therapies in a stepwise process before he or she can receive a new, more expensive therapy or another drug not on the formulary. According to Blue Cross Blue Shield of Michigan, for example, medications that require the use of step therapy can include those with serious side effects and those that can be misused or abused.[9] The try-and-fail process is another way for insurers to keep drug costs down. This tactic is not uncommon for hemophilia patients, despite the serious impact it can have on bleed management, joint health, and quality of life. Check with your health insurance plan to find out if you must receive prior authorization or undergo step therapy to get the new treatment you want to use.

How much "change" will a change cost you?

Let's say you now can switch to a new therapy because you and your hematologist have agreed that switching to this therapy is a good option for you, your insurance covers it, and no other barriers exist to your using it. How much will you pay for this new therapy? What you pay is dictated by the prescription drug coverage portion of your health insurance plan. It's critical that you review your health insurance plan information every year at open enrollment. The plan you select must meet both your health needs and your budget. You can check which drugs are available on your plan's formulary at open enrollment, or at any time during the year, by calling your health insurer or visiting its website.

Check to see how much you will pay for a new therapy before filling your prescription. Most health plans use a cost-sharing formula for drugs, in which drugs are placed into different cost-sharing levels, called tiers. Generally, drug formularies are broken down into four to six cost-sharing tiers:

- Tier 1: You may pay a $10–$20 copay for drugs that normally are very low-cost and mostly generics (there are no generics to treat hemophilia).
- Tier 2: You may pay about a $40 copay for higher-cost generic drugs and low-cost brand-name drugs.
- Tier 3: You may pay about a $60 copay for brand-name drugs for which there are no generics.
- Tier 4: You may pay a $100-plus copay or a coinsurance payment of 10%–40% of the cost of a drug for highest-cost drugs or specialty drugs (drugs on this tier are usually biologic drugs, like therapies used to treat hemophilia).

It's important to determine on which tier a new hemophilia therapy appears. Part of your decision to switch treatment should be your out-of-pocket cost for the new therapy. Beyond knowing how much you'll pay for a new therapy, watch out: you may have to pay full price for a new drug until you meet your plan's deductible.

Raising the bar on quality of life

It's a very exciting time in hemophilia treatment. New therapies could be incremental steps up in your quality of life or have positive, life-changing impacts. You have now, and will have in the future, no shortage of treatment options. And this means you'll have decisions to make. But your healthcare plan can limit or restrict some of those options. Do your homework, talk to your hematologist, and verify access to the therapy of choice and its associated costs.

And please avoid anyone who suggests you use only remedies found in a grocery store to treat your hemophilia!

8. Wendy Owens, "Could Cost Controls Prevent Access to Your Factor Brand?" *PEN's Insurance Pulse*, Sept. 2015. 9. "Prior Authorization and Step Therapy Coverage Criteria," Blue Cross Blue Shield of Michigan, June 2018. Available at www.bcbsm.com (accessed June 5, 2018).

Afterward, it looked like a blood bath. He had horrific bloody noses. He was ghostly white. Finally we started taking [our sons] to the ER, even for nosebleeds, which we normally treat at home. We weren't going to the ER for products (the ER doesn't stock them), but for documentation—proof of their bleeds for the insurance company!" The ER doctor told Samantha that her sons needed factor *now*. "We told the doctor we couldn't get the medicine in the correct dosage. Then he wanted to get involved in the fight!"

Samantha's youngest son had his first-ever joint bleed in his knee, which bled for a month. He was on crutches for six weeks and needed physical rehabilitation. "We started keeping the boys inside, like in a bubble, so they wouldn't risk getting hurt. Yet, the boys had spontaneous bleeds."

Assurance tried many tactics. The company didn't return Samantha's phone calls. It denied and even delayed preauthorization from the hematologist, denied bleed doses, and once, incredibly, even refused to send factor.

Despite the barrage of phone calls to Assurance from the specialty pharmacy, hematologist, and Samantha, the request for the prescribed dosage was denied. Together, Samantha and her healthcare team filed a grievance with the state against Assurance, following the online procedure. "Assurance became belligerent, to be honest," Samantha remembers. "It was horrible. We said this change in dosage was life-threatening, and they said it wasn't their problem. Our hematologist had never heard of any insurance company changing a physician's script. She would have screaming phone calls with them. But they just would not budge. It was all about the money. They said, if we weren't happy, we could go elsewhere. They wanted to get rid of us."

After filing the grievance, Samantha contacted her state insurance commissioner's office. She filed an online appeal. It was tricky, because applications are limited to a certain number of characters. But Samantha was clever: she filed an appeal for each child separately, giving her more space to describe what was happening. Samantha also contacted Michelle Rice, vice president, public policy and stakeholder relations of National Hemophilia Foundation, for advice about including key words and points.

