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Parent Empowerment Newsletter

The Coming Storm

How Insurers are Changing the Hemophilia World as We Know It

by Laureen A. Kelley and Michael Russo

Drastic cost cutting by those who pay for our factor could cause some surprising changes in your choice of factor brand, and in the way you receive and use it. In some states, health insurance policy changes dictating provider and brand are already being rolled out, and this is only the beginning. In the November issue of *PEN*, our feature "Hemophilia, Incorporated" described the US hemophilia business model and the way factor reaches you, the consumer. This model is changing now, as payers assume greater control. A storm is brewing in the hemophilia community.

What's behind this cost cutting, and why is hemophilia suddenly a target? Who should decide which factor brand you or your child receives and which company provides it? Who should determine the treatment regimen? Be prepared for a new era in hemophilia. Our community will need to either accept some unwanted sacrifices—or advocate to protect access to the highest standard of care for our children.

Parents in the hemophilia community are "experts" when it comes to treating their children. But when it comes to understanding hemophilia as a business, parents are less confident. Only a few parents understand the complex US health insurance system–the system that pays for the factor we use. Most of us remain in the dark year after year, unaware of policy changes and benefits, and how these can affect our children's treatment.

The time has come to wake up. Insurance payers will no longer accept the way hemophilia is managed in the US. Until now, the hemophilia business model has preserved almost unlimited freedom to purchase the factor we want from the provider we choose. But the costs associated with running this model are not sustainable, and the insurance companies can and will make deep cuts. These cuts may result in an industry shakeout. Products may disappear. Homecare companies may fade away. Your HTC may downsize, and more powerful companies new to the hemophilia community could dominate our care.

The hemophilia community feels threatened. We have long viewed choice of product and provider as our sacred rights. If we want to preserve access, we can no longer ignore what's happening now in the US healthcare system.

"People should know that choice is changing. And with change comes a greater risk to access to care," warns Kim Bernstein, director of A.C.C.E.S.S. Ann Rogers, executive

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inside PEN

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welcome


Sherrell Portrait Design

Our feature "Hemophilia, Incorporated" (*PEN*, Nov. 2004) was received with great interest by many in the hemophilia community. Indeed, we received requests for our highest reprint in *PEN*'s 15-year history: more than 20,000 copies of the November issue have been distributed so far. We expect the same level of requests for the current issue, second in our series.

Why the high demand for information on the structure of the hemophilia business? The topics we covered in our first feature struck a deep nerve in the community. The hemophilia world as we know it–which allows choice of product and provider, provides innovative products, and returns profits to the community–could be fading. This isn't hype; it's our potential fate unless as a community, we first identify our rights concerning choice of product and provider, then preserve them.

PEN is the first national hemophilia publication to alert families to the changing hemophilia business, and to detail the risks. But the idea wasn't ours. I remained as blissfully ignorant as most of our readers until I had a conversation two years ago with Dave Maderios, a man with hemophilia, HIV and hepatitis C. Dave told me that as a national publisher, mother and advocate, it was my duty to inform families about the coming storm in hemophilia care. Families, Dave explained, were too trusting, vulnerable and unaware. He predicted that choice would soon evaporate. And reduced choice would mean fewer innovative products, decreased funding for the community, and a reversal of the progress we have made over the past ten years. Was Dave an alarmist? I thought so at first. Ironically, at that time my husband and I struggled with our insurers over both choice and cost concerning our son's dwindling lifetime cap. In 1990, I wrote *Raising a Child With Hemophilia* to prevent others from experiencing the pain we experienced. Now, I want to prepare parents and patients for coming reimbursement changes.

Everything in the current issue of *PEN* is a result of what Dave brought to my attention. He had an infectious passion about making this community strong and vocal. His message was clear: *Know your insurance policy. Don't let anyone take advantage of you. Read. Join together to define and defend your rights. Speak out to identify your needs. Take action.* I promised Dave I would write this article, and here it is, almost a year to the day after Dave passed away on February 29, 2004. On that day our community lost another visionary, leader and friend. Dave left behind his wife Kim, who strives to carry out his dream, and their six-year-old son Jason, who also has hemophilia. I dedicate this issue to Dave.

PARENT EMPOWERMENT NEWSLETTER FEBRUARY 2005

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letters

I would like to praise you for the work you are doing to help children with hemophilia in third world countries. Please accept these donated supplies in memory of my sons Michael and Christopher Rusin.

DIANA RUSIN
New Jersey

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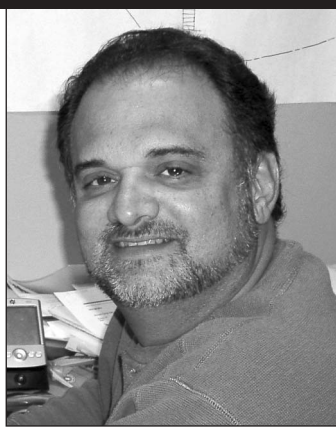
by Michael Rosenthal



got choice?

The hemophilia community has suffered great losses. Our suffering has steeled us to instinctively fight any potential loss that comes our way. The community is now being challenged by the insurance industry, and state and federal payers, who seek to cut costs of factor products. One proposed way to save money is by using a single provider of factor for a state or a group of patients. To many of us, the single provider model feels like another loss: we're losing choice of factor provider. In reality, however, we've never had real choice of factor provider–only the appearance of choice. An illusion. Let me tell you why.

Currently, most state governments contract with multiple health plans to provide service. These plans contract with a single factor provider, usually a home healthcare company. They don't give patients a choice of factor provider; patients must use the factor provider their plans dictate. The only way that a patient can "choose" a factor provider is to switch health plans at the end of the year. Ironically, a patient could switch plans, hoping to switch factor providers, yet find himself with the same provider! This could happen because insurance contracts with providers change annually, too. This is true with private and public insurance. All we've ever had is the *illusion* of choice achieved by switching health plans. And even that is not easy to do.

Think about it. Our current system of multiple providers has actually been the source of multiple problems. We've seen low bidders, with absolutely no experience in hemophilia or factor sales, win factor distribution contracts. Some of these "winners" have no local source of supply, no local employees, and no Spanish-speaking ability. For Arizona, this has been a nightmare. In addition, we've seen unethical behavior by provider representatives who are pressured to recruit patients in this highly competitive environment. This happens at camp, at consumer meetings, in support groups, and even among friends. Our system of multiple providers also has allowed price gouging, with total disregard for a patient's lifetime cap.

Single source provider contracts preserve the choice that matters most.

So what are the benefits of multiple providers? Everyone says "choice." If you don't like your homecare service, you can quit and go to some other provider. Well, just try quitting! See how responsive your health plan is to creating a new contract, just for you, with a home healthcare company that it doesn't do business with. Got choice?

Single source provider contracts, if properly constructed, preserve the choice that matters most–factor brand choice. These contracts should contain a clause that guarantees product choice. Here is the wording from the Arizona single source proposal:

"The Contractor [provider] shall not make any product substitutions without the prior approval of the authorized prescriber in accordance with accepted treatment protocols and medical necessity." This means that manufacturers must compete on the basis of product safety, efficacy and supply, and not on the basis of sweetheart financial deals made with factor distributors to carry their product over their competitors'. This spreads the business among competing manufacturers because profit margin is no longer a consideration in distributing factor.

The greatest benefit of a single provider contract occurs when a hemophilia treatment center with a proper 340B program becomes the provider. In the 340B program, the federal government gave our community a gift–a method to fund ourselves. With spiraling healthcare costs, how much longer before HTC funding is reduced or eliminated? Single source provider contracts, won by 340B centers, would be a gigantic step forward not to be feared, but embraced. Business as usual is not the answer for us.

Michael Rosenthal is executive director of the Hemophilia Association, Arizona. He has worked with the bleeding disorders community for more than 13 years, and every year hosts the popular NACCHO camp training conferences.

director of the Delaware Valley Chapter of the NHF, is already battling restrictive policies in Pennsylvania. She echoes Bernstein: "Prophylaxis is at risk, your choice of an HTC or doctor is at risk, and choice of product is at risk." At the heart of this storm is the sudden outcry from payers about the price of factor. What has caused payers to begin restricting choice?

It's All About the Money

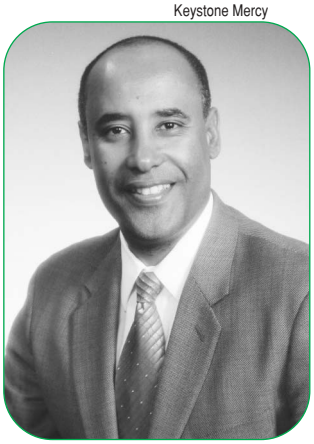
Look at the big picture first: you (or your child) are one of approximately 17,000 Americans with hemophilia. You are a key consumer in a $2 billion market involving the sale of blood clotting factor. The market is also composed of other players: six factor manufacturers; dozens of homecare companies and distributors delivering factor to patients; 145 HTCs providing medical care (half also sell factor); and insurance payers reimbursing the distributors and homecare companies for the cost of factor. The market is driven by a simple strategy: persuading customers–like you–to purchase a particular brand of factor from a particular source.

