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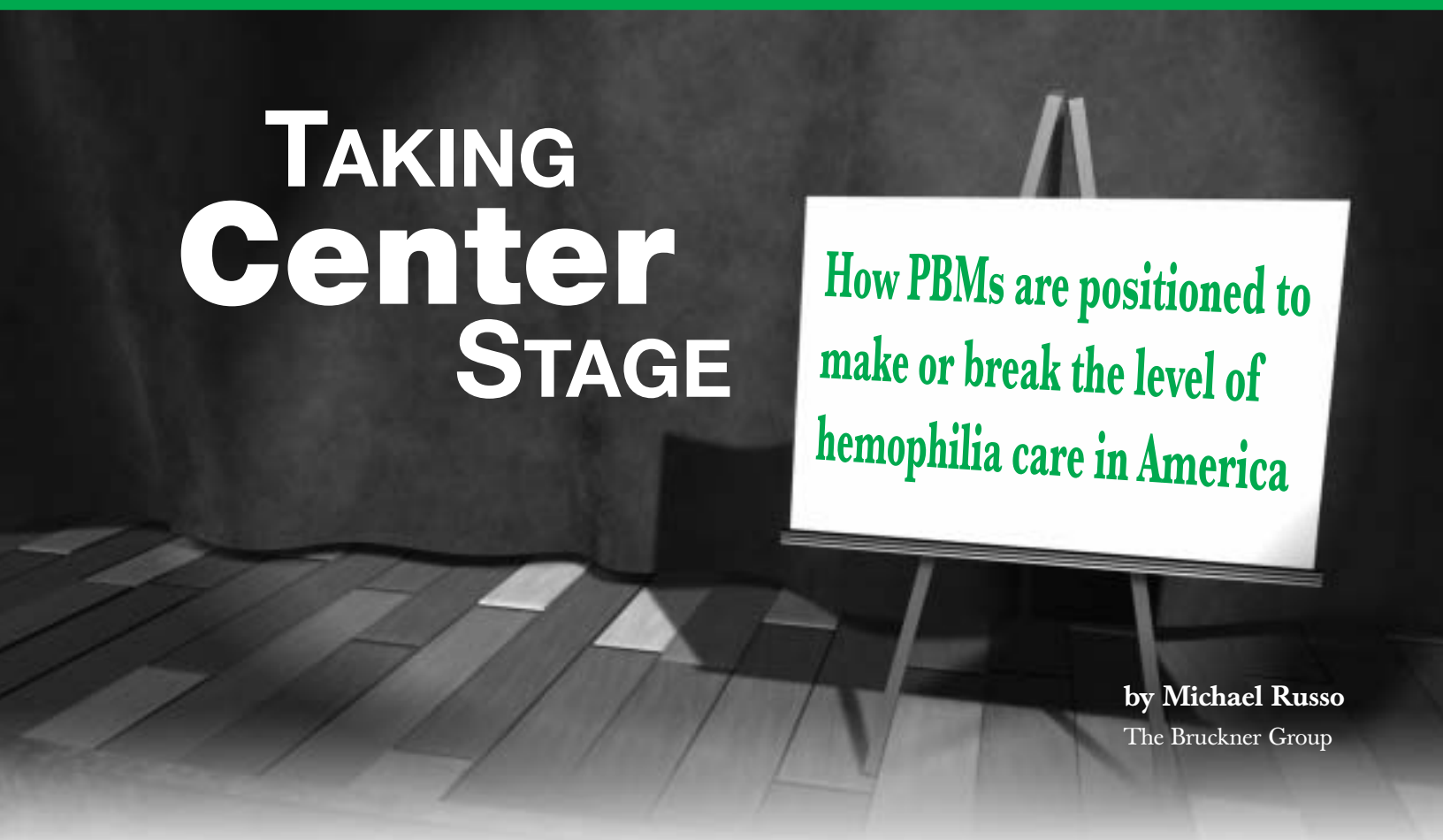
Parent Empowerment Newsletter

Taking Center Stage



by Michael Russo
The Bruckner Group

Hemophilia treatment has reached an unprecedented standard of care, guided and supported by a specialized network for care delivery. In the first two parts of our now three-part series ("Hemophilia, Incorporated," *PEN*, Nov. 2004 and "The Coming Storm," *PEN*, Feb. 2005), we described how changes in this network are threatening the hemophilia community's ability to preserve choice of factor, choice of provider and choice of protocol. In the third part of our series, we profile pharmacy benefit managers (PBMs), the new hemophilia players whose goal is to drastically cut the cost of hemophilia care–mainly by targeting factor prices and usage.

What are PBMs? How will they affect the future of hemophilia care? PBMs are growing in size and expanding in scope, quickly becoming the single most powerful decision maker in hemophilia care. But are they necessarily good for the hemophilia community? It's up to you to decide whether PBMs will bring a new, improved and streamlined system or one that is stretched for resources and will threaten our current standard of care. Your direct involvement could make the difference.

What is a PBM?

If you're unfamiliar with the term PBM, you're not alone. Over 80% of recently surveyed hemophilia patients or their parents (see "Research Bites," page 5) didn't know that PBM stands for Pharmacy Benefit Manager. Throughout the late 1980s and early 1990s, PBMs emerged as businesses hired by health insurers to help design and administer the process of filling prescriptions for their policyholders. In this traditional role, PBMs filled an accounting function–performing duties

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inside PEN

LA Kelley Communications, Inc.

welcome


Sherrell Portrait Design

Our last issue of *PEN* hit an all-time high readership of over 28,000 thanks to extra funding from Baxter BioScience, and because we hit on the year's hottest topic: Who's going to dictate our quality of hemophilia care from now on?

In "Hemophilia, Incorporated" (*PEN*, Nov. 2004) we described the US hemophilia business model as our community has always known it. In "The Coming Storm" (*PEN*, Feb. 2005), Mike Russo and I described the accelerating industry changes that establish who pays for, who buys and who uses factor. What began as a two-part series on the US hemophilia business soon became a three-part series when we realized the vast amount of information we all must learn and the challenges we all face. In a nutshell? Insurance companies and our government–the payers–are gaining tremendous influence in making decisions about our children's hemophilia care. What's their major goal? Cutting costs. How will they accomplish it? That's the scary part.

Sadly, through a "series of unfortunate events," our community was caught napping in what has become the single most important issue since the HIV crisis twenty years ago. Even now, we're just getting our minds around the issues and events that are creating change. All hemophilia advocacy groups must create a master strategy that outlines how to approach reimbursement, enforce protection of quality of life, and ensure choice of product and provider. Although segments of our national community represent different constituents with different needs, we all face the same challenges and risks, and we must present a unified front with a clear focus.

While our advocacy groups and national organizations come up to speed on the changes taking place in the industry, parents and patients must come up to speed, too. This isn't exactly fun stuff to read, but it's vital. Start by knowing your personal insurance policy and situation. Call your insurance carrier, ask questions, and read *PEN*'s last two issues. Bring these *PEN* issues to your local chapter meetings, to your clinic, and share them with other parents. Raise a ruckus! Know what you pay per unit, which factor product you use and, most important, what your insurance payer is doing now that may soon affect your care. Read and re-read our current feature, "Taking Center Stage." Know what PBMs are and how they are changing hemophilia care. PBMs are the future of healthcare–so we'd better get to know them fast and learn how to influence them, or our healthcare future will be theirs to determine.

PARENT EMPOWERMENT NEWSLETTER MAY 2005

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PEN is a newsletter for families affected by bleeding disorders that is produced and edited by a parent of a child with hemophilia. It is a forum that promotes an active exchange of information and support among divergent groups in the national and international hemophilia community.

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letters

Readers Respond to "The Coming Storm"

(*PEN*, February 2005)

"The Coming Storm" was extremely informative and a valuable resource for families with chronic illnesses such as hemophilia. Insurance issues can have a major effect on the hemophilia community. Some of us are already feeling the effects. We need to continue to be more vigilant. Unfortunately, many families don't have a clue about what the future holds. Articles like this one will help educate people who take their child's medical treatment for granted.

