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Parent Empowerment Newsletter

Your 2007 Insurance Primer

BY LAURIE KELLEY

We're fortunate to live in a country that produces most of the world's factor, and some of the world's highest quality factor. Factor in the US is both safe and abundant. But factor is also one of the most expensive medicines. It is essential that you have some form of insurance to pay for your factor concentrate and healthcare services. To be a responsible parent and advocate for your child with hemophilia, you will need to learn how to secure insurance, make sure it covers hemophilia care, and protect your coverage from changes that threaten to limit or eliminate hemophilia care.

Right now, healthcare insurance is undergoing massive reform in the US. Payers–the private insurance companies or state and federal programs that pay for our factor and hemophilia healthcare–are gaining greater control over our treatment choices. In their efforts to cut escalating costs, payers may limit your choice of factor product, factor provider, and even treatment regimen. To preserve access and choice, your first line of defense is to learn all you can about your personal insurance policy.[1]

Understanding the complexities of insurance may seem like an extra burden–especially when you're trying to deal with the diagnosis, learn about hemophilia, and cope with substantial life changes. But without adequate healthcare coverage, your stress will be greatly amplified. As much as you need to know about detecting bleeds, infusing and treatment options, you also need to know:

- who pays for your hemophilia care;
- how insurance reforms could affect your choices;
- what kind of policy you have;
- what your policy covers and does not cover;
- how to manage costs.

Who Pays For Insurance?

The hemophilia marketplace features various players: factor manufacturers, HTCs, home care companies, and the most important player–you, the consumer. Insurance carriers are also key players. They pay for the factor and healthcare services we use. In a previous issue,[2] *PEN* explained how the hemophilia business works: Factor concentrates are made at a manufacturing facility, sold to a distributor, and then delivered to you, the parent or patient. The distributor invoices the total cost of the factor to your payer, and is eventually "reimbursed" by your payer for each shipment or "sale."

In the US, health insurance is typically a commercial industry.[3] Health insurance is available from private insurance companies like BlueCross® BlueShield®, Cigna and Aetna. These companies sell

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[1] The information contained in this article is a general overview only. There are too many variations in healthcare coverage issues to explain all scenarios in detail. This information is a starting point, offering insights and increasing awareness of common insurance-related issues.

[2] Laureen A. Kelley, "Hemophilia, Incorporated." *PEN*, November 2004, Volume 14 Issue 4.

[3] The rising cost of healthcare in America is truly stunning: Healthcare costs represent 16% of total US economic output, or about $2 trillion annually. In 2004, costs rose at three times the rate of inflation, according to the National Coalition on Health Care. Today we spend more on healthcare products and services than on any other sector. www.nchc.org/facts/cost.shtml

inside PEN

welcome

Sherrell Portrait Design

The new year debuts with the most shocking of predictions coming true–a hostile takeover attempt of Caremark, one of the country's largest providers of clotting factor (see Storm Watch, page 6). I didn't foresee the future in quite *that* level of detail, but I have acted as town crier for the past two years, trying to waken this sleepy bleeding disorders community to threats to our freedom of choice and level of healthcare. Here we go again… What next? And are you ready?

If you don't feel ready, make a new year's resolution to get ready. And I'll make a new year's resolution to stop saying "the coming storm," and instead say "the current storm," because the storm is already here. The factor provider marketplace is changing month by month, and certainly will impact you eventually. Oh, the implications are too vast to list here, so here's what to do to get ready:

Read this issue *carefully* several times. Get motivated to understand your own insurance policy in detail.

Read our archived issues, especially November 2004, February 2005, May 2005 and August 2006. Luckily, these are all available through our website and you can print them directly at www.kelleycom.com.

Get *Storm Log*, your personal insurance guide–and I mean personal! Go to www.hemophiliagalaxy.com.

Contact your local hemophilia organization. Register, and ask what's happening in your state. Don't let me visit your state and learn that I know more about what's happening there than you do.

Contact the National Hemophilia Foundation. For goodness' sake, at least get on the mailing list for *HemAware*. It's free! Call 800-42-HANDI.

Share this issue of *PEN* with any new parent or patient who does not subscribe. There's no excuse for being uninformed about insurance changes!

Contact your home care company and find out if your service is at risk as a result of all the new mergers.

And when you need a relaxing break, read Ziva Mann's new column, "Homefront." Ziva is a Massachusetts mom (like me, only younger and funnier) who has a flair for writing, and an active life raising a young son with hemophilia. Her column debuts with this issue, and I know you'll enjoy her reflections on childrearing as much as I do. I "predict" you will learn a lot and laugh a lot. And so far, my predictions have been pretty darn good.