While the strategy is simple, the market is very complex. Each player that sells factor has a vested interest in promoting a selling price that earns profits. As the player reaps profits, the patient enjoys many benefits, such as high quality and innovative products, product choice, home nursing, scholarships, funding for community events, chapter support, educational materials, camps, and well-staffed HTCs.

The price of factor pays for many of these benefits. And the payers–private insurance companies like Aetna and Blue Cross/ Blue Shield, or federal- and state-funded healthcare assistance programs like Medicare and Medicaid–pay for the factor, either through the employers who fund them, or through taxes. But with rising healthcare costs creating a burden on employers and taxpayers to pay more, insurance payers are asking, *Must we continue paying these prices? At what point does profit become excessive? Are all these benefits needed? Has the hemophilia community become too entitled?*

"Hemophilia is one of, if not the most expensive chronic condition currently being treated by any healthcare system," explains Mesfin Tegenu, vice president of Keystone Mercy, an insurer. "Hemophilia has a small population, with a high cost of drugs and huge profit margins. A select group of companies are reaping the benefits, and this has been under the radar screen of payers for years. Now we have to put some management into this system, and it is stirring up all kinds of responses."

Keystone Mercy



Mesfin Tegenu of Keystone Mercy

The hemophilia community did respond–from manufacturer to distributor to HTC to patient–by going on alert. No one has ever challenged the existing business model. "Why is it that hemophilia is the only disease state with all these companies that exist only to serve and support its patients?" asks Greg Hamilton, vice president of Bleeding Disorders Programs, CuraScript Pharmacy (a division of Express Scripts Inc.). "The answer is exorbitant profit margins. Many of the players are making excessive money off the system. Cost cutting is really just realigning the market to more realistic profit margins. Insurance companies have been paying too much."

But cost cutting is a pendulum that can swing too far. "More and more, payers are gaining control over what they will pay for factor, and what their choices are," says Ken Trader, vice president of Hemophilia Health Services (HHS).[1] "Patient choice of product and provider is in some cases disappearing. Even choice of treatment regimen."

Could proposed cuts in the price that insurers are willing to pay for factor affect your or your child's hemophilia treatment

What are Medicaid and Medicare?

Medicaid is a program that helps needy and low-income people pay for necessary medical services. It uses **state** and **federal** government money.

Medicare is a **federal** insurance program for people age 65 and older and certain disabled people. The Centers for Medicare and Medicaid Services (CMS) operates Medicare. It also oversees the Medicaid program. The Medicare program consists of two parts: Medicare Part A (hospital insurance) and Medicare Part B (supplemental medical insurance).

Source: www.legal-definitions.com/health/medicare.htm

[1] A division of Accredo Health, Inc.

and lifestyle? You bet. Isn't cost cutting good? That depends. When cost cutting threatens choice of product or provider, new product development or a recommended treatment regimen, then we must ask *Who is making decisions about cutting costs, and with what authority and experience?*

The danger to our community is that cutting the cost of hemophilia treatment–with insufficient knowledge of the disorder–could result in a loss of long-term comprehensive care, and reduced quality of life standards like home infusion therapy and immediate access to plentiful factor. With no intervention from the community, cuts may produce short-term savings gains at the expense of the long-term treatment gains that the hemophilia community has achieved.

The Need to Control Spiraling Health Costs

There's no question that US healthcare costs have spiraled out of control. In 2003, our nation spent about $1.65 trillion on healthcare, the total output of goods and services. Of this, pharmaceutical costs were 11%, or almost $185 billion.[2] Healthcare costs consume one-fourth of the federal budget–even more than defense costs. And in the public sector, federal Medicaid costs increased by $30 billion between 2000 and 2002. Healthcare expenditures now exceed insurance revenues from premiums and taxes. To pay for care, either employers and taxpayers must supply more money, or cuts must be made. Since no one wants to pay more money into an already expensive healthcare system, payers have turned to slashing costs.

Hemophilia treatment costs, so long unquestioned, are now under a microscope. It isn't that the actual prices per unit of factor suddenly skyrocketed; factor has always been expensive. Factor concentrate was, after all, a revolutionary product. It was one of the first biologic products on the market in the early 1980s, in a field of only about ten other biologic products helping other chronic disorders. Initially, the high price of factor didn't raise red flags. The market expected breakthrough products to be expensive. Factor's price was outweighed by its value to patients' health and quality of life. Factor clotted blood quickly, preventing costly hospital stays, transfusions and rehabilitation.

Over time, the biotech industry grew. More products were approved to treat all sorts of chronic disorders, including rheumatoid arthritis, multiple sclerosis and psoriasis. These new products reached millions of Americans–with astronomical costs. Total healthcare costs for specialty biological products have skyrocketed. The number of biological products has increased from ten in the 1980s to 120 in 2004, with 90 more now on the horizon. In 20 years, biologics have

[2]Centers for Medicare and Medicaid Services.

Resources for Hemophilia Insurance *Information*

A.C.C.E.S.S. Program
▢ www.hemophiliahealth.com
☎ **(888) 700-7010**

Patient Services Incorporated
▢ www.uneedpsi.org
☎ **(800) 366-7741**

National Hemophilia Foundation
▢ www.hemophilia.org
☎ **(800) 42-HANDI** (Information Service)

Hemophilia Federation of America
▢ www.hemophiliafed.org
☎ **(800) 230-9797**

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▢ www.kelleycom.com
☎ **(800) 249-7977**

Find Your Local Hemophilia Treatment Center
▢ www.cdc.gov/ncbddd/hbd/htc_list.htm

GENERAL INSURANCE INFORMATION

Centers for Medicare and Medicaid Services
▢ www.cms.hhs.gov
☎ (877) 267-2323

Insure Kids Now!
▢ www.insurekidsnow.gov

National Association of State Comprehensive Health Insurance Plans (CHIPs)
▢ www.naschip.org

United States Social Security Administration
▢ www.ssa.gov
☎ (800) 772-1213

State Title V Children With Special Health Care Needs Programs
▢ http://cshcnleaders.ichp.edu/TitleVDirectory/default.htm

United States Department of Health and Human Services
▢ www.hhs.gov
☎ (877) 696-6775

Adapted from Holding on to Health Insurance, *American Red Cross*

evolved into a $22 billion per year industry, with an annual growth rate of 20%–40%, representing 8% of all medical costs. By anyone's standards, this escalation is alarming and shows no sign of slowing down. To the insurance industry, factor is a part of this growing monster.

Cost-Cutting Strategies

Cutting healthcare costs, including the reimbursement rate of factor, has become imperative across the board. Payers are beginning to employ certain strategies, in different ways, in various states.

Public insurers rely on taxes to pay healthcare costs. With tight state and federal budgets, Medicare set a new limit on what it will pay, or *reimburse*, homecare companies and distributors for factor products.[3] The new reimbursement policy in the Medicare Modernization Act[4] became effective in January 2005. Its proposed reimbursement limit for factor is drastically lower than homecare companies and distributors previously received. This means that profits are instantly reduced on all Medicaid and Medicare patients, who make up about 33% of all hemophilia patients. This revenue reduction is a direct and hard hit to hemophilia homecare companies (see "Canaries," top of page 7).

Private insurers pay for factor through premiums charged to the companies whose employees are insured. Under pressure to pay the escalating costs of medical care and drugs, insurance companies ratcheted up their premiums by 50% between 1998 and 2003.[5] The continuing rise in premiums is outpacing what employers are willing to pay. Employers are now firmly saying *No* to further rises. Insurance companies must find ways to reduce costs, including the high costs of biological products like factor.

Where will all the cuts originate? Here are three likely targets of cost cutting:

1. **Price per unit**: lowering price by negotiating large "brand" deals with manufacturers or by limiting reimbursement to third parties.
2. **Single provider**: allowing only one homecare company or entity to serve the insurer's entire patient base.
3. **Treatment**: limiting choice to a single product, reducing prophylaxis treatment, or switching patients to plasma-derived products.

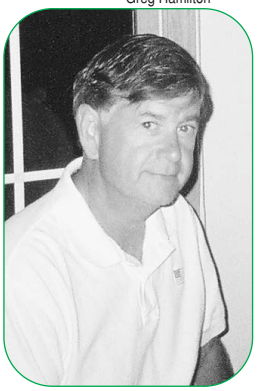
These strategies have angered players in the hemophilia community, including the manufacturers, homecare companies, HTCs and nonprofit hemophilia organizations. Homecare companies and manufacturers are railing against cutting the price per unit of factor, while homecare and HTCs criticize using only one provider. HTCs condemn changes in treatment regimens that affect the care options they offer patients. Overall, the community is urging payers to look beyond their profit margins and examine the negative impact that proposed cuts could have on access to hemophilia comprehensive care throughout the US.