Debbi Adamkin
Florida

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by Kris Richardson

Hemophilia Hits the Insurance Radar Screen

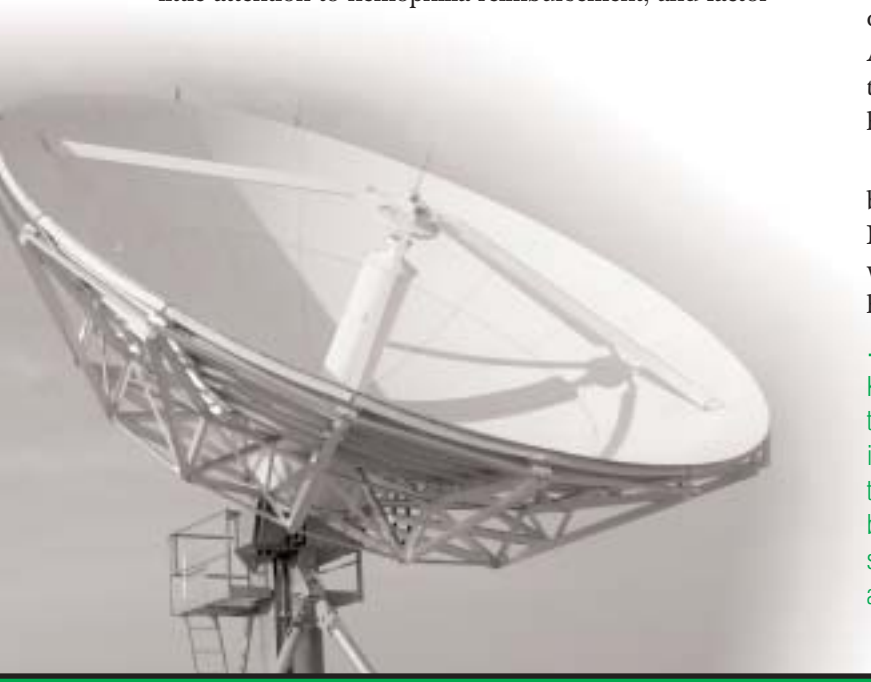
Twenty-seven years ago my family began a roller coaster ride that continues today. With the birth of my son Todd and his diagnosis of severe hemophilia, and the diagnosis of his brother Peter two years later, we were thrown into the ever-changing, always confusing world of chronic illness and insurance. Through the years we learned, often as a result of our own mistakes, how the healthcare system works and what we needed to do to ensure adequate coverage for the therapies our children required.

Fifteen years ago I entered the world of hemophilia homecare as an employee of a nationally recognized homecare company. My knowledge base grew from a personal perspective to include a professional perspective in my position as a consumer advocate and education specialist. I discovered the ins and outs of reimbursement, and I advocated alongside many others in our community to ensure access and quality of care at an affordable price.

One year ago, in January, I opened the only hemophilia medical specialty pharmacy in upstate New York. I am now able to provide to individuals living with hemophilia the same level of service I would expect and have always expected for my own family. It has been an incredible experience, although not without its unique set of problems. It is no longer 1995, when payers paid little attention to hemophilia reimbursement, and factor providers often received 15% to 25% over acquisition price for clotting factor. It is no longer a market where payers will readily carve out hemophilia from other disorders and contract with the consumer's provider of choice. There are no guarantees that an insurance plan will include your homecare company as a participating provider. Hemophilia has hit the radar screen.

Insurance is a business, and any good business will target its highest costs and determine ways to reduce them. For PBMs, the acquisition of homecare companies specializing in high-cost therapies such as hemophilia is a brilliant business move. Who else but PBMs can dictate which homecare company will provide services to indemnity plan owners, or close a network with only two providers: themselves and one other? Who else can purchase at a high enough volume to attain the lowest acquisition costs, then set their own reimbursement rates? Who else can set the rates that often force the smaller, independently-owned homecare companies out of the network by reducing their profit margins to unacceptable levels? Who else can refuse to pay for the very drug you and your physician have determined will work best for you or your child–because it isn't "cost effective?" Who do you know that can buy, dispense, bill and pay themselves for factor products? And who is watching over this marketplace to ensure that the best interests of consumers are not forsaken as hemophilia big business threatens to become a monopoly?

My son's care has always been a collaborative process between family, healthcare provider and service provider. I want it to remain that way. As a business owner, I want competition; it's what keeps a market honest and healthy. From where I sit, it's all about choice.



Kris Richardson is president of U & I, Inc., USA. She has raised two sons with hemophilia, and has more than 18 years' experience in the hemophilia homecare business. A former elementary school teacher, Kris founded Camp High Hopes, New York's only camp for boys with Bleeding disorders. Kris's presentations as a motivational speaker encompass such topics as AIDS, Enhancing Self-Esteem and Caring for the Caregiver.

A Pioneer at Age 5

by Julia Q. Long

photos: Alex Bediako

Kojo and little sister Maame Akua Serwaa: Kojo is one of the few patients in Ghana to receive factor.

Nana Kojo Opoku Bediako is a big name for a small boy. "Kojo," who will be six on August 9, has sparkling brown eyes, a beaming smile and factor VIII deficiency. In February Kojo became the first Project SHARE℠ factor recipient in Ghana, and possibly one of only two children in Ghana ever to receive factor concentrate.

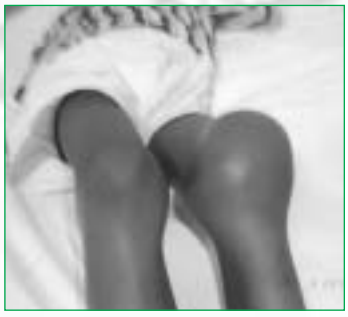
Kojo was born in a gold mining town in the Ashanti Region, and is the elder of Alex and Georgia Opoku Bediako's two children. "We realized something was wrong with his blood clotting during his circumcision," recalls Alex. Although they managed to contain this bleed and "other signs of internal bleeding during inoculations and vaccinations" for several years, Alex and Georgia naturally were distraught when Kojo suffered for days and weeks with no real idea of what was wrong. Doctors told Alex and Georgia to try to prevent injuries, and to use painkillers when Kojo's pain became unbearable. Keeping a toddler away from injuries is difficult enough, but having to decide when pain becomes "unbearable" is a heartbreaking task for any parent.

In September 2004, after a month-long knee bleed that was not eased by pain medication, Kojo was officially diagnosed at Ghana's main hospital, the Korle Bu Teaching Hospital. The family was relieved to receive a proper diagnosis for Kojo's problem, but their worst fears were confirmed: there is no hemophilia care in Ghana. "We asked for help from hemophilia societies in the UK, Australia and South Africa via the Internet," says Alex. "One member of the Haemophilia Society (UK) recommended I seek help from Project SHARE. I wrote to SHARE and they replied immediately with an offer to send factor for Kojo."

SHARE contacted Kojo's doctor and sent illustrated instructions on infusing. With physician contact established through email, and customs cleared, SHARE sent a trial shipment of factor. The infusion was successful, and the Bediakos were overjoyed.

Although Kojo missed six months of kindergarten, his teacher sent assignments home so he could keep up with his classmates. Alex says proudly that Kojo is a "brilliant" student and loves to read. When he is not doing schoolwork, Kojo enjoys playing with building blocks, or having fun with his little sister, Maame Akua Serwaa.

Kojo's story demonstrates how the needs of one child can pioneer hemophilia care in a developing country. The collaborative efforts of Alex and Georgia, the Haemophilia Society, Project SHARE, and Kojo's teacher and doctors have helped give Kojo what he needs to be a healthier, happier boy. These efforts may also pave the way to help many more children like Kojo.



Kojo's knee before help arrives from Project SHARE.

To learn more about Project SHARE℠ and how you can help patients like Kojo, please visit www.kelleycom.com/iha/projshare.html or contact Director Julia Q. Long at (978) 352-7657 or julia@kelletycom.com.

Taking Center Stage... continued from cover

like checking eligibility and initiating claims. But they quickly grew beyond their initial purpose, and over the past decade have solidified their position as a dominant power in healthcare, even exerting direct influence over clinical decisions such as treatment. On behalf of an insurance company, a PBM can now approve or deny coverage of a physician's or patient's choice of drug or dosing schedule if it is not in compliance with the PBM's preferences and regulations (see "The Traditional PBM Role," page 6). Although they have grown beyond simple pharmacy benefit management, the PBM label stuck.[1]

You probably already use the services of a PBM. If you receive drugs through mail order or show an insurance card when you fill a prescription at a pharmacy, a PBM is probably managing the process of filling and paying for your prescription. PBMs are ubiquitous: according to the Federal Trade Commission (FTC), PBMs handle 95% of all prescriptions filled for people with prescription insurance coverage. In this respect, PBMs act like a middleman. They purchase a drug from the manufacturer at a negotiated price, and then are reimbursed by the insurance company for the cost

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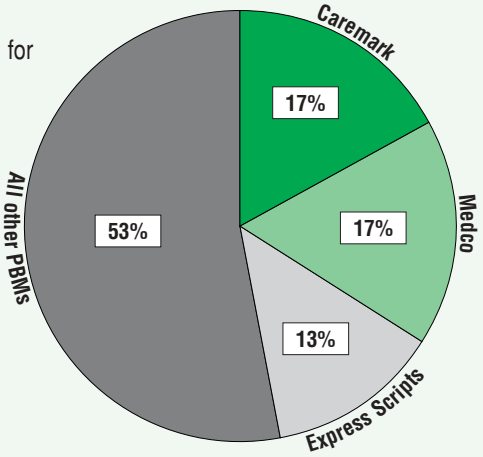
[1]A company is often associated with the product that made it famous. No matter how much clothing Nike makes, for example, most people think of Nike as an athletic shoe company. The same is true in healthcare. Despite their expanded functions, companies like Medco are still referred to as PBMs. Referring to the "PBM side" of one of these companies isolates it from the corporate entity. To add to the confusion, many other players, including payers and specialty pharmacies, have taken on aspects of pharmacy benefit management but are not commonly referred to as PBMs.