PARENT EMPOWERMENT NEWSLETTER FEBRUARY 2007

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letters

PROJECT SHARE

We were able to free from customs the shipment of factor you sent. We pleaded our case successfully and were able to gain quite a lot of sympathy from the Pakistani government agencies! Thanks very much for an extremely prompt response to our request for help. We are all grateful for the efforts of your team, which made it possible for us to have these factor concentrates.

Dr. Tahira Zafar
Pakistan Hemophilia Patients Welfare Society
Islamabad, Pakistan

I work in Cambodia as a volunteer, and we have many hemophilia patients who do not have access to factor concentrates. I am overwhelmed with joy to find your organization, as I have just implemented hemophilia diagnosis into Cambodia, and now we have found a way to treat them. All the families we serve are very poor farmers. One poor mother has two sons with hemophilia. It is like a miracle for us. A million thank-yous and blessings to you.

Robyn Devenish, Laboratory Technical Adviser
National Institute of Public Health
Phnom Penh, Cambodia

Words cannot express our feeling of gratitude for Project SHARE's generosity. You have practically given our son a new lease on life. The factor VIII was delivered to Cornwall

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BY RICHARD J. ATWOOD, MA, MPH

REMEMBERING AMERICA'S *First Hemophilia Author*

Why would anyone skip part of the NHF annual meeting in Philadelphia to visit a cemetery–on Friday the 13th? My compelling reason was the gravesite of Dr. John C. Otto. In 1803, Dr. Otto published the first summary of hemophilia in *Medical Repository*, America's first medical journal. On this early October morning, I drove across the Schuylkill River to Woodlands Cemetery to visit his gravesite out of respect, not mourning.

Born in 1774, Dr. Otto was the son of an eminent New Jersey physician and attended medical school at the University of Pennsylvania. He was elected to the medical staff of Pennsylvania Hospital in 1813, serving there until his retirement in 1835. Oddly, an inaccurate date of death is chiseled into the marble of his tombstone: Otto died in 1844, not 1884.

All subsequent medical journal articles on hemophilia can be traced to Otto's pioneering article, "An Account of an Hemorrhagic Disposition existing in certain Families." In this sense, Otto sparked a scientific revolution by motivating others to write articles for medical journals. His article has been reprinted several times in various journals, most recently in 1996.

Otto recognized that people with hemophilia have been a part of US history since our country was founded. The idea of sharing medical information about American hemophilia patients in our own medical journals was an important step in the independence of American medicine. Not only was Otto the first to describe hemophilia in this country, but he was also precise. He accurately depicted not only the bleeding manifestations of individual patients, but also the genetic inheritance pattern over several generations of "transmitters," now called carriers.

In his writing, Otto used the word "bleeders,"[1] a common term that has remained in use for more than 200 years. Significantly, Otto advocated for medical treatment of hemophilia. He recommended sulphate of soda as a "purging" treatment for bleeding episodes–a treatment that was mainly cathartic. From many citations in the medical literature, we know that sulphate of soda gained acceptance in the 1700s, and lasted until about 1900.

I had visited Dr. Otto's gravesite on previous trips to Philadelphia, noting that over time, a coat of green slime was growing. As no one was maintaining the tombstone, I vowed to do something. So on this visit, I arrived early in the morning with a bucket, brush, and can of household cleaner, and I scrubbed the tombstone clean. The few joggers on the trail between the gravestones didn't notice me; nor did a silent herd of seven deer, who provided an unexpectedly pastoral scene. I was a solitary visitor this Friday, paying homage to John C. Otto, MD, a distinguished figure in the history of hemophilia.

Richard Atwood, MA, MPH, lives in Winston-Salem, North Carolina, where he worked for 17 years at the Hemophilia Treatment Center at Wake Forest University. Currently he serves on the Board of Hemophilia of North Carolina.

[1] At this time, the term *bluters* was used to describe people with bleeding disorders in Germany. The term may have been brought to America by German immigrants.

BY PAUL CLEMENT

HEMOSTASIS

Or everything you wanted to know about clotting

The human body is incredible. It's composed of hundreds of trillions of cells, all organized into tissues, organs and organ systems. Trillions of chemical reactions occur every minute, and hundreds of complex feedback loops monitor and control every process. Each cell performs its functions, and all of these chemical reactions occur with no conscious input from us. Among the many complex processes of the body is blood clotting–a system so complex that even the smallest error can result in a disorder like hemophilia, and in complications like inhibitors.