Battle of the Bottle: The Rise of PBMs

With the cost of factor so intensely scrutinized, we must take a step back and ask, *Are factor prices too high?*

"Which price?" asks Greg Hamilton of CuraScript Pharmacy. "There are many prices: the 340B[6] price, the average wholesale price (AWP), the homecare price… Or is it price the distributor charges to the hospital, or the homecare charges to the patient? All these prices affect the consumer."

Greg Hamilton



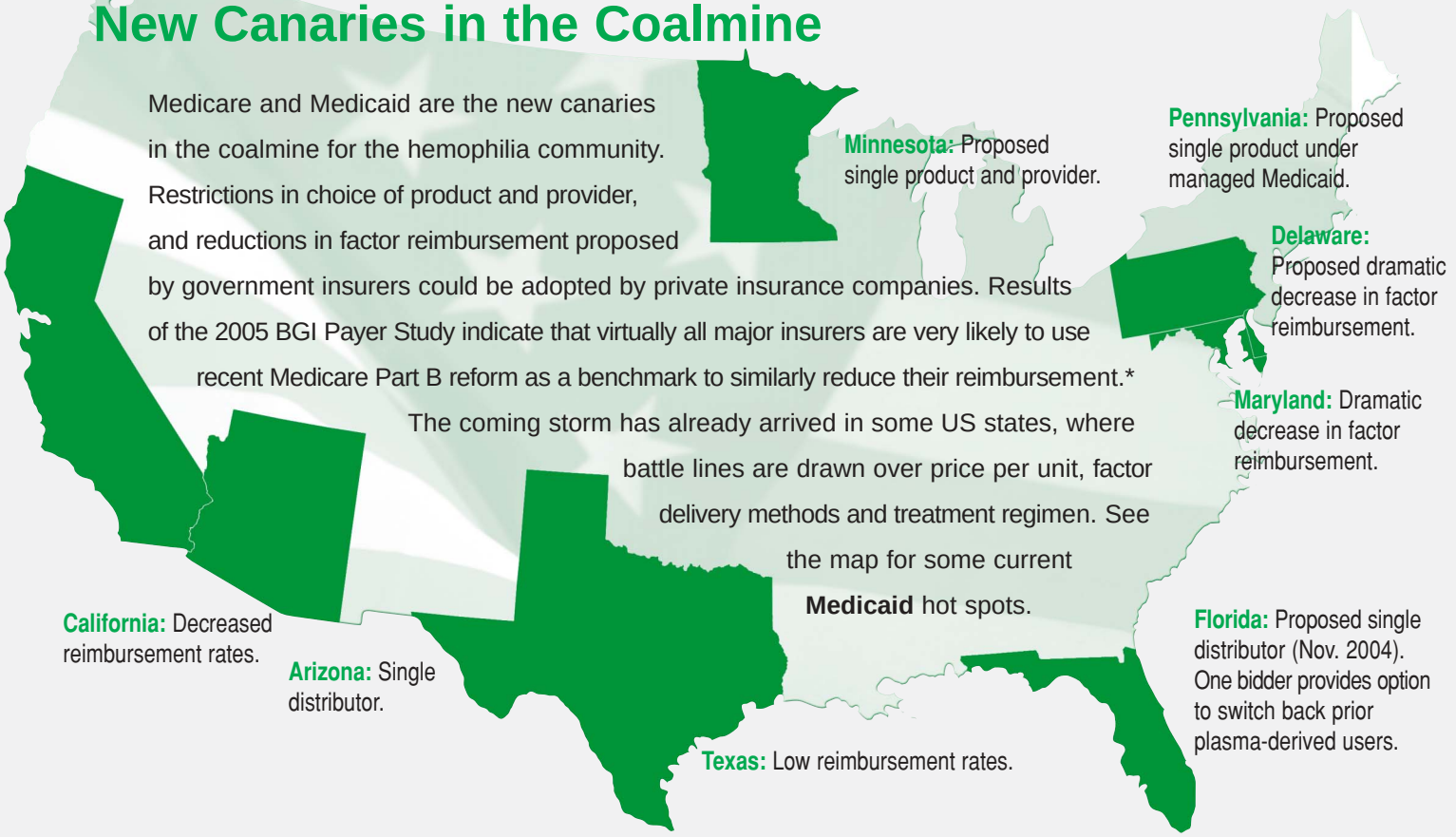
Greg Hamilton of CuraScript Pharmacy

Regardless of any one player's particular price, everyone is concerned about profit margins. Manufacturers want to preserve their factor price to pay for manufacturing plants, research and development of new factor products, quality control, varied assay sizes and liabilities in the event of a product failure. Homecare companies want to preserve their margin to cover purchasing, shipping, extensive home health services and disease management. HTCs use revenues to fund nursing salaries and HTC programs. But for payers, the reimbursement rates–the price per unit they pay to HTCs or homecare companies–is simply too high to maintain.

"The costs of nursing services, home services, ancillaries and management have typically been hidden in, or added to, the per unit reimbursement price for factor," says Mesfin Tegenu of Keystone Mercy. "As health plans and government entities attempt to negotiate more reasonable reimbursement

[3]Some state Medicaid agencies, such as MediCal, are also incorporating similar limits.
[4]The bill's full name is Medicare Prescription Drug, Improvement and Modernization Act of 2003. For more information, see www.cms.hhs.gov/medicarereform.
[5]Employment Policy Foundation.
[6]In 1992, Congress passed the Veterans Health Care Act (VHCA), which established section 340B of the Public Health Service Act. The PHS Act allows the lowest manufacturer purchase prices on prescription outpatient drugs for certain federally-funded entities and public hospitals that treat a disproportionate share of Medicaid and Medicare patients. The intent of Congress was to "... enable these entities to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services." (H.R. Rpt.102-384, 102nd Cong., 2d Sess., pt. 2, at 12 [1992]). These covered entities serve special groups of patients, typically the uninsured or low income, those facing catastrophic medical costs, or those who are underserved in the healthcare of certain diseases, such as AIDS. Federally-funded covered entities named in the VHCA include community health centers, black lung clinics, family planning centers, Native Hawaiian Health Centers, and HTCs that receive grants from the Maternal and Child Health Bureau (MCHB).

Medicare and Medicaid Reform:
New Canaries in the Coalmine



Dr. Ellis Neufeld, director of the Boston Hemophilia Center and associate professor of pediatrics at Harvard, warns, "The stories from Minnesota, Pennsylvania and Florida should be a wake-up call for everybody to what's happening." Mike Bradley, senior director of Healthcare Economics at Baxter BioScience, agrees. "What happens to Medicare policy is important for all of us. Every private insurer I speak to is aware of what's happening with Medicare and Medicaid. The precedent has been set to significantly alter the reimbursement of factor everywhere."

* The 2005 Bruckner Group Payer Study incorporates responses from managed care payers and PBMs representing approximately 90% of privately covered lives in the US.

rates, factor providers complain that they can't exist on these lower margins because of the associated nursing and supply costs. Our current policy is to separate charges for nursing services from the per unit cost of the product being purchased."

Kyle Callahan, HHS president and a person with hemophilia and HIV, believes that the price per unit is justified. "We went from a product that gave people HIV and hepatitis C to a biotech therapy that is safe and effective. We're paying for the cost of that progress. Is that too much?"

Enter a new player in the hemophilia market–pharmacy benefit managers (PBMs). Although new to hemophilia, PBMs have been around for decades. Some originally functioned as retail and mail order pharmacies, buying drugs from manufacturers, distributing them to patients, and receiving a reimbursement payment from insurers. Over time, PBMs began acting as broker between the insurance company and the manufacturer. Insurers hired PBMs to streamline their reimbursement process and negotiate better prices with manufacturers. Eventually, PBMs expanded their services, and now help the insurer make better decisions on purchasing drug products. This has led to the creation of sophisticated drug formularies.[7] PBMs now co-manage an insurer's entire budget for buying drugs. Their influence on drug management is tremendous.

And the trend continues. Now PBMs are seeking to manage factor purchases. The PBM style of management is perceived by many as a threat to the current hemophilia business model. Homecare

Who are the PBMs?