Prescription Giants

The top three PBMs filled nearly 2 billion prescriptions in 2004—almost half of all prescriptions in the US.

Because mail-order prescriptions are filled for three months—three times the number of doses of retail pharmacy prescriptions—the number of prescriptions reported here considers a mail-order prescription to have three times the value of a retail one.



To reflect its post-merger market share, Caremark's data includes any 2004 prescriptions that were filled by AdvancePCS prior to the completion of the two companies' merger.

Source: Company-released 10k reports, FY2004. IMS Health.

RESEARCH *BITES*

Unified in Need, But in Need of Knowledge

Ten years ago, most surveyed hemophilia patients rated product safety as their greatest concern. Just recently, when asked again to rate their greatest concerns for the future, a whopping 85% of families mentioned insurance coverage and preservation of choice of product. In addition, 100% of those surveyed do *not* want insurance companies to dictate factor brand.

To protect their insurance benefits, most members of the hemophilia community need a big boost in knowledge. Today PBMs are working with insurance companies to control drug costs—in some cases by limiting choice. Although PBMs are fast becoming lead players in the hemophilia industry, 80% of survey respondents don't know what PBMs are. And the vast majority of respondents rely on passive methods like letters from their insurance companies to learn about coverage changes.

One surveyed parent warns, "Find out about your insurance coverage now, or find out the hard way…. after [it changes]."

From a preliminary study by LA Kelley Communications and The Bruckner Group.

The Traditional PBM Role

Now that hemophilia care has moved into the PBM arena, it's critical that every member of the community know the evolving functions, policies and vocabulary of PBMs. The traditional PBM role covers four main responsibilities.

1. **Determine the formulary.** A formulary is a complicated system of classifying both branded and generic pharmaceuticals according to the drugs your insurance company prefers you to use for a particular therapeutic need. The formulary is usually broken up into tiers, each with its own copayment (see illustration below).

   Formulary details vary by health insurer and policy, based on employer needs and resources. But because PBMs perform most of the final analyses that determine the formulary, PBMs are an integral part of the process and exert a powerful influence over which drugs are available to which patients.

2. **Provide a network of retail pharmacies and a mail-order pharmacy service.** PBMs contract with retail pharmacies, such as your local CVS, Walgreens or Wal-mart chains, to accept prescriptions from their customers. By using these "in-network" pharmacies, you can use your insurance coverage to save money when you fill a prescription. An "out-of-network" pharmacy may not accept your insurance, and you will be charged the full cost of the drug. The PBM usually limits a retail pharmacy to dispensing a 30-day supply.

   The major PBMs also fill prescriptions through their own mail-order pharmacies. PBMs purchase medications directly from drug manufacturers, mail them to you, and obtain a reimbursement payment from your health insurer. A PBM's mail-order service usually allows up to a 90-day prescription for the same copayment you would pay for the 30-day supply at the retail pharmacy.

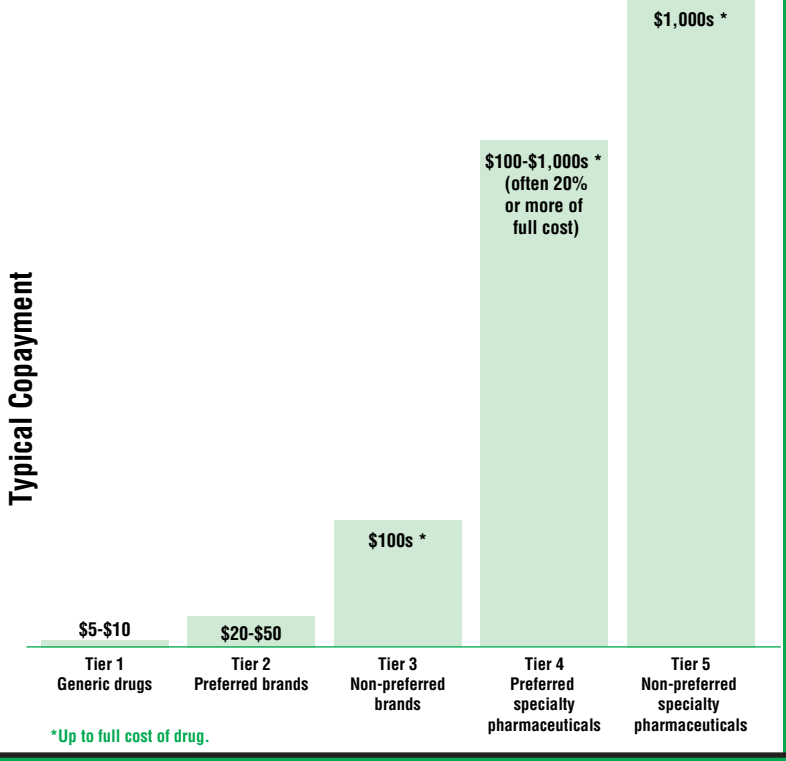
3. **Negotiate drug prices with pharmaceutical manufacturers and reimbursement levels with insurance companies.** PBMs handle a tremendous amount of medication, giving them strong negotiating powers to obtain lower prices from manufacturers. Sometimes, a PBM designates a particular manufacturer's drug with a preferred formulary status as part of the deal. PBMs also negotiate with private health insurers to maximize the amount that PBMs will be reimbursed for their services.

4. **Streamline processes and maintain quality.** PBMs reduce the overall cost of filling your prescriptions by streamlining the process. Instead of a large number of insurance companies, manufacturers and pharmacies dealing independently, each with its own paperwork, formularies and procedures, PBMs create "one-stop" shopping.

   PBMs often have other added-value services, such as the ability to crosscheck a patient's medications for dangerous interactions, and call-in services for patients to obtain advice from pharmacists.

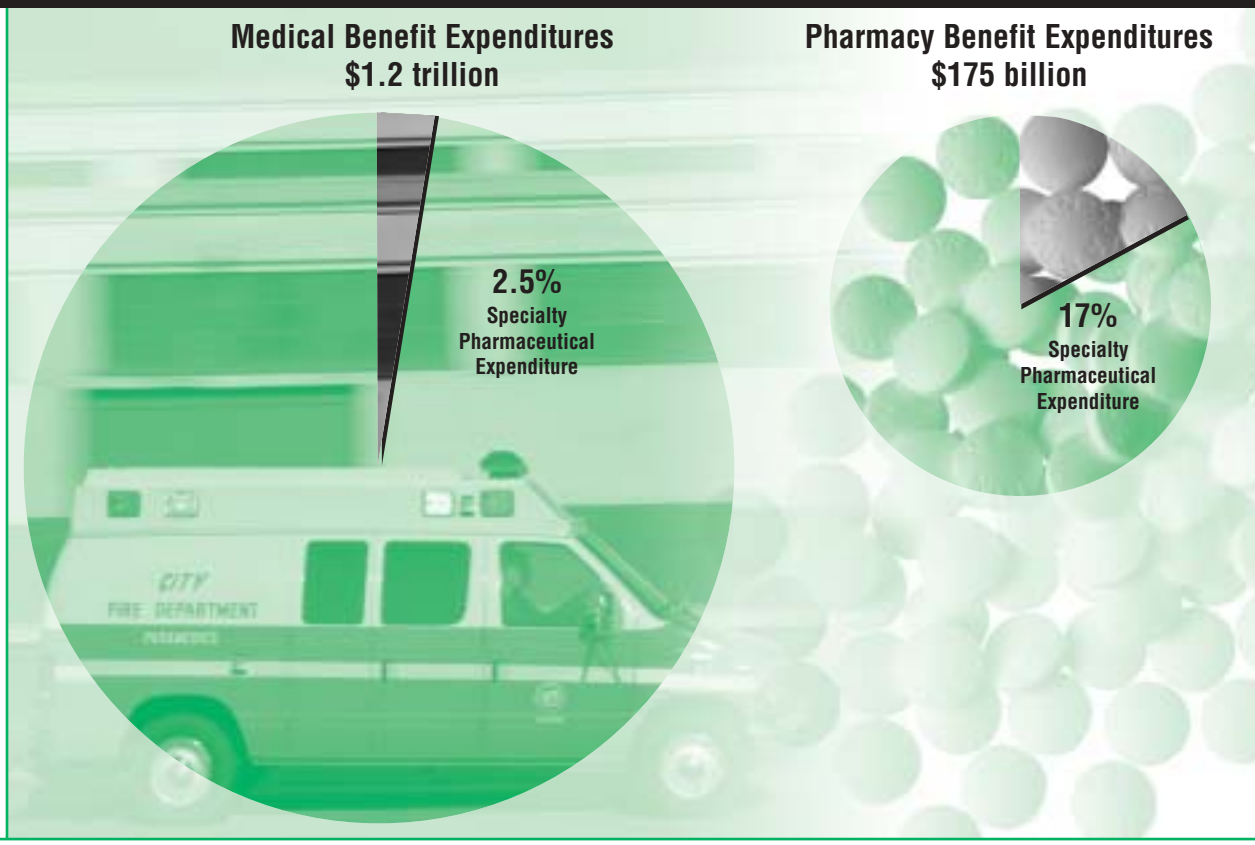
Do You Really Have Brand Choice?