Blood clotting is known as *hemostasis*. "Hemo" comes from the Greek root meaning "blood," and "stasis" comes from the Greek word "stop." So hemostasis literally means to stop blood, as in stopping a bleed. But hemostasis means not only *coagulation*–the formation of a blood clot to stop bleeding–but also *fibrinolysis*–the breakdown of blood clots so healing can occur. Hemostasis involves a delicate balance: forming clots to plug holes in blood vessels so we don't bleed to death; preventing unwanted clots; and dissolving clots so the blood can continue to flow through blood vessels unobstructed. Upsetting this fine balance in hemostasis may result in uncontrolled bleeding or blood clots in the lungs, heart, brain or other organs–possibly causing death. To compound matters, when we consciously try to correct problems in hemostasis, for example by infusing factor, the body sometimes tries to thwart our attempts by producing antibodies called inhibitors, which fight against clotting factors.

Let's take a closer look at how hemostasis works, with an emphasis on how we attempt to compensate for failures in the system by infusing the missing clotting factors so clotting can still occur.

How Blood Clots

Primary hemostasis involves steps 1 and 2: vasoconstriction and platelet aggregation. **Secondary** hemostasis involves step 3: the formation of a fibrin clot through a process known as the clotting cascade.

1. Vasoconstriction: the blood vessel tightens.

2. Platelet aggregation: platelets stick to the injury site.

3. Fibrin clot: 13 clotting factors work together in a clotting cascade.

Primary Hemostasis: First line of defense against bleeds

Coagulation normally begins with a process called *primary hemostasis*. Here's how it works: First, *vasoconstriction* occurs. This means that when a blood vessel is injured and bleeds, the muscles in the blood vessel tighten and constrict, reducing

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PROJECT SHARE
It's time to give back.

BY JULIA QUIGLEY LONG

Cucumbers to Factor: Appreciating Rare Gifts

What do birthday gifts and cucumbers have in common? Quite a lot, if you are Dilmurod Poshohodjaev from Uzbekistan. As a child living with hemophilia in the rural village of Chust, these things were scarce or simply unavailable to Dilmurod.

So many modern conveniences were just dreams for "Dil," now 26, and his three older sisters, Surayo, Saodat and Saida. "Our parents never had a cake or gifts for our birthdays," recalls Saida, the second youngest, "and it was okay with us because we could see they hardly had enough money for food and clothes." What did the siblings cherish most? "We loved fresh cucumbers and apples like American kids love chocolates and ice cream."

When Dil was growing up, the family lived on a large plot of land with a cow, sheep and chickens. They had no hot water, and their bathroom was an outhouse—an unpleasant walk on a wintry night. His parents, both teachers, couldn't afford a car with their combined income of $20 a month. From Monday to Saturday, the children woke early to walk three miles to school, even when a foot of snow blanketed the ground, because there were no school buses. On Sunday, while his sisters did labor-intensive, all-day chores like laundry, Dil rarely extended himself more than to walk to the grocery store to buy bread because his mother worried that he would hurt himself.

His mother's worry was reasonable: Dil was diagnosed with hemophilia A at age two. When he bled severely, his parents took him on the train to the capital, Tashkent, the site of the only hospital equipped to treat people with hemophilia. Arranging time away from work and buying the ticket took "a week or two," explains Dil, and the trip recurred every three to six months. "Those two weeks were a nightmare for me," he remembers. "I couldn't tolerate the pain and stayed in bed crying and sobbing. My mother and sisters would try their best to help me, to distract me from the pain. I would laugh at their jokes but continue crying after the entertainment was over."

Dil with his sisters, Surayo, Saodat and Saida, and mother (second from left).

In early 2006, 29-year-old Saida, who now lives in Ohio, used the Internet to research hemophilia information for Dil. When Saida first told him about American summer camps for young people with hemophilia, says Dil, "It was amazing! And I learned that there are special books written for children with hemophilia, and books for their parents. I wish we had had such great opportunities, great treatment facilities and enough medicines."

In late June, a tooth extraction left Dil bleeding for eight days. The hospital in Tashkent had no plasma, and cryoprecipitate wasn't able to stop his bleeding. Saida grew nervous and sought advice from the hematology department at Cincinnati Children's Hospital, where she first learned about Project SHARE. She submitted a request for factor, and on July 7, 2006, the first Project SHARE factor shipment to Uzbekistan was sent to Dil's doctor. The little boy who never received candy or presents at last received his first infusion of factor concentrate—at age 26.