CVS

Medco

Caremark/ AdvancePCS

Express Scripts

[7]A formulary is a complicated system of classifying drugs that is used by insurers to define the drugs that it will make available to its customers, and under which circumstances. The system is largely organized according to the specific medicine, a particular brand or generic, that the insurer prefers the patient to use for a specific medical reason.

companies and HTCs worry that the lower price offered by PBMs may cause them to lose business. They also fear that, as patients move to a highly managed setting, PBMs will not replace the critical care services of homecare. "A PBM's job is to provide safe products at affordable prices," says Greg Hamilton. "PBMs typically negotiate great discounts and rebates. We have huge resources; our stock price is not driven by hemophilia solely, so we don't need huge profit margins off of factor." Still, the hemophilia community wonders, *Without those profits, will hemophilia services be discontinued?*

Hemophilia Health Services

Kyle Callahan of Hemophilia Health Services

Kyle Callahan of HHS offers a more balanced approach, recognizing that cutting costs cannot compromise the quality and availability of services. This view is encouraging, considering that HHS has a long-term contract with a PBM. "We are not just a distributor," says Callahan. "Our philosophy shows the difference between a full service homecare provider and a mail order or drop shipment provider." Vice President Ken Trader adds, "Our mission is to help the payer as well as the treatment center. We monitor therapy adherence to help reduce bleeds and infusions, manage assay shipments, and measure quality of life. We measure everything. We classify new patients into one of seven clinical pathways. Within each pathway are measurement points that allow us to quantify our performance for the payer and HTC."

PBMs are relatively small players in the hemophilia model, but they may grow and gain influence if they deliver on their promise of providing the lowest per unit prices. For both the medical and business hemophilia communities, the possibility of a PBM-style managed hemophilia business model is alarming. In exchange for lower prices, the community fears losing homecare services, freedom of product and provider choice, and instant access to care.

Single Provider

Another way for payers to control costs is to limit the number of factor providers, even to a single provider. This strategy helps payers negotiate better prices by guaranteeing a certain minimum number of patients, and streamlines administrative and legal fees. But the hemophilia community is accustomed to a competitive marketplace where patients can negotiate and "shop around" for the homecare services that best meet their personal needs. Homecare creates an intimate, patient-focused relationship between company and customer; good hemophilia care is taught, encouraged and monitored, and factor usage is closely supervised. So strong are the bonds between homecare representative and patient that patients often switch homecare companies when their personal representative is hired by another company.

Currently dozens of homecare companies service hemophilia patients, creating a secure network of local, highly specialized care options. Indeed, some of these homecare companies are devoted solely to serving hemophilia patients. Homecare companies compete with each other to become "in network" with a payer. An in-network physician, hospital or homecare company is one that is preferred by your insurer, who contracts lower prices with in-network providers.[8] Usually the insurance policy limits the number of in-network providers, while still offering enough choices of provider. This system preserves choice while eliminating the cost of dealing with dozens of separate companies. The hemophilia community has traditionally accepted this system.

Yet in fall 2004, several states requested proposals on pricing and services from the homecare industry in order to select a *single provider* for their public assistance patients. This latest step enraged a community accustomed to having choice.

Cutting Costs, Cutting Treatment

Modifying hemophilia treatment regimens represents a final area in which payers may seek to gain control of rising costs. Medical treatment is typically the arena of medical experts, the physicians who treat hemophilia. Payers may be overstepping their bounds when their policies begin to interfere with the clinical choices made by physicians. Payers' influence over treatment decisions can range from mild–like requesting documentation of factor usage–to extreme–like insisting on limited doses.

Many in the hemophilia community believe that cost cutting becomes unacceptable when payers institute these extreme policies:

- Requiring prior authorization for each home delivery of factor
- Attempting to limit prophylaxis
- Monitoring factor usage through frequent home inspections
- Requiring the return of empty vials
- Excluding patients from using recombinant products

[8]If a particular policy allows any out-of-network services, insurance companies will try to steer patients toward the in-network choices by charging nominal copayments for in-network and more expensive copayments for out-of-network.

This final tactic produced a major outcry in the hemophilia community. In November 2004, the state of Florida accepted proposals from several homecare companies to choose a single factor provider for all Medicaid patients. The proposals provided profiles of each company, its services, and how it planned to hold down costs. As one of four cost-cutting options, one large national homecare company suggested that adult patients currently receiving recombinant factor could be switched back to plasma-derived products. Its proposal accurately cited that no plasma-derived product has been proved to cause viral transmission in the US since 1987, while admitting that a switch was not an ideal solution. Yet when this proposal became public, it was considered an outrageous step backward by the hemophilia community.

These tactics are not limited to Medicaid. Private payers are also rolling out cost-cutting strategies that may infringe on the quality of hemophilia care. One example is "step therapy." Step therapy allows the payer and its contracted PBM to approve and reimburse one preferred brand of factor; if a patient does not react well to the selected brand, he can proceed to the next approved brand. Step therapy may come as a shock to a community accustomed to complete factor brand choice based on individual patient preference and need. Although all factor products in the US are considered safe and effective, and step products are all recombinant, there is anecdotal evidence that different products have different clinical effects in some patients.

Restricted choice may save money, but it makes some in the hemophilia community uncomfortable. "I am not in favor of step therapy," declares Ann Rogers of the Delaware Valley Chapter. "A major Medicaid HMO in Pennsylvania has proposed step therapy as a way to manage hemophilia," she explains. "Step therapy is encroaching into treatment. The payer determines what factor brand we should use, how many doses we should have, and when it is delivered. Medical treatment needs to stay with the medical team, and payers need to stay in the payer arena. Costs should be looked at, but *as a team*."

Dr. Ellis Neufeld, director of the Boston Hemophilia Center and associate professor of pediatrics at Harvard, disagrees in part, particularly about the effect of step therapy on direct clinical care. "I don't necessarily object to step therapy," he says. "In some diseases it's perfectly reasonable. A single hemophilia product is probably OK if it is an advanced generation product."

Mesfin Tegenu of Keystone Mercy agrees with Dr. Neufeld, stressing the influence that manufacturers might have on creating demand for a product. "We recommend preferred brands, but they are all recombinants, and all factor products are on our formulary." Tegenu urges patients to think carefully about who is recommending which products, and why. "While factor providers, patients and advocacy groups feel that the payer should not be involved in deciding factor brands, drug companies are allowed to market to patients and providers, and provide financial support to hemophilia advocacy groups. The decision on which product is 'most appropriate' can be greatly influenced by goals other than what the patient truly needs."

Derek Robertson

Derek Robertson of the Hemophilia Alliance

"The hemophilia community needs to decide what they will and won't compromise on."

Attorney Derek Robertson, consultant to the Hemophilia Alliance,[9] dislikes step therapy because it represents an artificial manipulation of a competitive market where products are *not* all equal. "In addition to a medical recommendation, payers must also look at the market when it comes to single product decisions. No one manufacturer can satisfy the market. We saw that during the Kogenate® shortage of 2002. You need *all* the manufacturers competing in a single market. These are not pills that you push out by the millions. These biological products can be differentiated."

Mike Bradley, senior director of Healthcare Economics at Baxter BioScience, agrees with Robertson. He faults payers for trying to follow a treatment formula that worked with other disorders, and cautions that hemophilia is different. "PBMs and payers are trying to mimic what they did for oral meds," says Bradley. "But there's no standard of care for any kind of hemophilia therapy. There's no path for PBMs to follow. There is a huge need to maintain standards of care if PBMs and payers are going to get into this business."

Already, in fact, payers are redefining standards of care, sometimes allowing doctors inexperienced in hemophilia to make decisions. According to Kyle Callahan, "As payers cut costs, they are making clinical guidelines around what types

continued on page 12

[9]The Hemophilia Alliance is a nonprofit consortium of 36 340B programs.

"Relentless state advocacy may be the single most important thing we can do for our community now."

Maintaining Access to Care and Reimbursement—

Our Number One Priority

by Elena Bostick

The state of the nation's healthcare and reimbursement system has reached a dangerous point, with no solution in sight. Since Medicaid expenditures are among their largest single budget items, states are struggling to bring Medicaid costs under control. In 2003, 25 states restricted eligibility, 18 reduced benefits, and 17 increased copayments. For hemophilia, this translates into a number of threats:

- Single source providers/single products
- Prior authorization to order factor
- Home audits of factor inventory
- Treatment decisions by unqualified people

The Hemophilia Association of New Jersey (HANJ) believes that hemophilia healthcare is an issue too vital to be left to bureaucrats or administrators. Consumers are entitled to a place at the table when decisions are made about standards of medical care that affect their lives and survival. Before going to that table, consumers should understand the following facts:

1. Current and future healthcare changes *will* impact hemophilia.
2. Most policymakers do not understand hemophilia.
3. Decision-makers listen to people who speak up.

You and your local hemophilia organization have the capability to plan a strategy, approach your legislators, and defend and secure the needs of you and your people with hemophilia.