A formulary places drugs in different tiers, each with its own copayment. Copayments can vary from one health plan to another, but they are always set up to give the patient a financial incentive to choose the drug that is best for the PBM's bottom line. If you insist on using a specific brand that is not preferred by your PBM, you may have to pay top dollar for it.



Factor Becomes a Target

At 2.5% of total reimbursed expenses, specialty pharmaceuticals were safely buried in the medical benefit portion of health insurance plans. Now, covered by the smaller pharmaceutical benefit, specialty pharmaceuticals represent 17% of expenditures—a prime target for cost cutting. Currently, factor represents a whopping 7% of the $30 billion spent annually on all specialty pharmaceuticals.



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of the drug plus a small fee. There are currently about 50 PBMs in the US. The three dominant companies–Medco Health Solutions, Caremark and Express Scripts–have a combined prescription market share of about 50% (see "Prescription Giants," page 5).

Since the late 1980s, PBMs have enticed their clients–health insurers–with promises of cost savings by streamlining processes and negotiating better drug prices from manufacturers. As pharmaceutical spending grew by leaps and bounds, insurers eagerly signed on, and PBMs emerged as major players in the healthcare industry. In fact, the PBM model of actively managing insurance costs has quickly and powerfully altered the way pharmaceuticals are bought, sold, prescribed and used. According to many reports, including a 2003 report from the US Government Accountability Office (GAO)[2], PBMs' data-driven cost savings techniques and buying power have delivered extensive savings to health insurance carriers.

Driven by the need to grow, PBMs have adapted quickly, always looking for new products to offer, new areas to manage and new ways to cut costs. It was only a matter of time before they found hemophilia.

PBMs Become Involved in Hemophilia

Factor is an injectible: a "specialty pharmaceutical." Before PBMs turned their attention to specialty pharmaceuticals, health insurers categorized the cost of using these drugs differently from the cost of oral or topical drugs available at local pharmacies. This difference in categorization protected factor from PBM cost-cutting efforts. As injectibles, factor products were often delivered in a clinical setting, so they were charged to the *medical benefit*, outside the reach of PBMs. The medical benefit is typically managed directly by the health insurance company, which lacks the ability to closely monitor the use of specialty pharmaceuticals (see "Speaking PBM Language," page 8). But over the last few years, health insurers gradually moved the costs of specialty pharmaceuticals, including factor, to the *pharmacy benefit*–where they became obvious targets for PBM cost cutting. Factor alone now accounts for $1.14 of every $100 spent on all pharmaceutical products (see "Factor Becomes a Target," above).

Factor's position as a PBM target is an urgent matter that the hemophilia community must now address. Several years ago, the most proactive PBMs rolled out programs to lower factor costs. Because they collectively served only a small number of hemophilia patients, these PBMs have quietly reduced overall factor usage–negotiating better prices with manufacturers and streamlining the economics of delivery. One affected health insurer reports that it was able to reduce inappropriate factor usage by 10% to 20% in one year. Combining reduced usage with lower negotiated purchasing prices, the health insurer claims that it lowered factor costs

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[2]The Government Accountability Office is an independent and nonpartisan agency that studies the way our federal government spends taxpayer dollars. It is commonly called the investigative arm of Congress or the congressional watchdog. *Source*: www.gao.gov/about/what.html

Speaking PBM Language

What is a pharmacy benefit, anyway?

Your health insurance policy has two major parts, each with a separate department and budget, and usually with separate staffing.

The medical benefit. This part of your policy covers doctor visits, diagnostic tests, surgery, and any other clinical services. The medical benefit for everyone in the US with insurance coverage totals $1.2 trillion annually.

The pharmacy benefit. This part of your policy includes drug coverage for your medications outside of a hospital stay. The pharmacy benefit often has a lifetime cap of $1 or 2 million, which is practical for the average patient but clearly inadequate for someone with hemophilia. The pharmaceutical benefit for people insured in the US is a comparatively scant $175 billion annually.

What is a specialty pharmaceutical?

Specialty pharmaceuticals are drug therapies that encompass a range of human proteins, blood products and various other injectibles. These therapies are produced in one of two ways: 1) Created outside the human body by living cells that are genetically engineered to make the protein (known to the hemophilia community as recombinant products). 2) Removed directly from human donor tissue or blood (plasma-derived). Factor is a specialty pharmaceutical. These drugs are often expensive to make and expensive to stock, and have relatively short expiration dates. They are delivered to the patient either directly by a healthcare professional or by a trained caregiver at home.

Specialty pharmaceuticals are very different from non-specialty drugs. Non-specialty pharmaceuticals are small chemicals that can be produced by comparatively ordinary chemical processes in large batches. They are usually stable at room temperature and are delivered as pills, nasal sprays, inhalers, and topical creams or ointments.

What is a specialty pharmacy?

A specialty pharmacy is a company with a pharmacy that distributes specialized biologicals and blood products. These products are too expensive, too infrequently ordered, or require too much care in handling for local pharmacies or routine mail-order services. To the hemophilia community, specialty pharmacies are commonly known as “homecare companies.” But unlike hemophilia homecare companies, specialty pharmacies might carry more than 150 products in 50 different disease areas.

What is disease management?

For decades, independent companies, academic institutions and hospitals have analyzed the treatment patterns of patients with chronic disease to find the best approaches to their care. Such studies have most often been performed for physician professional societies and patient advocacy societies, as well as for political organizations and insurance companies. These typically publicly distributed studies incorporate all therapy and lifestyle choices that could affect the health status of a patient with a particular disease: doctor and hospital visits, medications, nutrition, smoking or alcohol habits, compliance with doctor’s orders, support and education. Researchers determine how such choices contribute to the overall stability of patients. As healthcare value has become increasingly important, more of these studies have also incorporated the *overall cost* of treatment. What often emerges from this kind of analysis is a disease management plan that maximizes patient care and health while trying to minimize cost and eliminate waste.

Disease management plans also create a system for determining the most likely therapy needs for different patient types. In a simplified hypothetical example, the plan may determine that, on average, a ten-year-old severe factor VIII deficient child using on-demand treatment “should” use about $100,000 of factor per year. From this framework, health insurers can determine whether a patient is using resources in excess of what “should” be required to achieve excellent clinical results. Health insurers can then assign caseworkers to the most extreme cases, to help patients properly comply with therapies and make lifestyle adjustments to improve their health while using fewer resources.

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from $7.7 million for 47 members in 2001 to $6.8 million for 66 members in 2002.[3] These statistics also indicate a 36% reduction in factor costs per patient per year, from $164,000 in 2001 to $104,000 in 2003. Such victories have helped energize a steadily growing cost-cutting movement that will reach every factor user in the US over the next few years.

But this kind of success isn't the whole story. Many PBM programs aimed at controlling the costs of drug therapy, while innovative, aren't fully transferable to hemophilia. Streamlining and cost cutting that has worked well for other diseases could be destructive for hemophilia treatment. For example, cost cutting must not interfere with prescribed factor dose or frequency, factor brand choice, or essential educational resources. These treatment benefits aren't a luxury; they are the core of live-saving and health-building hemophilia therapy.

Streamlining and cost cutting that has worked well for other diseases could be destructive for hemophilia treatment.

But not all cost cutting is bad. The analyses that drive cost-cutting efforts often result in better resource allocation and improved services. Hemophilia treatment can benefit from such an honest assessment. Traditionally, the costs of many delivery and support services for hemophilia have been added to the basic per unit price for factor; so the actual cost of each component–nursing, ancillaries, factor–is not delineated. PBMs can separate out these add-on services from the per unit price of factor, thus removing them from the pharmacy benefit. The resulting lower per unit factor prices would mean longer-lasting insurance caps. Further, by identifying the add-on services with the greatest healthcare value, PBMs can ensure that these services remain exceptionally well funded and staffed even in the face of cost cutting.

The key to preserving our current standard of hemophilia care is to ensure that the entities making cost-cutting decisions, such as PBMs, are educated about *this* particular disease and *this* community's unique needs. Decision makers must employ a long-term vision toward maintaining high levels of patient care and quality of life, despite the immediate call for trimming existing fat. Throughout this process, as large amounts of patient data are lumped together to examine overall costs, the needs of the individual patient cannot be compromised.

Ask yourself: *What do I value most in hemophilia care?* Choice of brand? Choice of factor provider? Preserving your insurance lifetime maximum? Continued prophylaxis? Are these considerations just as highly valued by your insurance company and product provider? Do you know which PBM cost-cutting programs and initiatives could most threaten the standard of your care? What can you do to protect what you value most?

PBMs Under Fire

Over the last decade, PBMs have grown in popularity and strength. But they have also faced extreme scrutiny by health insurers, employers, advocates and government agencies concerned with centralization of power, growing conflicts of interest and lack of transparency.