Next time you spear that boring old cucumber in your salad, or curse the school bus that slows your morning commute; next time you see the glowing candles on yet another birthday cake, perhaps you'll remember Dil and his sisters, and appreciate these common things for the truly rare gifts they are.

To make a donation of factor or funding to Project SHARE, contact Julia Long, director, at julia@kelleycom.com or call (800) 249-7977.

BY LAURIE KELLEY

Monstrous Acquisition of Factor Providers Looming

A year ago, *PEN* predicted that specialty pharmacies would continue to merge as the home care industry faced unprecedented change while battling the "coming storm" in the insurance industry. Cost-cutting methods by payers are forcing the industry to rethink how it does business; and mergers provide ways to gain power, market share and possibly lower prices.

We've seen a tremendous number of mergers and acquisitions, but nothing on the scale of what is happening now. Express Scripts, one of the nation's top pharmacy benefit managers (PBMs), has launched a hostile bid for Caremark, one of the largest factor providers, and also a PBM. The surprise $26 billion bid came in December.

In "Taking Center Stage" (May 2005), *PEN* explained that PBMs are business middlemen hired by employers and insurers to negotiate better prices from drug manufacturers and retailers. They encourage employees to use lower-priced generic drugs and less expensive mail-order pharmacies. PBMs are hired to save employers money by managing healthcare contracts and costs. But in the current wave of mergers, PBMs are emerging with great power, and can exert strong influence over pricing, especially for biological products like factor.

The *Los Angeles Times* predicts that an Express Scripts/ Caremark combined company will control about 30% of the total PBM market, with $49 billion a year in revenue. That's compared to its next biggest rival, Medco (which owns Hemophilia Health Services), with $38 billion a year in revenue. The *LA Times* also notes that not everyone is confident that more consolidation is good. There's no guarantee that this merger will eventually pass along savings to employers and employees, including those with hemophilia. It's true that the merged company will be the most powerful in factor distribution. Smaller, independent factor providers–a traditional and important part of the bleeding disorders community–face troubled times ahead as they play David to the industries' newly-created Goliaths.

For more information, see "Express Scripts tops CVS in hostile bid for Caremark Rx" by Daniel Costello, *Los Angeles Times*, December 19, 2006. Also visit the archived article at www.kelleycom.com/archives.html.

Ed. Note: On January 8, Bloomburg.com reported that Caremark rejected the Express Scripts bid. Caremark was quoted as saying the deal "would result in a highly leveraged and weakened business"and "insurmountable antitrust risks." Watch the newswires for more on this hostile takeover attempt.

Insights... continued from page 4

blood flow and thus blood loss. The break in the vessel wall exposes *collagen fibers*. Von Willebrand factor (VWF) sticks to the collagen in the vessel wall, and also to platelets, which are circulating in the blood. This is called *platelet adhesion*. Once they're stuck to the VWF, the platelets become "activated" and release chemicals that cause additional platelets to clump at the site of the injury. This *platelet aggregation* results in the formation of a *platelet plug*. A platelet plug may temporarily stop the flow of blood; but by itself, it's weak and easily dislodged or broken, causing bleeding to recur. To form a successful blood clot that effectively seals off the hole in the blood vessel, the platelet plug must be reinforced with many interwoven *fibrin fibers*, forming a strong *fibrin clot*. This is the primary job of clotting factors and the clotting cascade.

Secondary Hemostasis: Forming the fibrin clot

The formation of fibrin fibers and a strong fibrin clot is called *secondary hemostasis*, the next step in coagulation. Fibrin fibers are produced through a series of chemical reactions called the *clotting cascade*. The clotting cascade involves 13 *clotting factors*, represented by Roman numerals, and a half-dozen other compounds and ions. The clotting cascade is sometimes compared to a line of standing dominoes: If one domino is knocked down, it will knock down the next domino in the line, and so on, until the whole string of dominoes has fallen. If one domino is removed from the line, the dominos will fall until they reach the gap, and the dominos beyond the gap will remain standing. Similarly, when a clotting factor in the clotting cascade is activated, it in turn activates the next clotting factor, which then activates the next one. The reaction concludes with the formation of fibrin fibers interwoven in the platelet plug, or the fibrin clot. Some clotting factors are critical for the formation of fibrin fibers, and the absence of one of these factors is like a domino missing from the line–the clotting cascade stops where the factor is missing.