A second external review was requested by the commissioner. When Samantha received the results of this review, she couldn't believe it. "It was nuts! It *agreed* with the dosage change by Assurance, and this time we knew who the company was that conducted the review. What info did they base this on? What info was given to the external review company? What do they know about my boys? I started crying. My kids could die."

Samantha phoned the commissioner's office, but the commissioner wasn't there. In desperation, she called Donnie Ackers of Hemophilia Federation of America (HFA), who coached her on using the right words: "Call the commissioner's office right away and get a manager. Don't lose this!" With Donnie's urging, Samantha called back: "I got ahold of Brenda*…she was literally a godsend. She said this was not acceptable. She took over our case. If not for her, we would still be fighting this. She said what they are doing is feeding you half-truth and lies." Brenda asked for only one lawyer from Assurance to be her contact, and spent hours on the phone with the boys' hematologist.

By now it was August 2016, and Samantha and her boys had gone a month and a half with limited factor. "Brenda emailed me, and said she was going to force Assurance to send the prescribed amount of factor. She challenged them on the external review board's finding. She called their bluff: Assurance wrote a letter saying it was not their fault; it was the external review board's fault. Assurance had the audacity to claim the review board put their own numbers in the

review, which coincidently matched the lower preauthorization!"

To keep medical records up to date, Samantha recalls, "We eventually had a new pharmacokinetic [PK] test on all three boys, a 7-hour drive for us to our hematologist. At that time, my youngest had a knee bleed, and had to endure having all these labs drawn. I'll never forget the drive to the hematologist; they all hurt so much that they slept a long way to overcome the pain."

The stress took its toll. Samantha says, "It was a tough time for all five of us. On top of this, we are working full-time jobs; our oldest had to quit his summer job for a while. We were so busy making phone calls, taking notes, caring for bleeds, and worrying about the long-term joint damage on the boys that we never stopped to breathe until Christmas!"

The state insurance commissioner's office made Assurance conduct a third review, with the hematologist's prescription this time. In her incredible efforts to prepare, Brenda compiled records from birth to present for each child, including all scientific evidence (lab results, PK testing), a list of 20 items that an external review board needed, the PI, the original script, the changed script, and documented bleeds.

"This third external review came not only in our favor, but even *more* in our favor," says Samantha. "The board said these boys are not getting enough factor, and they changed the dosage to every 48 hours! And Assurance can never,

ever change this, because it's from the insurance commissioner's office and follows the procedures outlined in the Americans with Disabilities Act.

Samantha never dreamed that a manufacturer's PI would be used as a tool in an insurance company's attempt to lower costs by lowering dosage. When an insurance company comes between a hematologist's prescription and a patient's need, this is playing with life and death. Samantha's story shows the value of documenting everything—every call, every email, every letter. She was smart to work with her healthcare team, and to never quit. When all seemed lost, Samantha called HFA, and from there, the commissioner's office…one more time. And that was the straw that broke the back of Assurance's ludicrous claim.

Assurance hid behind the PI to start the battle. But the hemophilia community takes any challenge to children's health head-on. To win.

Transitions *from page 3*

claims—such as those from a recently hired employee with hemophilia—may trigger a steep rise in an employer's insurance cost, forcing the company to seek out a new policy for the following year. This is especially true in smaller businesses, with fewer employees to spread the cost, so be prepared for changing insurance plans at smaller employers.

At a larger employer, the situation may be better, especially if that employer is fully insured. That's why many people with bleeding disorders work for large institutions like school systems and the government. But if the employer is self-insured (self-funded), that company faces significant financial risk if its employees' claims rapidly jump. In a self-funded plan, the company provides the health insurance and pays all the claims and associated bills. This is done to save money by not paying the administrative fees of an insurer. So when you join that employer's workforce, the company will be paying your healthcare bills. Although protections are in place, and employers can't discriminate against you due to illness or preexisting condition, the reality is that your employer will be paying your $30,000 salary *and also* paying your $270,000 factor costs.[8] Does this affect hiring? Legally, it shouldn't. But realistically, a company is driven by earnings, and the unspoken question will loom: Are you worth $300,000 or more for this job? Because that's what the company will be paying.

Marketplace/Affordable Care Act policies are certainly an option, especially if your employer offers reimbursement for ACA Marketplace plans, and these may be worth considering if you want to keep your condition—and the cost of your disorder—confidential.