PBMs have been accused of putting their own financial needs ahead of the needs of clients and patients. Recent lawsuits and inquiries question whether PBMs have abused their position, because PBMs are able to both negotiate financial deals with manufacturers *and* make product and brand decisions on behalf of their clients. PBMs are accused of essentially acting as both buyer and seller while maintaining a veil of secrecy and refusing to disclose arrangements to clients, and even to the GAO.[4] As a result of these conflicts of interest, health insurers and employers have sought to reduce their reliance on PBMs and to obtain greater transparency in PBM dealings.[5]

The risk that these conflicts of interest represent to the hemophilia community has intensified in the past two years. During this period, a flurry of mergers and acquisitions has created companies that are the result of combining PBMs, specialty pharmacies, and hemophilia specialty homecare companies. These newly expanded entities have eliminated provider choice simply by consolidating. They have also created new conflicts of interest regarding the buying and selling of factor and the delivery of related hemophilia care services.

PBM Expansion: Reducing Options, Creating Conflict

From 2000 on, PBMs have grown tremendously due to two strategies. First, PBMs have consolidated market share and geographic coverage by merging with each other, creating a handful of dominant companies. Second, by expanding into specialty pharmacy and disease management, PBMs have absorbed functions that they had previously outsourced. In fact, every major PBM has acquired a leading specialty pharmacy (see page 10, "Is PBM Expansion a Conflict of Interest?"). Hemophilia revenues–which represent 33% of one major specialty pharmacy's earnings–play a large role in PBMs' merger decisions.

Before these mergers, the process of delivering hemophilia care was divided among several separate entities, including specialty pharmacies, homecare, HTCs, and disease management. Because many different companies were involved, the industry maintained a natural system of checks and balances. Multiple players in each category had to compete openly for

[3] *Specialty Pharmacy News*, v1n9, October 2004. [4] There are dozens of past and pending lawsuits and investigations regarding the practices of PBMs. In 1993, the federal government determined that a close arrangement between a PBM and a manufacturer resulted in the preferential sale of all eight of the manufacturers' top products, to the exclusion of many legitimate alternatives. In 2004, a major PBM settled a suit with the Department of Justice and 20 state Attorneys General accusing the PBM of switching patients to certain brands in order to get larger rebates from manufacturers. PBMs have also been accused of keeping such rebates rather than passing them along to their health plan clients. The FTC is engaged in an ongoing investigation to determine whether a PBM's ownership of its own mail-order service represents a conflict of interest. [5] Two different groups of large employers are attempting to contract directly with a PBM to ensure a model of complete transparency in which the employer groups retain the authority to have the PBM audited by an outside firm. Health insurers Cigna, Aetna and AmeriHealth/Mercy brought their pharmacy management and specialty pharmacy services in-house, reducing their need for an outside PBM.

contracts, offering better prices and additional services to win business. Now, because the largest PBMs have combined many of these companies–and their services–under the umbrella of one corporate PBM entity, the hemophilia community must be concerned that PBMs may start making self-serving decisions among their internal divisions without external oversight.

Conflicts of interest create an environment in which PBMs can make decisions that are in their financial best interests, but not necessarily in the clinical or financial interests of their clients and patients. For hemophilia, as we explained in "The Coming Storm," these decisions could mean limited choice of factor provider, brand and dosing regimen. Negotiating factor prices, selling factor through a specialty pharmacy, and providing homecare services requires *transparency*–especially with limited or nonexistent bidding from competitive providers. A neutral outside group must be able to examine a PBM's books and contracting arrangements in order to detect unethical activities.

Although these conflicts of interest still must be addressed, PBMs have already identified hemophilia as a target for cost cutting. The first round of PBM cost cutting is still underway; it focuses mainly on the overall cost of factor, through attempts to reduce both usage and price. PBMs have applied two traditional tools to this effort–formularies and the development of an in-network provider system–as well as one of their expanded programs, disease management.

Using Formulary and In-Network Providers to Control Factor Costs

PBMs have a decade-long history of using formularies–their managed drug lists–and in-network providers as a way of controlling the costs of pharmaceuticals. Most PBMs have now extended the use of these tools to manage factor costs as well.

Many PBMs already offer health insurers the option of putting factor on the formulary of their health plans. This opens up the possibility that factor will be subjected to any or all of the same restrictions that currently dictate the use of other pharmaceuticals on the formulary: prior authorization, preferred brand, and "step" programs. Once on formulary, factor costs will be routinely reviewed by the PBM. Based on these reviews, a PBM can tighten its control over factor choice and price by incrementally imposing new limits. For example, your formulary might initially offer all brands of factor. But over time, you might be asked to switch to a preferred brand. The PBM may switch you not for a clinical reason, but to obtain a lower negotiated purchase price. PBMs are also likely to place additional restrictions on factor use through their formularies–for example, a limit on the amount of factor delivered at one time. Instead of allowing a three-month supply, your insurer may allow only a 30-day supply per prescription. Or a PBM may issue specific limits on dose and frequency based on a patient's clinical profile, severity and health status. Finally, a PBM might require re-authorization of your factor prescription from your doctor on a regular basis.

In addition to using formularies to control factor use, an increasing number of PBMs are also dictating which companies will deliver your factor. These factor providers are under contract to follow the restrictions that the PBM places on factor use. Even more directly, the PBM may own the factor provider that you are required to use.

For people whose health insurers use a PBM, hemophilia care delivery will be subject to the same rules governing a PBM's retail and mail-order pharmacy network. These rules

Is PBM Expansion a Conflict of Interest?

Current Corporate Entity	Merged Companies		
	PBM	Specialty Pharmacy	Specialty Pharmacy with Hemophilia Focus
Medco Health ←	Medco	Accredo	Nova Factor Hemophilia Health Services (HHS) Alpha Therapeutic Services (ATS)
Caremark ←	Caremark AdvancePCS	Accordant Theracon	Choice Source Therapeutics
Express Scripts ←	Express Scripts	CuraScript	

During the past three years, PBMs have scrambled to acquire specialty pharmaceutical and homecare companies. Each PBM has acquired one of the major specialty pharmacies. Is this a conflict of interest? The acquisitions create larger corporate entities that both buy and sell factor, while also trying to cut the costs of factor. Other large PBMs without a hemophilia focus, such as Walgreens and CVS, also have their own specialty pharmacies.

include a choice: obtaining factor from an approved in-network specialty pharmaceutical provider for a low copay; or obtaining factor from an out-of-network provider for what could be an astronomical copay. While "choice" appears to be available, it may soon be impractical or impossible to actually choose your own factor provider.

Without community intervention, the current hemophilia delivery model is likely to change from one that offers a variety of provider choices and brands to one in which the only affordable choices are the providers owned by the PBM and the brands with the cheapest price tag.

Hemophilia Disease Management

Another way for PBMs to control costs is to apply principles of "case management" to hemophilia, as they have done in more than 20 other disease areas. In one sense, this strategy is logical: PBMs' extensive databases and data analysis abilities make them the perfect powerhouses for creating disease management programs. Using these abilities, PBMs have initiated programs to determine the therapies and lifestyle changes that will most benefit patients at the best cost and value. But many watchdog groups and health advocates are now questioning whether PBMs are capable of objectively creating and running disease management programs because of the direct financial stake they have in the programs' design and execution. These concerns are especially relevant because PBMs are unlikely to volunteer their data and analytical process for public scrutiny.

It's up to you to decide whether PBMs will bring a new, improved and streamlined system or one that is stretched for resources and will threaten our current standard of care.

A primary goal of hemophilia disease management is to help patients take the right amount of factor on a proper schedule to achieve patient stability and avoid a serious adverse event. In support of this goal, there is evidence that improper factor use strains the healthcare budgets of insurers and employers. One insurer found that "the difference between appropriate and inappropriate treatment, which typically stems from improper [factor] dosage amount and frequency, can be $20,000, $30,000, $40,000 or even $50,000 per member per month."[6]

Unfortunately, after examining their entire patient population with a particular disease, what a PBM might consider "right" and "proper" dosing may differ from what actually works for individual patients and their physicians. One health insurer explains that the company's process to help match its disease management patients with the appropriate therapy "identifies and stratifies individuals with applicable diseases, using patented algorithms to analyze procedure, drug and diagnosis codes from claims data." In other words, health insurers use computer software to perform a complicated series of analyses on an enormous amount of data. While such analyses certainly provide guidance and are likely performed by skilled staff, "patented algorithms" and the result of a computer analysis should not interfere with physician, parent or patient decisions regarding the unique needs of an individual with hemophilia.