The process of secondary hemostasis and the formation of a fibrin clot begins with the part of the clotting cascade called the *extrinsic pathway*. Factor III, also known as *tissue factor* (TF), which has been exposed by the injured blood vessel, helps activate factor VII. In turn, factor VII activates factor X and, after some additional reactions, this can lead to the formation of a net of cross-linked fibrin fibers within the platelet plug. You might wonder: If TF and factor VIIa (the letter "a" means "activated") can produce fibrin fibers without factors VIII and IX, why do deficiencies of factors VIII and IX cause bleeding problems? The answer: Almost as soon as the TF-factor VIIa complex starts activating factor X, it's shut down by a plasma protein called *tissue factor pathway inhibitor* (TFPI), which binds to the TF-factor VIIa complex, preventing further factor Xa formation.

Although the extrinsic pathway is rapidly shut down by TFPI, a compound called *thrombin* (factor IIa), which is produced by this reaction, kicks in a second clotting cascade pathway. This second pathway is called the *intrinsic pathway*. Thrombin accelerates the activation of factor XI and factor VIII in the intrinsic pathway, which was slowly gearing up

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BY ZIVA MANN

Ziva Mann joins *PEN* to write "Homefront," a regular column about day-to-day life with hemophilia. Ziva earned her MA in medieval literature from Harvard University. She lives in Massachusetts with her husband, Ezra, and sons Shai, age five, who has severe hemophilia A, and Akiva, age one.

Puzzling Our Way Through

I like to imagine my son, Shai, as a puzzle. He is made up of interlocking parts: One piece is the bit that likes baseball; another piece likes to play card games. It's a nice, tidy image, and it's absolutely wrong. Real life is messier than puzzles. Real pieces of people slide around, change, overlap. You can't label the pieces of a person and put them in a box. Still, the puzzle is an image I revisit when I try to think about hemophilia, because sometimes hemophilia is the simplest piece in the shifting, interlocking puzzle that is my son. A bleed? Fine, we'll infuse, rest, follow up and do physiotherapy. A bully in Shai's class? I spent months on that one, wading through a maze of school politics and small persons' emotions.

Most of the time, hemophilia really is simple. It fades into the background… until something unexpected happens. Then, hemophilia runs the show. I'm talking to hematologists instead of making dinner–trying to be precise, aware of how much they rely are on my ability to observe and analyze symptoms. Sometimes I just get it wrong, and I can't untangle the mundane from the medical. Sometimes I almost *want* to get it wrong. Why? Because I want someone or something to help me fix it. Because sometimes, the piece of me that accepts normalcy is covered by the piece of me that worries.

One Sunday, Shai was trying to reach a toy near the top of a bookcase. The bookcase rocked, dumping a heavy clock on his head. He howled in pain and fury, and I came running. We calculated how much factor he had in his system and gave him some more, but decided not to go to the ER. This was, I was told by the HTC when I phoned, a "borderline case." We took a deep breath, and as our nurse-practitioner says, learned to "sit with the anxiety a bit." Sit? Ha. For me, this mostly means pacing, chewing my fingernails and muttering under my breath.

Monday morning we all went on a hike, strolling past pine trees and rhododendrons while Akiva gurgled to himself in the backpack. At lunch, we were debating the merits of raspberry picking when Shai exploded. *He* didn't want to go raspberry picking. Furious, biting words came spewing out of his five-year-old mouth. Astonished, I waited for his head to start revolving on his shoulders. Did the kid need an exorcist? A hematologist? Does he just hate raspberries that much?

Tuesday morning, I woke up to more furious shrieking. Was Shai's outburst the behavioral symptom of a head bleed, or was the kid trying out tantrums? "I just don't know what it is," said my husband. "You'd better call it in."

I spent the rest of the day at the ER, repeating my husband's words to doctors and nurses: "I just don't know what it is," I told them apologetically. "It might just be normal kid stuff…" I waited for someone to tell me, "Honey, this is a tantrum. You've heard of them, maybe? Need a book on parenting, perhaps?" But since everyone took me seriously, I started to feel less silly and more worried. One CT scan later, I was really worried, and I went back to the pace, chew, mutter routine. Is this CT scan just to make me feel better? What did I say that catapulted all of them into action? Couldn't they just give the crazy hemo-mommy a Valium instead?

Eventually, the word came back. The head CT scan was clear. "Good news!" chirped the doctor. "Yes," said my husband slowly, turning to me, "good news." Shai, happily drawing pictures for the ER staff, grinned at us. We stared at him for a while, wondering what kind of factor you give to cure tantrums. No such luck. We were deep into standard parenting territory, and without a medical map. Clearly, we'd have to untangle the pieces of this puzzle on our own.