Besides your living expenses and health insurance concerns, you'll need to consider your physical limitations and the physical stress some jobs inflict. Jobs that require you to be always on your feet (most retail and restaurant positions) or jobs that require daily physical exertion (warehouse stocking, construction) increase your risk of joint bleeds. With the success of modern prophylaxis and the healthy joints it provides, these jobs may be manageable in the short run, but aren't practical options for long-term careers. The damage you do to your body in physically demanding jobs is irreversible. Plan for a healthy future, and look for jobs that don't punish your body.

Are you considering transitioning into hemophilia industry positions, like specialty pharmacies and pharmaceutical companies? Caution: Many of our community members now work as representatives for these companies, which desire our expertise to promote their services and products. They offer lucrative positions. And the increased competition in factor products over the past five years has led to increased sales roles for people like you. But the industry is changing, and job availability may change too. For example, the insurance industry's efforts to control the cost of hemophilia through specialty tier programs and mandated pharmacy contracts will change the employment opportunities in the hemophilia specialty market, because of lower reimbursement rates. Also, the promise of subcutaneous products and gene therapies (which may be one-time treatments) are unlikely to need the "high-touch" care currently required by factor products, so hemophilia care positions may be cut. And the pharmaceutical industry may reevaluate its sales needs to fit market needs. Weigh other career options, and plan an exit strategy if you're considering an industry position.

This advice isn't meant to be discouraging, but to help prepare you for the working world. Being naïve about the actual cost of living sets you up for disappointment and undue financial stress. Talk to community members at your local hemophilia meetings. Meet with your hemophilia treatment center (HTC) social worker. Be smart, and get your career plan together!

Shelby Smoak is a writer and musician. His book *Bleeder: A Memoir* (Michigan State University Press, 2013) received praise from *Minneapolis Star Tribune*, *Library Journal*, and *Glamour*, and has won several awards, including "Best of the Best" by the American Library Association. Shelby has been playing and touring since the 1990s and has released several albums. Recently, he formed the indie-rock band Bleeder, releasing a debut album in June 2017. Shelby holds a PhD in literature and an MA in English, and received a Pen/American grant for writers living with HIV. He lives, writes, and plays music in the Shenandoah mountains of Virginia.

8. "Miracle of Hemophilia Drugs Comes at a Steep Price," National Public Radio, Mar. 5, 2018, npr.org.

people with hemophilia the ability to get an infusion and have their hemophilia cured without using factor (although factor may still be needed for acute bleeds). Payers currently have no idea how they're going to pay for gene therapy. No one knows how much gene therapy will cost, but it likely will be very expensive.

Since gene therapy is so new, and the cost so high, payers are determining what information they will need to create coverage guidelines. How long will gene therapy last? What is the target level of factor expression? How much will it cost? Will factor still need to be infused? Will payers have to pay the entire cost all at once? Where will the therapy be infused? Will the HTC be coordinating the infusion? What will be the role of the specialty pharmacy? Will the cost be based on the positive outcomes of the patient? What if it doesn't work?

Payers are trying to figure out ways to cover and pay for gene therapy. These can include consumer loans to pay large upfront prices, third-party financial institution financing, government financing, or manufacturer-managed financing.

Payers will rely heavily on centers of excellence, such as HTCs, and the expertise of clinical specialists to determine a patient's need for a new therapy. Since gene therapy will be considered "cutting edge," it will be up to clinicians to work with both the patient and the payer in prescribing this therapy. Payers will expect clinicians to guide them on selecting the proper patients to be prescribed gene therapy. Clinicians will need to be very specific about who should receive a treatment and which specialist should administer it.

The bleeding disorders' advocacy and consumer communities will need to work with consumers, caregivers, clinicians, and payers to educate them on the benefits of these new "frontier" therapies. Affordable patient cost-sharing policies will also need to be devised and advocated for. Since gene therapy will likely entail just one infusion at a very high cost, it may be difficult, if not impossible, for those with hemophilia to pay their share up front.

Manufacturers should seek dialogue with payers and regulators as early as possible in the development of new gene therapies, and work with clinicians, patient groups, regulators, and payers to establish robust patient registries. Manufacturers should also be prepared to address high price concerns; many payers will want manufacturers to develop specific finance mechanisms.

Hemophilia treatment is rapidly changing, and it will be up to clinicians, HTCs, consumers, manufacturers, and advocacy organizations to work together to ensure that these lifesaving therapies are available at a cost that will allow access for all.

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INSIDE:
The Road to New Hemophilia Therapies

September 2018

PEN's Insurance Pulse
Inspiring Advocacy

Sponsor a Child!

You can improve the life of a child with a bleeding disorder.

Our sponsorship program provides direct assistance to children in developing countries, who suffer the double burden of a bleeding disorder and poverty.

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or 978-352-7652

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$420 per year
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