Warning Signs for the Hemophilia Community

New restrictions and programs rolled out by PBMs evolve slowly, building on one another. Each incremental policy change may not seem relevant or alarming, but together these changes can result in tremendous upheaval. The hemophilia community can expect that as new rules and regulations emerge, PBMs will describe them as "improvements in service and support" resulting in improved patient care. PBMs may go so far as to claim that any resulting cost savings will be passed on to you. But remember this: *you* are not the PBM's customer. The PBM's customers are employers and health insurers to whom they have promised deep cost savings. Cost-cutting programs are implemented by PBMs because health insurers lack the resources to fully serve *all* the needs of *all* their patients. As costs are reduced in one treatment area, the resulting savings are made available to fund other treatments for other diseases. The savings are not put back into your pocket. Perhaps over the long haul, cost-cutting programs that put restrictions on your health coverage will reduce the rate that your premiums increase; but in the short term, savings will be passed on to you only in the unlikely event that health insurers and PBMs can't find a use for the money.

Unfortunately, many people don't routinely track the modifications made in their health coverage, so they miss the gradual changes that eventually affect their healthcare needs. Often a patient's first encounter with a new cost-cutting program occurs when a prescription gets rejected, new forms need to be filled out, or the copay has increased. Your physician may not even alert you to these changes because he or she may be required to comply, or may risk not getting paid by the insurer for the patient visit. Before you can challenge any program that might compromise your health, or your child's, you must first know that such a program exists. How to do this? By consistently monitoring changes in your insurance coverage. More important, you must be ready to challenge the seemingly small changes in coverage that are the building blocks of broad, larger restrictions. Once a series of incremental changes is implemented, it's difficult to challenge any new alteration that appears to be a tiny and logical addition. For example, removing one factor brand might not seem important until the next removal, and the next, and the next. To protect your interests, watch for changes, however small, and monitor the effects they may have on your treatment decisions and standard of care.

[6] *Specialty Pharmacy News*, v1n9, October 2004.

Moved to a PBM Without Your Knowledge

Have YOUR rights been violated?

As some PBMs, homecare companies and specialty pharmacies merge, and others re-negotiate their contracts with each other, your health insurer may legally transition you to a new factor provider. If this happens, you may be angry that you were switched to another company without your consent. Many switched parents feel that their privacy has been breached. While you may worry about losing control of your child's care, remember that this kind of "customer migration" is considered normal business practice. Your primary concerns should be: *What does the switch mean for the standard of my child's care? Will more switches happen in future, with greater frequency?*

Changes Affecting Factor Brand Choice

- Lower copayment for a preferred brand that forces you to use a brand you don't want.
- Prohibitive copays on a brand you want.
- "Step" treatment program that directs you to try a specific brand first.
- Declaration that all brands are equal, perhaps even lumping together recombinant and plasma-derived.
- Attempts to move patients to lower-priced plasma-derived factor.

Changes Affecting Choice of Factor Provider

- Restricting choice of homecare or mail-order provider.
- Copayments that vary based on the provider you choose.
- Acquisition of a homecare company or specialty pharmacy service by your health insurer or PBM.

Changes Affecting Treatment Regimen

- Questions from an insurance or PBM representative about a patient's factor usage.
- New and more frequent schedule for authorization and re-authorization of your factor prescription.
- Introducing a new hemophilia disease management program, even if voluntary.
- Restrictions on dose or frequency.
- Development of patient categories by the health insurer or PBM, based on severity, age, health status or previous factor use, that will "guide" decisions your physician is expected to make for your regimen.
- Restriction of prophylaxis.
- Over-emphasis on limiting risky activities–such as contact sports–and using supportive items (helmets, Cryo/Cuff™) in an effort to reduce prophylaxis.

Hemophilia advocacy groups must watch these possible developments carefully, as some may create an overwhelming financial hardship for some patients, or even eliminate factor coverage for others. Why? Within the next two years, specialty pharmaceutical coverage is likely to be recast by the PBMs as an entirely separate category in health insurance policies, to be called the "specialty pharmaceutical benefit." As a result of this re-categorization, insurance companies will present specific coverage options to employers; employers can then choose among options, depending on how much they are willing to spend. Such choices will range from full coverage, requiring a low patient copayment for each prescription, to no specialty coverage, leaving patients fully responsible for specialty drug costs–including factor.

Pick Your Battles

Although PBMs are gaining control over hemophilia care, it's still early in the process. Now is the time to question and

challenge the changes that will *most* impact your ability to obtain an excellent standard of care. You can't expect to win a battle by fighting every skirmish. Instead, the hemophilia community must join together and prioritize, deciding what's most valued and what's worth fighting for.

Of the many players in the hemophilia community, the most important is the patient. Patients must determine the path that advocacy will take. And advocates must determine what's more important: the clinical functions–factor delivery, determination of dosing regimen, emergency services, education–that are essential to hemophilia care? Or the institutions that have traditionally provided this care? The main task of patients, parents and physicians should be to preserve the services that are part of excellent hemophilia care; *which company* provides these services is a secondary issue. However, if a particular type of company or provider is superior at delivering essential services when compared to the competition, then its survival must be ensured.

Holding PBMs to High Standards

Regardless of how the hemophilia community chooses to respond, PBMs will continue to take on hemophilia with well-conceived plans and tremendous resources. To ensure that PBMs will meet the critical care needs of patients and parents throughout this process, PBMs must be held to high standards of functionality, quality and availability.

Functionality

The hemophilia community has relied on many different companies and organizations to provide a wide range of care and support *functions*–products and services that contribute to patients' standard of care and quality of life. With the rise of PBMs, some products and services may disappear. The community must determine the most valuable of these functions, from factor brand availability to nursing care to scholarships. Which brands, protocols and services are essential to your care or the care of your child? Some of these functions help bolster family stability, keeping parents at work and children at school–but this kind of value may not be evident to a PBM administrator inexperienced in hemophilia care. Don't be afraid to educate these administrators about your needs and the reasons behind them.

You must determine which functions are most important to you personally. But it's also important to ensure the survival of services that may be essential to others in the community, such as therapies for people with inhibitors or specialized services for new parents.

Quality

Just because a function exists, it's not necessarily adequately fulfilling a community need. The quality of services must be held to a high standard reflecting the serious nature of hemophilia and the consequences of under-treatment. For example, medical personnel assigned to hemophilia cases must be trained and experienced specifically in hemophilia.

Availability

Services and products must be readily available to everyone, regardless of financial means. They must also be available within a reasonable geographic distance. Parents requiring a 24-hour emergency call-in center should not reach a recording or be put on hold.

Protecting the clinical needs of people with hemophilia is paramount. But retaining strong and effective standards in the new model for hemophilia care delivery may not be easy. The hemophilia community's concept of healthcare value may conflict with the PBM industry's definition. A PBM's quest for value reflects its representation of tens of millions of lives–with scant healthcare resources to go around, and with a driving need keep health insurers happy by reporting significant cost savings. Yet as parents and patients in a small community, you place the highest value on the needs of the individual and the desire to live a full life.

How can you avoid losing ground in the currently high standard of hemophilia healthcare? List the aspects of your healthcare that you value most. Read "Hemophilia, Incorporated" and "The Coming Storm." Read Elena Bostick's article about lobbying and advocacy in the February issue of *PEN*, "Maintaining Access to Care and Reimbursement–Our Number One Priority." Find out what your local hemophilia organization is doing about this issue–are they even aware of it? Let your organization know about any changes in your healthcare plan. Be aware of changes being made to your coverage. Call your health insurer or PBM regularly–and send a copy of this article, explaining that you are monitoring changes in your plan because the next change could compromise the standard of care for you or your child.

Healthcare change is happening fast. In a sea of more than 200 million PBM members, the hemophilia community represents only 10,000 individuals. The only way to protect what you value in healthcare is to be organized, focused, consistent and loud.

Michael Russo is a partner at The Bruckner Group (BGI), a strategy and research firm serving the healthcare industry. 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 Michael at mrusso@brucknergroup.com. Learn more about BGI by visiting www.bruckergroup.com.

The Bruckner Group

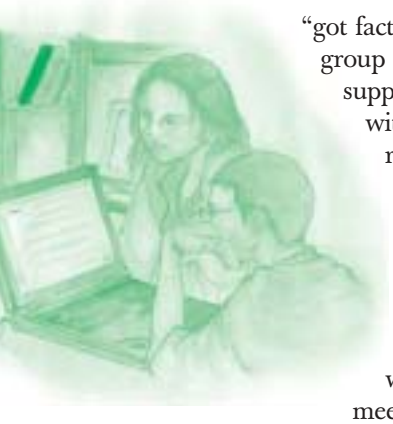
Factor Freedom

"Factor Freedom," Wyeth Pharmaceutical's new patient service, includes The Hemophilia Insurance Coverage Program. This program provides insurance case management and free product when there is a gap or cap in the patient's insurance. To be eligible, you must be on ReFacto® or BeneFIX® for three consecutive months covered by a third party. No coupons are required. Learn about this and other Wyeth programs by signing up for *Lifelines*, a bi-monthly newsletter.

For more information: visit Wyeth's updated website at www.Hemophiliavillage.com or call the Wyeth hotline at 1-888-999-2349

Wyeth lifelines™

New Hemophilia Family Online Support Group



"got factor" is an online support group that provides knowledge, support and friendship to families with hemophilia. It has links to resources and organizations, message boards for general chat, and specialized boards such as "Great Finds!" "Tips and Tricks" and "Venting Room." The site offers information on websites, publications and meetings, tips for infusing and reducing injuries, advice about treatment, ports, bleeds and sports, and an opportunity for you to share your own hemophilia story.