How…irritatingly normal.

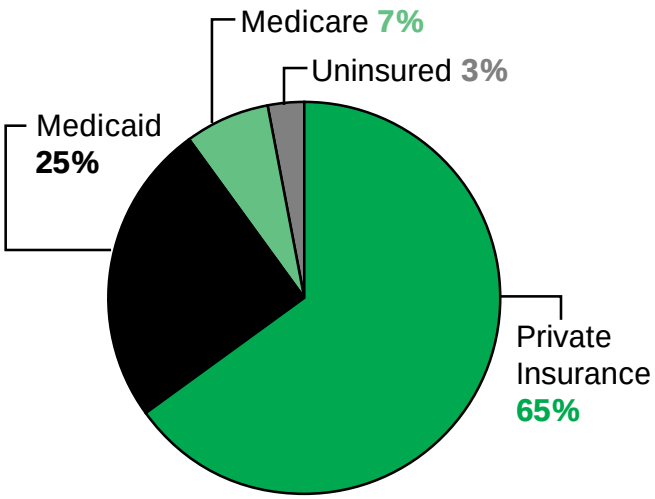
Your 2007 Insurance Primer... continued from cover

policies to employers, who then cover their employees' medical expenses. Policies are also sold to individuals. Group and individual policies cover roughly 65% of Americans with hemophilia.

Public programs also exist to help people with their healthcare coverage. Medicaid helps low-income people, and Medicare is for people age 65 and older, as well as people with certain chronic conditions. Of Americans with hemophilia, approximately 25% use Medicaid, and approximately 7% use Medicare.

To get a handle on your personal insurance situation, you'll need to determine whether you are covered by private insurance, Medicaid or Medicare.

Who Pays For Factor?



Source: Baxter Healthcare, Reimbursement Department

Hemophilia High on Insurance Radar

Payers are becoming more prominent and powerful in the hemophilia marketplace. Why? Hemophilia is one of the most expensive of all chronic disorders, and payers are now scrutinizing all healthcare costs, particularly high-cost treatments. Factor concentrate is a biological product–an injectible. It's fragile, and it must be carefully manufactured, transported, stored and distributed. Each lot number is tracked from manufacturing plant to patient. When recombinant factor was introduced in 1992, payers didn't balk at its higher price tag, as this cutting-edge product was revolutionary and spent years in development. Payers *expected* to pay more for a better, safer product.

But as more and more biological products, for many chronic disorders, were released into the marketplace–from 10 in 1992 to more than 120 now, and with dozens more on the way–payers' budgets were stretched to the limit. While total costs for all biological products have risen at a shocking rate, budgets have not kept up with rising costs, especially for chronic disorders. Law requires state budgets to be balanced, and Medicaid alone consumes 20% on average of all state budgets. As more advanced factor products entered the market, and more patients started prophylaxis, hemophilia costs began to crush state Medicaid budgets. Private employers begged insurance companies to negotiate with manufacturers for lower prices. Payers responded. But new products continued rolling out, faster than payers could negotiate lower prices. And since 2004, payers have said, "Enough is enough. Costs must be cut."

While everyone acknowledges the need to reduce healthcare costs, cost cutting could adversely affect your child's treatment. Why? Unfortunately, payers sometimes don't know enough about hemophilia when they make budget-cutting decisions. So it may be up to *you* to educate them! You can best educate them when you know their language, and understand your coverage, so you can challenge payer policy changes that might adversely impact you.

What Kind of Insurance Do You Have?

Once you've determined whether you have private or public insurance, you need to know the type of insurance you use: traditional or nontraditional. Most insurance policies can be classified as traditional *indemnity plans* or nontraditional *managed care* plans.

Traditional Indemnity Plans

Approximately 10% of hemophilia patients with private insurance use traditional indemnity plans, also called Fee-for-Service (FFS). These plans generally allow more freedom and choice than nontraditional plans. Some typical features and benefits of a traditional FFS plan:

- You can choose your own doctor.
- You don't need a referral from your primary care physician (PCP) to see a specialist.
- You may have a *deductible* (annual amount you must pay before your plan begins to pay).
- You may have a *copayment* (amount you must pay per doctor visit, procedure or prescription).
- You may need to fill out claim forms for each visit.
- You may have a *lifetime maximum* (limit or "cap" on the amount of money paid out on your behalf over your lifetime).