HANJ has waged many successful legislative campaigns, most recently "Standards of Care in Hemophilia Homecare." This was a bill directed at insurance companies and HMOs that stated, "If you contract with homecare providers to deliver services to people with hemophilia, *the homecare provider must first meet the following standards*," which include:

- Access to all products
- Availability of nursing services
- Emergency home treatment
- Experience with hemophilia and other bleeding disorders

We didn't pull these standards out of a hat. They were created by a committee that labored for almost a year to evaluate our state's healthcare system, check the statutes and develop the language for the bill. The committee consisted of consumers, physicians, nurses, homecare company representatives, two lawyers, an insurance expert and a legislative aide. Our goal was to protect patient choice and access to care. The standards bill was passed in September 2000, and has served us well—but not well enough to allow us to let down our guard.

Over the years, our opponents have been powerful and well-organized lobbyists. But we were united, single-minded and relentless. The mindset must be this: *We are right, we have scientific data to support our arguments, and we are not going away until access to care, treatment and choice is secure*. You may wonder where to deliver this message. It's important to remember that almost every activity that goes on in your state has a corresponding legislative oversight committee.

If hemophilia healthcare in your state is threatened by lack of choice of factor provider or product, you can successfully lobby and campaign to preserve choice. Below I've outlined

what we did in New Jersey. You can use the same approach, regardless of whether your goal is to help Medicaid "see the light," or to lobby in support of legislation. Be sure to work with your hemophilia chapter as a team.

Let's assume that Medicaid policy changes are threatening access to care and choice. What can you and your hemophilia community do?

1. Determine which legislative committees oversee Medicaid. There will be two sponsoring versions of each bill, one from each side of the state house—the senate and the assembly. Determine which legislative committee oversees Medicaid or will hear this bill. For example, it may be the Health Committee, Human Services Committee, or Insurance/ Banking Committee.
2. Identify the legislators who sit on these two committees, as well as the committee aides. Make friends with the aides: if they like you, believe your cause is noble, and know that you will be relentless, they may facilitate meetings and open doors for you.
3. Determine the legislative districts of the legislators on the two committees.
4. Identify your chapter/association members living in these legislative districts. Then ask all these individual members or families to call their legislators and set up personal, one-to-one meetings, preferably at the legislator's local office. When you meet with the legislator, prepare "talking points," or a package of pertinent information. Also bring general information about your chapter/association. Be prepared to offer solutions that will help your cause, and always feel free to discuss your personal situation. As a voter, your needs are important to your legislators. Explain how the bill directly affects you and your family.
5. Get to know the key decision-makers in your state: large insurers, Medicaid officials, and legislators on key committees, such as Health, Human Services, Insurance/Banking, and Appropriations.
6. Encourage your hemophilia chapter/ association to write to every legislator and to the governor. Seek an appointment with the governor.
7. Monitor changes in legislative, administrative and private policy concerning healthcare, insurance reform, insurance policy divisions and coverage options, insurance reimbursement issues, state line budget items, and projected or enacted program cuts and deficits within your state.

And most important, speak up! Let the media know what's going on by talking to members of the press and issuing press releases. Schedule radio, TV or newspaper interviews for members of your organization to tell their stories and explain the negative impact of issues that concern them: limited access to care and treatment at HTCs, inadequate insurance coverage, skyrocketing premium rates, and lack of choice of products and service providers.

Your campaign can be a success. The hemophilia community has some unique advantages:

- We are organized: we can identify the person or entity, like your hemophilia chapter, to coordinate efforts.
- We have the recommendations of MASAC's renowned scientists in hemophilia to support our arguments.
- We can demonstrate the superb benefits of adequate and appropriate treatment of hemophilia.
- We have a grim history of HIV and HVC infection in our community.
- We have the passion and strength that come from enduring tragedy.

I wish I could say that it's easy, but it's not. I wish I could say that in New Jersey we accomplished our goals in a few weeks' time, but we did not. We evaluated the nature of the threat and its impact on the hemophilia community, formed a strategy and took action. You must take the bull by the horns and stand up for your healthcare right—the right to comprehensive care from experienced hemophilia professionals, with the appropriate means to pay for that care. If your legislators aren't aware of the challenges you face, and don't understand how changes in insurance affect your access to necessary treatments, then they cannot help you. Their ignorance may hurt you when they begin cutting budgets at your expense.

To the extent that changes in Medicare and Medicaid set the standard for changes in private payer policies, relentless state advocacy may be the single most important thing we can do for our community now, and for the future. We should not allow this period of our history to be marked as the time when we accepted less because we were not numerous enough, not organized, and not strong enough to make a difference. We know that we must preserve patient choice, access to comprehensive care and adequate reimbursement—for our hemophilia community now, and for future generations.

Elena Bostick has been executive director of the Hemophilia Association of New Jersey for more than 25 years. Under her direction, HANJ has been instrumental in securing full insurance coverage for hemophilia care in New Jersey. Elena also spearheaded the drive to legislate the state's Hemophilia Homecare Standards of Care, which was accepted by the National Council of State Legislatures as a model piece of legislation. It is now being copied throughout the country.

The Coming Storm... continued from page 9

of therapies they'll pay for. A health plan may even use contracted physicians *outside* of hemophilia to determine these standards. If your child's hematologist does not follow the prescribed treatment protocol, the insurer won't pay the claims. They may recommend plasma-derived products, or limit prophylaxis. And they *do* have the authority to do this."

These are some of the strategies that payers will try to employ in order to control costs. What is at risk if payers are able to implement them? Will PBMs become major players? How would the hemophilia model change? What could the community gain?

There are four basic risks to the hemophilia community:

1. Potential conflicts of interest
2. Barriers to product innovation
3. Decreased HTC funding
4. Short-term gain over decreased long-term economic benefit

Potential Conflicts of Interest

PBMs may be playing too many roles in the hemophilia business model. For example, some PBMs not only sell factor and offer their own homecare services, but they also help insurance companies determine which homecare companies to contract to create their networks. Because the PBM reviews the competitive contracts submitted by homecares, HTCs and even other PBMs, it gains a tremendous advantage. From these contracts, a PBM can learn what its competition will offer, and at what price.

PBMs are hired both to control costs and to sell factor. As providers, it is in their best interest to sell as much factor as possible, but in an effort to control costs they must also run programs aimed at reducing factor utilization. Without a long-term vision, these are not compatible goals.

Further conflict of interest may arise when PBMs engage both in negotiating lower prices with manufacturers and in determining which products should be preferred on formulary. When lower prices mean that a particular factor brand will be a preferred formulary item over another factor brand, is the PBM allowing financial concerns to affect the clinical care choices available to patients and doctors? In this heavily managed business model, the factor your child is prescribed could be chosen solely on the basis of contract negotiations. Most of the hemophilia community maintains the conviction that brand choice is a clinical decision made between physician and patient.

Barriers to Product Innovation

Cost cutting could have far-reaching implications to innovation. In our revised hemophilia model, new factor products must clinically demonstrate a clear, measurable benefit–or they won't be covered. If payers are unlikely to cover products that do not have measurable benefits, manufacturers must reconsider the risk of spending millions to develop them. The fact that a product is innovative, groundbreaking and theoretically safer will no longer be enough to get it listed on formulary.

The US hemophilia community demanded safer products as a result of the HIV catastrophe, and continues to call for cutting-edge products in light of emerging pathogens found worldwide. But if insurers refuse to pay for this kind of innovation, the incentive to develop it will dwindle. Research and development efforts will focus instead on measurable benefits, such as increased half-life, which would extend the activity of factor in the bloodstream, meaning fewer infusions. In the terminology of payers, we are all moving toward "outcomes-based access" or OBA. In other words, higher-priced products must demonstrate measurable therapeutic value before they will be reimbursed.

The bleeding disorders community praised the development of third generation clotting products. But to payers, there is *no clinical difference* between Advate and Recombinate, Kogenate, Helixate® FS or ReFacto®; paying a higher price for a theoretically safer product makes no sense to them. If new products have no direct additional value to the payers who control product choice, then no hemophilia patient will be allowed to use third generation factor VIII products–unless they cost the same as second or first generation products.