For more information: contact Brenda Milo at bmilo@comcast.net

Editor's note: "got factor?" was created and is maintained by a mother of a child with hemophilia to provide general information. When confronted with a medical decision about your child's health, always consult a qualified physician.

Kogenate® FS Website Launched

Bayer Biological Products (Bayer BP) recently launched a new website at www.KogenateFS.com. The site educates the hemophilia community about Kogenate FS and the many Bayer BP-supported programs and services for the hemophilia community. As part of the launch, Bayer is offering hemophilia patients and their caregivers an opportunity to qualify for a trip to its manufacturing facility in Berkeley, California, to see how Kogenate FS is manufactured.

For entry details and more information: www.KogenateFS.com

American Red Cross Leaves Hemophilia Business

The American Red Cross (ARC) will exit the plasma processing and supply business, selling its recovered plasma to Baxter Healthcare under a new long-term plasma procurement contract effective July 1, 2005. While the two parties have agreed to terminate their plasma manufacturing arrangement, Baxter will continue to make and supply products under the ARC brand names until the company transitions to Baxter-branded versions. Both Baxter and ARC promise no interruption in supply to customers who currently use Red Cross products.

Source: IB/PN, Volume 22, Issue 8, March 2005

Wyeth Grants $3 Million to CDC for Inhibitor Study

Why do some people with hemophilia develop inhibitors? Wyeth Pharmaceuticals has granted the Centers for Disease Control and Prevention (CDC) Foundation $3 million over three years to study this question. "CDC scientists have long wanted to determine how and why certain hemophilia patients develop this inhibitor, but have not had the funds for a comprehensive study," says Charles Stokes, president and CEO of the CDC Foundation. "With the help of Wyeth Pharmaceuticals, CDC can now take the first steps toward ultimately finding a solution to this problem."

For more information: www.cdcfoundation.org/pages.html?page=520

Largest Hemophilia Homecare Purchased

In February 2005 Medco Health Solutions, Inc. announced a definitive agreement to acquire Accredo Health, Incorporated in a cash and stock transaction valued at about $2.2 billion. This deal will pair one of the largest US pharmacy benefit managers (PBMs) with a leading provider of specialty pharmacy services, creating the nation's largest specialty pharmacy business–and largest hemophilia homecare provider–with annual revenues exceeding $4 billion.

Source: www.medco.com and *IB/PN*, Volume 22, Issue 8, March 2005

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For more information: www.marketingresearchbureau.com

Letters... continued from page 2

I found "The Coming Storm" to be well written, easy to understand, timely, and extremely important in mobilizing industry and the consumer community to take action in making our case to insurers. Be assured that Bayer is steadfast in its commitment to support initiatives that help ensure patient choice and access. Kudos for a great article.

Terry Tenbrunsel
Vice President, Bayer HealthCare,
Biological Products Division

Congratulations on a balanced and well-written issue. We are in the thick of the proposed state reform here in Minnesota!

Nigel Key, M.D.
Minnesota

A knockout! Great job.

Richard Lipton, M.D.
Long Island Jewish Hospital
New York

As a homecare company representative, I thought the latest issue of *PEN* was insightful and clear. For families, this article will be a clear and understandable tool. It also lets us all know what is going on in the hemophilia community. When I read "The Coming Storm," I found myself taking notes! *PEN* is a great resource for everyone in the community. I really appreciate your dedication, support, and focus on all individuals.

Jenna Fuka
Regional Hemophilia Resource Manager,
HemophiliaOptions

I thought both "Hemophilia, Incorporated" and "The Coming Storm" were very good. Most impressive was how you stressed that it's imperative for patients to become involved in their own care and really speak out on this issue. I think you were effective in communicating that message.

Laval R. Sans
Northeast Bayer Account Executive

"The Coming Storm" is timely and most needed as it affects all of us within the hemophilia community. For too long, crucial decisions have been made for us that have had a long-lasting impact. It's encouraging to see the hemophilia community pull together to fight for our freedom to make choices—not only for a particular factor product, but for insurance coverage. Thanks for your efforts to keep us informed and updated on crucial issues.

Dorcas Walker
Tennessee

I think there are solutions to the problems faced by all. Average sales price (ASP) is a reality that we are all stuck with. And private insurance has already been following in Medicare's path for years. This community needs to couple with other chronic disease state communities and lobby for realistic lifetime guaranteed coverage. A good place to start would be the 50-odd diseases named as automatic qualifiers for high-risk pools. If these disease states could band together and ask to be included with End Stage Renal Disease to participate in the Medicare insurance program without going on disability, this would be a long-term solution. I have felt for a long time that this is one of the most pressing concerns of this community, but like the rest of the world we are easily distracted by other things.

John Reed
Oklahoma

Insurance coverage is always a concern. I had to change my insurance recently and join a plan that was our only choice, so I am praying that our factor is covered. My son Lee has been doing well, but I have to admit that insurance is a concern for us all, and one that will require having the issues explained to us. Thank you for your hard work.

Eleth Ridenhour
Kansas

In our experience, it's the rare exception when a patient seriously looks at his or her medical benefit/reimbursement situation before a crisis is at the doorstep. Most are unconcerned with Medicare or Medicaid because they don't currently use them; and, unless a particular insurer's changes impact their own policy, they are uninterested. Unless patients have been severely 'burned' before, they usually become aware only after the effects begin to show.

I am unsure how to get the message out. We tried many approaches over the years without finding one that is particularly effective. We need to keep on trying. Thank you for raising awareness about the many facets of bleeding disorder community reality.

Dennis Penning
Clinical Services Coordinator,
Hemophilia Foundation of Illinois

Thanks for tackling such a complicated issue. The hemophilia community needs to understand how the insurance industry is changing the way care is delivered, but the story has many angles. "The Coming Storm" does a superb job of covering the issues in a way people unfamiliar with the industry can understand. Information is empowering!

Kyle Callahan
President, Hemophilia Health Services

Advocacy for patients with hemophilia and their families has always been the cornerstone upon which Hemophilia Federation of America (HFA) was founded. It is the life and breath of our mission and vision statements, and the main focus of our emphasis for 2005. The information provided in *PEN* is a complete primer for the patient on understanding the maze and how to

deal with it. Thank you so much for assimilating all of this very important data for our community and providing it in readable format. Once again, your organization and your publication have hit the nail on the head. What a wonderful tool!

Jan Hamilton
Executive Director, HFA

When I received the *PEN* issue featuring "The Coming Storm," I was mesmerized and read the entire publication from cover to cover. I immediately forwarded a request to your office to have multiple copies sent to my attention at the hotel in Nashville where HFA was having a meeting of our Board of Directors prior to the annual educational symposium. Our organization gives highest priority to advocacy for our constituents, and I believe your publication should be mandatory reading for all advocates.

Barbara Chang
Past President, HFA

The February 2005 issue is very helpful, but it misses the point. It's true that insurers need to limit costs. It's also true that the blood-based pharmaceutical industry must make a profit. This is not the problem. The problem, since approximately 1985, is the predatory pricing practices of the blood-based pharmaceutical industry, directed at people with hemophilia and their families. We need to join with the insurers and government entities to force an investigation of these practices, negotiate with the industry for lower prices and, if necessary, litigate.

A look at a pricing model for the past 20 years would show extreme price increases. A look at an industry cost model would likely show little if any relationship to the price paid for these products. In addition, the pricing increases across the industry do not follow market forces, but instead seem to be part of a concerted and coordinated effort to gouge people with hemophilia and their families as well as the insurers and government benefit plans.

None of this is a function of the market. It is the function of fear, and Kyle Callahan's statements in "The Coming Storm" speak to this. We are now paying a blackmail price for safety and effectiveness. This has been ongoing since the first series of drastic price increases in the mid-1980s related to the change to heat-treated products. People with hemophilia and their families were first conditioned to price increases by the horrific effects of the HIV/AIDS epidemic; this conditioning allowed the industry to exact whatever price it wanted for safe products. People with hemophilia and their families are given a Faustian choice—pay the price and risk community-wide bankruptcy, or don't pay the price and risk life-threatening bleeding and/or a repeat of the epidemic. Safety and effectiveness should always have been in place, but the boogeyman of viral contamination makes us all too willing to accept any price that the industry demands. We can no longer accept this insidious form of blackmail. We must insist that the disconnect between pricing and costs be investigated. We must join hands with those who pay for clotting factor to bring an end to the outrageous pricing scheme we now face.

Peter Hoffman, Esq.
New York

Absolutely wonderful work! Thanks for representing our community and our issues in the terrific way that you do. Thought you'd be interested in our front page for this edition of *The Winning Spirit*.