Different FFS plans have different expenses. To estimate your annual out-of-pocket expenses per year, you need to ask about each portion of your policy. For example, you will pay a portion of your medical costs (the deductible, typically 20%) out-of-pocket, up to a certain maximum per year, before the plan starts paying 100% of the allowable charge. The allowable charge is the amount the insurance company allows for a doctor visit or procedure, and is usually based on a community standard. Your annual deductible could be as low as $100 or as high as $1,500. This could be per person or per family.

Your FFS plan may also have a lifetime maximum (cap) that limits the total amount of money the plan pays on your behalf. Drugs like factor concentrate are expensive and could quickly deplete your lifetime maximum. Some plans offer separate prescription drug coverage, so prescription charges don't apply to the lifetime maximum; that's best for factor concentrate coverage. If you have a choice, select a plan with prescription coverage *separate* from the cap.

You may have a lot of paperwork with FFS plans. Usually, you must fill in a claim form for each medical treatment or doctor's visit, pay for the visit or treatment, and then be reimbursed by the insurance company. It's vital to keep accurate records.

Nontraditional Managed Care Plans

An estimated 60% of American employees use some kind of nontraditional plan, also called a managed care plan.[4] There's a good chance that your child with hemophilia will be insured through a managed care organization (MCO). MCOs typically allow less freedom of choice than FFS plans allow. In general, MCOs attempt to contain rising healthcare costs by managing your use of medical services. For example, they may require you to use doctors, hospitals or pharmacies approved by the insurance company as part of its network. MCOs are used by both private and public insurers.

In MCOs, the primary care physician is often called the "gatekeeper." The PCP controls access to specialists and decides which diagnostic tests are necessary. Some MCOs pay PCPs a flat amount per patient per month to manage the healthcare needs of a group of patients—a process known as "capitation." Here's how it works: The group of patients to be managed can range from a few hundred to a few thousand people. If a patient needs to see a specialist, the cost of the specialist visit must come from the amount of money the PCP receives every month to treat that particular patient. Critics of capitation say that this payment system allows money to affect the doctor-patient relationship, creating an ethical dilemma for doctors. But you need specialists: Many PCPs are general practitioners, and their knowledge of hemophilia may be limited. Diagnosing and treating a bleeding disorder may be beyond their scope of expertise. Make sure your PCP is a team player willing to work with the HTC. Your child *must* be treated by a hematologist.

If you have a managed care plan, you will have one of three basic types:

- **Health Maintenance Organization (HMO):** Prepaid plan in which the healthcare provider is also the payer. You or your employer pay a monthly or quarterly fee, which often insures you for 80% to 100% of all medical costs, including hospitalization. You may also be charged an additional amount, or copayment, for certain services, such as office visits, outpatient surgery and prescriptions. You are required to use HMO network doctors, who can refer you to in-network specialists, such as a pediatric hematologist. If your PCP's knowledge of hemophilia is limited, you may need to assert yourself to get to a hemophilia specialist. You must use HMO-approved hospitals, which may or may not include your HTC. Benefits? You shouldn't have to process any bills because they are sent to the HMO. Out-of-pocket costs are low with an HMO. Drawbacks? Limited choice of medical services. And if you need to go out-of-network, expect to pay a high price.
- **Preferred Provider Organization (PPO) or Exclusive Provider Organization (EPO):** More choice, better out-of-network benefits, and fewer restrictions than HMOs. These plans offer reduced copayments and lower deductibles when you use in-network doctors and hospitals. Going out-of-network is allowed but may mean higher copayments. An EPO may not cover services outside its plan. In both PPO and EPO plans, prior authorization is usually required for hospitalization and most outpatient procedures, and for expensive medicines like factor concentrate.
- **Point-of-Service (POS):** One of the most rapidly expanding forms of managed care in the marketplace today, offering flexible options. Consumers can use in-network healthcare providers at a reduced cost (HMO option), or out-of-network providers (traditional indemnity options). However, increased freedom to choose providers out-of-network brings higher monthly premiums and higher copayments. Some employers simply add a POS plan to an already existing managed care plan; employees can choose the existing plan or POS plan. Some employers allow employees to sign up for the POS option and pay extra only if the POS plan is actually used; this is called a *dual option* program because the employee always has two options.

Which Services Are Covered by Your Plan?