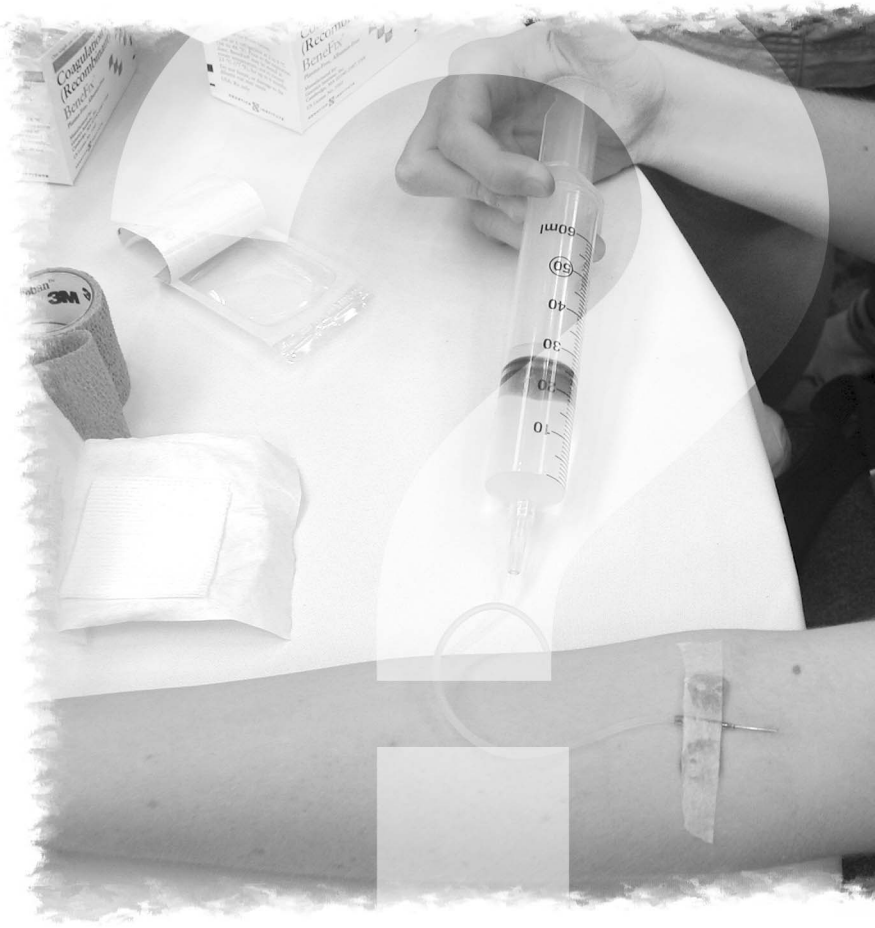
Decreased HTC Funding

As a player in the hemophilia market, the HTCs that sell factor will be hit particularly hard by reimbursement cuts and the entry of PBMs. Known as 340B programs, these HTCs are able to purchase factor at the lowest available prices and sell it through their clinics. This factor revenue provides necessary funding for nursing staff and services. In an era of national healthcare cost cutting, HTCs have seen their federal grant revenues drop. Factor sales can represent a substantial source of essential income. Without it, some HTCs are in danger of collapse.

"We rely on 340B program revenues to run our treatment programs," admits Dr. Ellis Neufeld. "The 340B program pays for our nurses. Our per unit prices are very low, because our acquisition costs are low by federal mandate. We need only a tiny profit margin to take on Medicaid and Medicare patients, so we can actually save the state a

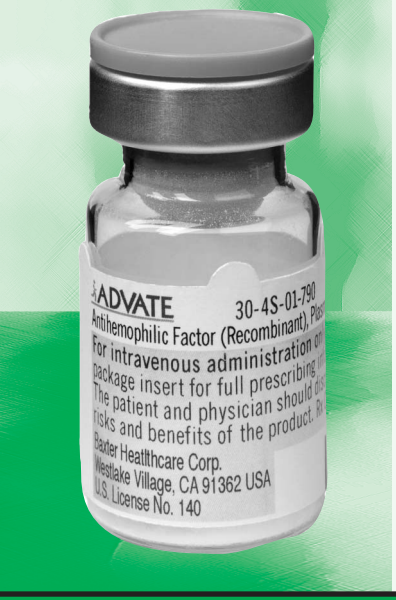
lot." Neufeld paints a bleak picture of future hemophilia treatment if payers continue unchecked: "We don't need single providers, we need *choice*. If Massachusetts were to use a single provider, it couldn't be worse for hemophilia comprehensive care. The 340B program would be threatened, and this would threaten the hemophilia center."[10]

David Linney, reimbursement specialist for the Great Lakes Hemophilia Foundation and national expert on hemophilia reimbursement, agrees. "HTCs are losing business, and will keep losing business if they do not have a contract with the payer." Linney explains, "HTCs with 340B programs only have the ability to provide service in an immediate geographic area. To a payer, that's very limited–that's a negative. An HTC can lose a lot of revenue if a major payer suddenly uses one provider, and that provider is not the HTC." But Derek Robertson of the Hemophilia Alliance tempers this point: "The lowering of reimbursement is not necessarily a bad thing; prices have been too high. Still, we're concerned about the effects of lowered reimbursement prices on treatment. When reimbursement is lowered, it has a greater impact on 340B programs. We not only offer expert hemophilia care, but also



[10]For one point of view on 340B programs, see "Got Choice?" by Michael Rosenthal, *As I See It*, page 3.

ADVATE: The Last Generation?



by Michael Russo and Laureen A. Kelley

In summer 2003 Baxter introduced Advate, its recombinant third generation antihemophilic factor. The development and marketing of Advate is an ideal illustration of the challenges facing the hemophilia community in the new, heavily managed business model. Advate represents a technological breakthrough. Its production uses no animal protein. This gives it a theoretically higher level of safety. *Theoretical*, because no one can yet "prove" Advate's safety level relative to other recombinant products. Ten years ago, consumers and physicians would have rushed to select a such product, at almost any price. And such a product would have commanded a premium. But the market is very different now.

Today, payers do not see the extra value of a theoretically safer product—if it costs more. To payers, "value" means that the higher price of a product must be justified by the product's clinically proven benefits. But to the hemophilia community, Advate's value in the marketplace is based on reducing the risk of contamination by an infectious agent through the removal of animal protein in the processing. When Advate was introduced in 2003, some payers thought that the initial price was too high relative to the actual risk of another virus or pathogen infecting the factor supply. (The price has since been lowered.) To the payers, all recombinant factor products have equal value. Their definition of value differs from that of the hemophilia community, which believes that avoiding even a slim, theoretical chance of viral transmission is worth paying higher costs.

Advate was developed in direct response to the hemophilia community's outcry for safer products. If left unchallenged, payers will limit choice—purely by looking at cost per unit in relation to a proved, comparative clinical benefit. This may kill any incentive for manufacturers to invest millions to develop advanced generation products. Advate could well become the last generation of recombinant factor products if the hemophilia community does not prove, promote and defend what it considers to be the value of advanced products.

"We don't need single providers, we need *choice*. If Massachusetts were to use a single provider, it couldn't be worse for hemophilia comprehensive care."

Dr. Ellis Neufeld

lower pricing. Lower prices are not everything; they are good because they benefit patients, but reimbursement cannot be so low that it affects access to care."

Access to proper care is one of the greatest concerns of the hemophilia community. HTCs with 340B programs fund themselves through factor sales. But they are small players with little economic clout. With increased payer cost cutting, some HTCs could close, leaving patients to backtrack 20 years to the days of emergency rooms and non-specialized hematologists. Dominated by a handful of large PBMs, a few homecare companies and some struggling HTCs, how would a new hemophilia market function? Who will fund HTCs? Will PBMs or homecare companies compensate by offering services normally offered by HTCs? No one knows.

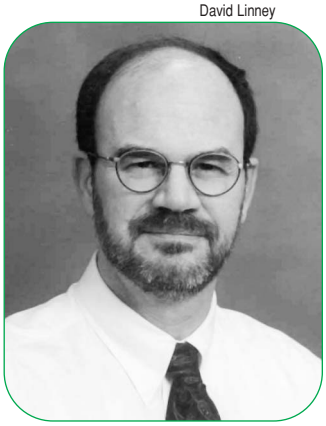
Short-Term Gain Over Decreased Long-Term Economic Benefit

The short-term goal of cutting the cost of hemophilia treatment is to minimize overall costs within a given fiscal year. For private payers, this can help ensure employer satisfaction, a steady stock price, and coverage of all policyholders. For public assistance payers, cost cutting enables coverage for all people with hemophilia under the state or federal budget. For these reasons, many payers may be eager to work with PBMs or to enlist new policies like step therapy or single providers.

Kyle Callahan warns that adopting a short-term perspective will threaten care. "Cost cutting decisions may be made by payer medical directors; at best, maybe a hematology consultant who has never seen a patient. Their standards may not be consistent with MASAC[11], and these standards differ from region to region. This jeopardizes quality of care, access to care, and cost controls. Their lack of knowledge of hemophilia as a chronic disorder can actually lead to higher costs in the long term."

David Linney adds, "We must understand the payers' perspective, too. We have the most complicated reimbursement system in world. It's not that someone is a bad guy. It's a sophisticated marketplace. Payers are just starting to tighten the screws after many years of paying high prices."

David Linney



David Linney of the Great Lakes Hemophilia Foundation

But hemophilia, like all chronic disorders, requires a long-term vision. "We must look at things other than price per unit," advises Derek Robertson. Indeed, to make better economic choices, such as working toward cost savings, a medicine's true *value* must be understood–in terms of its competitive short-term benefit and, more important, in terms of its long-term effect on patients' health and quality of life.

Hemophilia Business Model of the Future

If the challenges facing the current hemophilia business model continue unchecked, what will happen? What are the long-term consequences?