Ann Rogers
Executive Director, Delaware Valley Chapter of NHF

I really appreciate your time and effort in passing along critical information about the sweeping changes in our community. As a person with severe hemophilia, I take my healthcare options very seriously. As I read about some of the changes that might result in the removal of providers and products, I was reminded of receiving my medication 30 years ago at the hospital ER, my only option at the time. It was a long, drawn-out process each time I needed an infusion, which caused prolonged bleeding and joint damage. That's how I perceive these changes may affect our treatment and care: taking our options back to where they were 30 years ago.

Thanks again for providing our community with such important information, so we can raise our voices to keep the standards we have fought so hard for.

Erik Melde
Utah

Keep up the great work. Everyone is talking about that famous February *PEN*. "The Coming Storm" is a great tool for us in the field to help families understand the issues better.

John Williams
Florida

The last issue of *PEN* was extremely informative, yet disturbing.

Penny Isenbergh
Florida

I really appreciate the overview of concerns currently being addressed by the hemophilia community. As a marketing person dealing with people involved in all sides of the debate, I am thankful for any article that summarizes the issues so well. I especially enjoyed the diversity of opinions from the various sources—manufacturer, insurer, HTC, chapters. Thanks for your efforts.

Art Wood
Vice President, Marketing and Development,
Patient Services Incorporated
Virginia

Thank you for your comprehensive essay on the emerging crisis in hemophilia care reimbursement. The underlying cause of this crisis, which you repeatedly allude to but seem to forgive, is the unsustainable high cost of clotting factor, and the lucrative industry delivering product and services to people with hemophilia.

It is ludicrous to assume that this cat is not out of the bag, and that insurers will not attempt to regulate the disease-specific boutique industries that exist precisely because they are extremely lucrative. Indeed, the profitability of clotting factor sales reflected in 'value-added community philanthropy' pales in comparison to shareholder returns and profit margins that are consistently two- to three-fold higher than that of other major US corporations.

I agree that the hemophilia community in general, and the consumer in particular, should act immediately to ensure the future of the current excellent standard of care now threatened by insurers. However, action that purely defends the status quo as entitlement defies the long-term interests of this community. Instead, we must preempt payer intervention with a proactive self-initiated strategy that critically re-evaluates and reasonably re-defines access to hemophilia therapies and product delivery systems, while maintaining, or even improving, overall standard of care.

Since replacement product represents more than 90% of the cost of hemophilia care, the cornerstone of this activism must be the demand for a major reduction in the cost of clotting factor product to payers of all types. This would significantly help to ensure product choice. Additionally, a reduction in overhead (and, if necessary, the value-added services that inflate this overhead) must be demanded at all levels of product supply and delivery. This action has the potential to conserve choice of distributor based chiefly on patient-specific need, and not on the economics of the payer-distributor relationship. To lobby effectively, the hemophilia community must first convince itself that this effort will not place current and new product availability at risk. This will require a critical look at its exceedingly close relationship with industry, and at the myths perpetuated as a direct result of industry's unencumbered access to the consumer, unparalleled in the world.

Although a sizeable decrease in the final per unit cost of clotting factor will contribute significantly toward reducing the cost of replacement therapy, a critical re-evaluation of the safety and effectiveness of current treatment regimens must also be part of an activist strategy. As emerging data suggests, treating with more product does not necessarily insure better clinical outcomes. Treaters and consumers alike should demand and participate in well-designed clinical research trials that study and document the cost-effectiveness of our very expressive interventions. Payers have begun to demand such data. Consumer indignation and physician opinion will not succeed in replacing scientifically reliable data for much longer.

Your article claims that the future is now. I couldn't agree more. The hemophilia community is at an important crossroad. Only a new paradigm of action will protect access to the current and evolving standard of hemophilia care. Historically, the hemophilia community has fought hard for the standard of care it now enjoys. Nonetheless, in a country that upholds a right to healthcare profit rather than the right to healthcare itself, the battle is far from over.

Donna DiMichele, M.D.
Director, Regional Hemophilia Diagnostic and Treatment Center,
New York Presbyterian Weill Cornell Center
New York

Thank you for the comprehensive update on the hemophilia community and the current status and trends within the product provider and insurance industries.

It will be important for our community to not reject these new changes out-of-hand, but carefully consider all impacts,

consequences and costs. Asking us to try an alternate product is not unreasonable if the product is equally effective. Demanding the most advanced products when obtaining supplies through no-cost government programs may be unreasonable. We will need to pick our fights carefully, and not appear to be willing only to maintain the status quo.

We need to keep in mind that our community receives a generous monetary contribution toward our treatments from those outside the hemophilia community. If we appear to be inflexible, obstructionist or spendthrift (wasting the money that we receive from them), we risk increased opposition, funding cutbacks, or more drastic proposals to save money that may infringe on our rights or healthcare choices.

Name withheld

"Hemophilia Incorporated" Makes an Impact

(*PEN*, November 2005)

PEN is making an impact. I was present when a nurse at an HTC told her team that she was very impressed with "Hemophilia, Incorporated." A few days later, I got a copy of a letter from one of our patients who was writing to a patient relations staff member at a hospital where her son was treated. According to her letter, she was charged $7.10 per unit for a recombinant product, at a total cost of $24,000. She quoted the prices you outlined in your article as proof of overcharging.

Debbie de la Riva
Executive Director, Lone Star Chapter of NHF

I am the mother of a three-year-old son with severe hemophilia A. I want to congratulate you on "Hemophilia, Incorporated." Since the birth of my son, I have been looking for a breakdown of how the homecare company/manufacturer system works. This article couldn't have been better! It is straight to the point and very informative. I wish I had seen this when Colton was born. I had no idea what I was getting into, and felt that I would never understand the big picture. I can't wait to read the next part of the series. Keep up the good work.

Randi Clites
Ohio

The November issue of *PEN* was the first I have read, and I was very impressed. You bring up many important issues about the business behind the treatment of hemophilia. I was not aware of much of this information until very recently because I was too busy learning about hemophilia. Understanding the issues that underlie hemophilia treatment—factor sales, homecare companies, insurance companies and the deals that are made among them—is nearly as important for patients, families and doctors as understanding and treating the disease itself. We need to work together to ensure ongoing proper care for people with hemophilia. I look forward to future issues of *PEN*.

Carolyn M. Bennett, M.D.
Division of Hematology, Children's Hospital Boston

courtesy of the Kourtakis family

Bobby Kourtakis:
His memory inspires brother Tony in Iraq.

I am so pleased to read in *PEN* that younger parents are getting empowered. I have mild hemophilia and my older son Bobby had severe hemophilia. He was a bright, gifted sportswriter and adored his younger brother Tony with all his heart. It was an honor to have Bobby for a son and he is always with us. My younger son Tony is a Marine, just home from Iraq, and he cares so much about the hemophilia community. I lost Bobby in 1993 from contaminated blood products. I contracted hepatitis C myself. We need to do everything possible to protect our family's future. God bless you for your work.

Roberta Kourtakis
Michigan

Thank you for the important and thought-provoking work you are doing on behalf of families with hemophilia. I always learn a lot from *PEN*.

Bob Massie
Massachusetts

Our six-month-old son has been diagnosed with severe hemophilia A. It was a spontaneous mutation, unexpected and difficult to accept at first. Our family has been through a lot already as a result of our son's birth experience, including blood transfusions and several bleeding episodes. As we tried to understand and cope with all the issues surrounding hemophilia, we couldn't find many places toturn for practical family support; there are few local support groups. I would love to talk to other moms (and dads!) who are dealing with this and share tips, experience and knowledge.

Brenda Milo
Illinois

Editor's note: See News Notes, page 14, for more on Brenda's efforts.

A Mother's Plea

I have a 17-year-old old son who has been struggling with depression. We saw a positive change when he used Prozac. Unfortunately, a recent article in a hemophilia magazine mentioned that antidepressants can cause abnormal bleeding. I noticed that my son was experiencing more bleeds during the month he used Prozac. His pediatrician immediately discontinued the drug. It's been about a month now, and my son has been experiencing all the classic signs of depression once again, and wants help. I am currently waiting for a psychiatrist to see him and prescribe another medication that might not interfere with his hemophilia. Is anyone else going through this with their child? Any suggestions for me about antidepressants and hemophilia?

Chris (last name withheld)
Ohio

Project SHARE℠ Saves Lives

I acknowledge the receipt of 15 vials of factor received at the Iduapriem Mine. I wish to thank Project SHARE so much for offering us this great assistance. We are very grateful for your readiness to assist once we approached you. We hope that you will continue to assist us when the need arises. Once again we thank you very much. Little Kojo Opoku is very elated, and much grateful for your assistance to him.

Dr. Ishmael Ato Yamoah
Ghana

Editor's note: See A Project SHARE Story, page 4, for more on Kojo.

Thank you very much for the medication you sent us. It came when we were without any. Lawrence's life was actually saved by the boxes of factor VIII you sent. He was bleeding in the head and we had nothing to give him. He is now much better although he has lost his speech, but he is playing with other children. Project SHARE, you actually saved the boy's life.

Tachiona Svunurai
Zimbabwe Haemophilia Association

PROJECT SHARE
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