Ann Rogers warns, "If we don't watch and take action, this is what I see in eight to ten years: families with hemophilia may get a letter from their HTC saying that as a hemophilia patient, they will not be able to get their annual clinic at the HTC anymore. A different center will do the annual assessment plan, and that physician and center will be an 'approved provider,' not necessarily a physician familiar with managing hemophilia. It's already happening in other disease states. We must hold payers to the highest standards, being mindful of costs."

To the manufacturers, product innovation seems most at risk. According to Pete O'Malley, vice president and general manager of the US Hemophilia Business for Baxter, "Pharmacy models that limit choice of products and distributors will restrict patients' and physicians' ability to access appropriate therapies. This could lead to reductions in the services that HTCs are able to provide, including access to innovative therapies."

Reimbursement specialist David Linney believes that in any future model, the community must keep the payers' perspective in mind. "I hope everyone has full choice, but I don't see that happening. I see everything getting tighter as

[11]Medical and Scientific Advisory Council of the National Hemophilia Foundation.

there is greater control over costs in the marketplace. If you are given a single recombinant factor VIII product, with no choice, what's wrong with that? MASAC recommends recombinants, but does not recommend a particular brand. As a community, we'd better be prepared for this."

Mesfin Tegenu believes that the future of the hemophilia market depends on the flexibility of current players, and their willingness to accept coming changes. "Homecare companies *can* provide a valuable service," he admits. "If they are willing to reinvent themselves to serve what we hope to be the new business model for managing hemophilia, they will survive. If they hope to continue to make large profits while simply being centers of distribution, they will eventually be put out of business by the specialty distribution companies [like PBMs] that are willing to provide product only at rock bottom prices."

Mike Bradley of Baxter BioScience sees a future without the patient-centric services that homecare brings. "The hemophilia community is used to a 'high touch' service model," he explains. "The community will probably lose many of these personal touches. I assume that funds to camps and scholarships would go away, too. Homecare currently channels profits back to the community because its main customer base is the patients. That isn't the way with PBMs."

Baxter BioScience

Mike Bradley of Baxter BioScience

"Our business model is totally different," agrees Greg Hamilton of CuraScript Pharmacy. "We present a program and a price list to the payer, and we let them decide. Our main customer base is insurance companies and large employers. I foresee continued consolidation in the homecare industry. And some of the community perks will probably go away, like funding golf tournaments and scholarships."

Kyle Callahan echoes concerns about the value that homecare brings beyond price per unit. "PBMs can have a cost advantage in their per unit price. But homecare relies on the ability to provide a high level of service for members, including educational programs, community support and specialty-trained clinicians." Despite this, predicts Greg Hamilton, "I believe the PBMs will absolutely dominate the hemophilia market."

Choice: Privilege or Right?

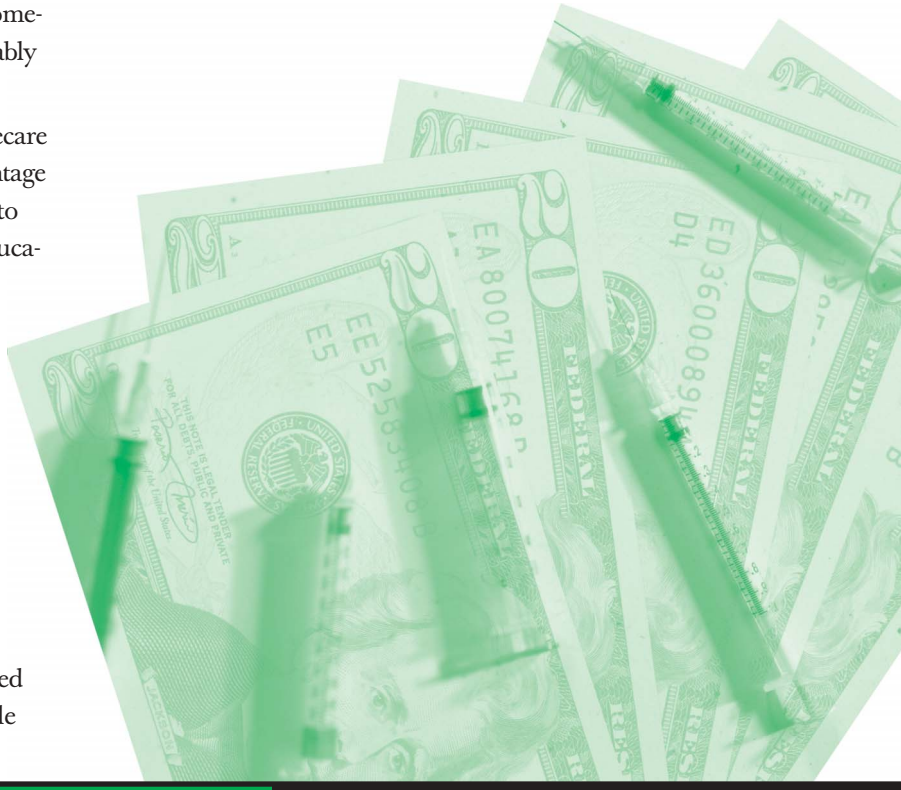
It's no secret that the hemophilia community is vocal, united and proactive–and fiercely protective of what it perceives to be its rights. Once the community takes a stand on an issue, it works relentlessly to reach goals. The hemophilia community has clout in Congress; it has suffered the loss of approximately 10,000 people due to preventable mistakes, lack of action and lack of advocacy. The US hemophilia community works tirelessly to ensure that history will never be repeated.

Critics contend that the community feels a distorted sense of entitlement–because hemophilia is somehow different, somehow more deserving than other chronic disorders. The community's demands are many and loud. Yet payers see hemophilia as just another disease state.

Kyle Callahan, a person with hemophilia, agrees that the community is a bit spoiled "because an industry has grown up around them–specialized, skilled and experienced to meet their specific needs. This is now threatened by those companies solely focused on price per unit. Service is going to companies that don't know our daily needs."

"There is an incredible push by advocacy groups to give hemophilia patients a sort of 'special status,'" observes Mesfin Tegenu. "We are constantly getting letters suggesting that the healthcare payer should have absolutely no input or voice when it comes to the provider's choice of treatments and treatment regimen."

The argument to preserve services to the community because of its past suffering–at any cost to the payer–is weak, charges Greg Hamilton. "Yes, this group has suffered more than most," he admits, "but you are not allowed to milk the insurance industry. This is an emotional argument and it isn't an effective one. It needs basis in fact."



David Linney adds, "We have a more sophisticated marketplace now, and to say we always had choice and we had a tough history–well, we may need a stronger argument to advocate for continued choice."

Choice is an integral part of the hemophilia community's commitment and perception of rights. Preserving choice is fast becoming the mantra of hemophilia. But when payers are contracting with providers for single products, is this truly a threat to choice? David Linney suggests, "We must ask the community and medical treaters: *Is* there something wrong with a single recombinant product being the only product? *Is* there a problem with delivery of homecare services through an insurance company with a specialty pharmacy? Our community needs to separate its emotion, get the facts, develop a position and *then* bring emotions into it."

Derek Robertson stresses, "We still should raise emotional issues; our community is distinguished by this. The hemophilia community has gone through issues that other disease states have not. We want to make sure we continue to have a level of choice that ensures access to care. We need to prevent viral infection and lack of access to care from happening again."

The hemophilia community continues to prove that it can stand up for its rights. Just recently in Florida, five diverse national and state hemophilia organizations united to speak out against the state's request for a single provider proposal. "The state of Florida thought this was a done deal, but it wasn't," says Kim Bernstein of A.C.C.E.S.S. "If no one had acted, a single provider contract would have been signed and we wouldn't have known what was in it. We must stay vigilant and ask questions." Florida reconsidered, and will now allow up to three providers of factor.

The Future is Now

At risk in the new era of cost cutting is your freedom to choose the product you need or want, the distributor you prefer, and perhaps even where and how you get your medical care. Although choice is at risk, the hemophilia community is equipped to battle the coming storm. But in many ways, the future is already here.

"You have to admit that this is where we're going, and decide what you are going to do about it," recommends Greg Hamilton. "As the hemophilia model is changing, ask *Where do I want to be?* You want to be talking to the PBMs. Tell them, and your employer, your concerns about product choice. If you do this now, you save yourself a world of hurt down the road."

Yet some believe that the future is still ours to create. "There's a change coming," predicts Kyle Callahan. "Our national healthcare system is responding to rising costs by restricting choices that have become dear to us. We need to send a message that that's not the way to manage hemophilia. If we are passive and patient, insurance companies will dictate our future. We were victims in the past and we changed things. It's happening again–not from HIV or hepatitis–but it still threatens our ability to make the best choices for ourselves. If we don't take a stand now, we will lose our ability to choose."

Laureen A. Kelley is the mother of a child with hemophilia, and president of LA Kelley Communications, a worldwide provider of groundbreaking books, newsletters and market research about hemophilia. To comment on this article, contact her at laurie@kelleycom.com.

Michael Russo is a partner at The Bruckner Group (BGI). |Michael is a recognized expert in helping stakeholders–manufacturers, payers and patients–preserve and defend their interests in the emerging value- and outcomes-based healthcare system. To comment on this article, contact him at mrusso@brucknergroup.com.

New Website for Moms

ZLB Behring's new website *A Mom's Story* chronicles the everyday events and challenges faced by three mothers raising children with hemophilia A. The site offers a subscription to *The MOM's Factor*, a monthly electronic newsletter featuring stories from three mothers about a variety of topics: helping children tackle school, sports and hobbies; meeting everyday challenges; encouraging and supporting children; securing outside assistance; relating to friends and other family members.

For more information:
www.hemophiliamoms.com

Bayer Sells Plasma Division

In an agreement with Cerberus Capital Management, L.P. and Ampersand Ventures, Bayer AG has sold its plasma business, including production of its plasma-derived factor VIII product Koate®-DVI. Bayer BP's blood coagulation business, which includes the recombinant factor VIII brand Kogenate® FS, is not included in the deal and will become a separate division of Bayer HealthCare. The US sales and marketing operations for Kogenate FS will be based in West Haven, Connecticut.

Source:
www.bayerbiologicals.com/News_Center/Press/2004/20041213.asp

For more information: Terry Tenbrunsel, vice president, sales and marketing, Bayer Biological Products (203) 812-6392

NHF Clinical Fellowship Program Awards from Baxter

In November 2004, Baxter Healthcare Corporation and the NHF announced the fellowships awarded through the NHF Clinical Fellowship Program. Funded by a $2.125 million educational grant from Baxter over five years, the program is designed to train clinicians in providing comprehensive care for the treatment of bleeding and clotting disorders. Since the program's inception in 2003, nine physicians have been selected as fellows by an independent panel. Funding is awarded to the HTCs where the candidates will be trained. Baxter's sponsorship of the program will support fellows through 2007.

Source: Baxter press release

For more information: Cindy Resman,
Baxter Healthcare Corporation (847) 948-2815

EXTENDED-DATE Stimate® Now Available

According to ZLB Behring, the recent supply and extended dating issues concerning Stimate have been resolved, and an adequate supply with extended dating (expiration date September 30, 2006) is now available. As the sole US distributor of Stimate, ZLB Behring is working closely with the drug's manufacturer, Ferring AB in Sweden, to maintain adequate supplies and avoid inventories of short-dated product.

Source: ZLB e-NEWS Update

"Beige Substance" Formation on Butterfly Needles

The National Hemophilia Foundation has been contacted by several hemophilia treatment centers regarding a "beige substance" on the tip of butterfly needles distributed by a former Abbott Laboratories division, now an independent company called Hospira. Hospira has stated that the substance is a silicone coating that did not dry properly on the needles, and causes no safety concerns. To date, Hospira does not plan to recall the needles. No adverse events have been reported as a result of using the needles.

Source: NHF Medical Advisory #402

For more information: www.hemophilia.org/News/advisories/ma402.htm

Longer-Acting Factor on the Horizon?

Bayer Biological Products (BP) is developing a longer-acting Kogenate® recombinant factor VIII product. The next generation Kogenate product could cut factor VIII infusion frequency to once a week or less, dramatically improving hemophilia management. Bayer hopes to launch the next generation Kogenate in five years, pending continued positive clinical results, required regulatory reviews and necessary license approvals.

Source: Bayer press release

For more information:
www.research.bayer.com/news/kogenate/page3145.htm

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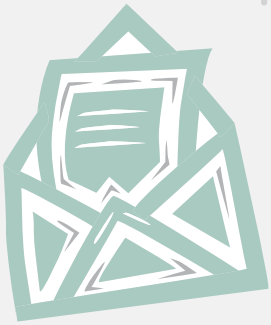
Letters



Readers Respond to "Hemophilia, Incorporated"

I have been involved with the diagnosis and treatment of people with hemophilia since my days as a fellow at Children's Hospital in Boston, 30 years ago. I am writing to tell you how much I enjoy *PEN* and appreciate receiving each issue. The November 2004 feature, "Hemophilia Incorporated," was superlative. In my opinion, *PEN* is by far the best of the many dozens of publications dealing with hemophilia and its treatment that cross my desk. Thank you for the very good work that you do.

George R. Buchanan
M.D., Professor of Pediatrics, UT Southwestern-Children's Medical Center
Texas

I want to congratulate you on a terrific issue of *PEN*. It is incredibly informative and spells out current market issues in very clear and understandable terms. It is a terrific piece of work–a must read for parents and all interested stakeholders.

Christopher P. Healey
Executive Director, North America and Source, Plasma Protein Therapeutics Association

What a fantastic job you all did with this issue. We definitely need to wake up the community... and fast.

John Williams
Florida

This was one of the best hemophilia newsletters I have ever read. A lot of newsletters say how important it is to know certain things; your issue said that, *plus* what it is that we should know. Everything was presented in clear and easy-to-read articles and I truly learned a lot.

Mark Zatyrka
Connecticut

We found the latest edition of *PEN* fascinating. Thank you for addressing the crucial issue of factor reimbursement. The price of healthcare in general–and hemophilia factor in particular–and the resulting effect on insurance costs and families are of major concern.

In our own instance, we've seen factor skyrocket from $15,000–$20,000 per year 20 years ago to $300,000 today. This current cost is for a lower-priced monoclonal product, and without the extra expense of an inhibitor or prophylactic treatment. These costs quickly devastate insurance policies with a typical $1 million lifetime cap.

Our employer is self-insured, and we hit our employer's cap in fewer than four years. We brought the situation to attention of our employer, who negotiated with the homecare company for a 50% reduction in factor price. The fact that a homecare company can so easily reduce its prices while absorbing a 20% copay shows the huge profits involved.

The factor manufacturers and homecare companies have successfully courted the hemophilia community, and we've had a good working relationship with them. However, rapidly rising costs suggest that the industry may not be acting in our best interest.

We appreciate the concerns you raise about assuring that those with bleeding disorders continue to receive appropriate healthcare and treatment. We agree that as a community, we need to band together once again to defend ourselves. However, we must disagree with you that the insurance companies are the ones we need to defend against. Over the years, most of us in the hemophilia community have had numerous battles with insurers while trying to get reimbursement for

necessary care. Rising insurance premiums and copays impact us all. However, in view of the escalating costs of prescription medications, should we be so quick to vilify insurance companies?

If *all* the payers refused to pay the current prices and negotiated lower rates, the suppliers would be forced to realign their practices. Can we do this? Yes, we can. But in order to make it happen, everyone has to participate.

Medicare and some insurance companies are already setting limits on what they will pay. As a community, we cannot stand for a third party telling us how much of what kind of factor we need. We also cannot abide the manufacturer or supplier forcing us to choose a lesser product. The hemophilia community will be best positioned to preserve treatment options if we are part of the reform process rather than an adversary to those paying the bills. Let's join forces to work toward reducing the price of factor.

Some suggest that challenging the drug industry's current hemophilia model would endanger the financial support the hemophilia community receives. However, the thousands given to camp programs, local chapters, scholarships and newsletters pale in comparison to a system that is conservatively estimated at $20 billion–and the impact that high factor prices have on everyone affected by hemophilia.

We need to publicize our plight in the national media, through the NHF, in our communities, with our local representatives and anyone who will listen. We are joining the rising tide to effect change for the betterment of us all. The time is now.

Miles B. and MaryElizabeth Norman
New York

It has been three hellish months since my baby was diagnosed with severe factor VIII deficiency. It still feels strange to write the words, and even more strange to look at a perfect, healthy baby and know the painful and uncertain future that will unfold.

During the past few months, I have fought to keep my son insured, struggled to afford the costs of what the insurance doesn't cover, and prayed to my God for a cure. It truly sickens and scares me to know that insurance companies are lobbying to cut our coverage and choose product for us. I am aware that these companies are in business to make money, but the bleeding disorder community needs help. We are small enough for the companies–and dare I say the government?–to absorb the cost of our kids' treatment.

Something must be done to help the community on a national level. Our precious babies deserve all available help and medical technology at an affordable rate, and insurance companies and our government should pay up.

Marcy Jones
North Carolina

Correction

In our November 2004 issue, *PEN* incorrectly identified Novo Nordisk as a Swedish company (page 8). Novo Nordisk is a Danish company. On page 10, we incorrectly stated "In 2001, Bayer closed its Kogenate FS production plant to investigate..." In fact, only the production of that one product closed, not the entire plant. We regret these errors, and corrected them in the second printing of 17,315 copies of the issue in December.

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