Whether you have a traditional or nontraditional plan, you need to know exactly which services or products are covered, or budgeted, under your current policy. The insurance company should explain your coverage in writing, in either a summary of benefits document or a letter.[5] *Never assume that coverage exists for any aspect of hemophilia.*

A health insurance plan has two parts, representing two different budgets: 1) the medical benefit, which covers all clinical services, such as doctor visits, diagnostic tests and surgery; and 2) the pharmacy benefit, which covers outpatient drugs. Either budget can have a lifetime cap, sometimes $1 million, or if you're lucky, even more. When you order factor, its expense is applied to your lifetime maximum. For most people with hemophilia, factor is covered under the medical benefit (also called "major medical"), which usually has a lifetime maximum. You'll have to monitor factor usage carefully.

When factor is covered under the pharmacy benefit, be aware of copayment amounts. These out-of-pocket maximums, typically $3,000 to $5,000 annually, must be paid before prescription drugs are covered at 100%. Regardless of whether your plan covers factor under the medical or pharmaceutical benefit, if you have no lifetime maximum, consider yourself fortunate!

Be sure that factor is covered. Your plan may use a drug *formulary*. A formulary is a list of prescription drugs that are approved by an insurance plan for its members. If you use a drug that is not in the formulary, it may not be covered under your plan. Before you enroll in a new plan, find out if the plan uses a formulary, and if factor concentrate and all the medicines and supplies that you need are on it.

[4] MCOL. Managed care fact sheets. Available at http://www.mcareol.com. Accessed March 14, 2006. [5] Insurance company websites sometimes contain thorough information about the plans they carry, including detailed information on which medicines are included in the drug formulary.

Regardless of which plan you use, you need to know what's covered under your current policy, and under which budget–medical or pharmacy. Ask these questions to find out what your plan covers:

1. Is factor covered? Payers may or may not cover factor concentrate as a drug. If it isn't considered a drug, factor may be covered under the patient's major medical side of the policy. Know whether your major medical has a lifetime maximum, and the amount of that maximum.

2. Is there a copayment for prescriptions? This can get expensive when you are ordering factor.

3. Are there brand restrictions? Which brand of factor did you and your physician choose, and is it covered? Does the plan have a formulary and a preferred drug list (PDL),[6] and is your brand on it or excluded?

4. How is factor delivered? By hospital outpatient pharmacy, 340B program, specialty pharmacy, home care company, or mail-order company? Do you have choice of provider?

5. Is there a pre-existing condition clause? This clause allows insurance companies to restrict or deny coverage if you have an illness before you sign up. Fortunately, in 1996 the federal Health Insurance Portability and Accountability Act (HIPAA) began protecting patients with pre-existing medical conditions from being denied insurance when switching to a new group policy.

6. Is there a lifetime maximum? This is the maximum amount of money a payer will allow for healthcare services during your lifetime, using the same plan (see #1 above). Once you reach this cap–which could be $50,000 to $1 million or more–you are no longer covered. For healthy people, these amounts might last a lifetime; but children with hemophilia could easily reach the cap before young adulthood. Find out if you have a lifetime maximum, and know its dollar amount.

7. What is the coinsurance? This is the amount of money (or a percentage of costs) you must pay out-of-pocket before the insurance plan will cover you at 100% of all costs in a given year.

8. Is prophylactic treatment covered? In contrast to on-demand therapy, prophylactic treatment can dramatically increase the cost of hemophilia medical care in the short term.

9. Is home treatment covered? Treating your child at home can significantly reduce a single infusion cost by eliminating ER and physician fees. Can you infuse at home? Can you store factor concentrate at home? Can you use a home care company to deliver factor concentrate? Will your plan cover the expense of having an infusion nurse from the company assist at home? Your HTC or local hemophilia organization can help you make these decisions.

10. Does the policy have riders? Riders are legal documents that modify or amend coverage under an insurance policy. For example, riders may be linked to dental and eyewear coverage. Ask how any riders will affect your standard benefit package.

11. Does the policy include catastrophic illness coverage? This is coverage for a major healthcare expense, such as an organ transplant, or for an expensive disease or disorder, such as hemophilia. Find out if catastrophic illness coverage is available, how much it costs, and whether you qualify.

12. At what age will your child no longer be covered under your policy? Coverage for your child may cease at anywhere from age 18 to 24 years, depending on school status and the policy. School status is determined by the individual policy and relates to the total number of school units/credits per semester. Try to maintain adequate insurance for your child until he or she is independent.

Mind-boggling, isn't it? But it's well worth your time to understand your policy. The US insurance system is complicated, and it's essential that you learn about it to protect your child's wellbeing. At the very least, remember this: *Never accept a medical insurance policy without first knowing the answers to the questions above–in writing*. If you are currently unsure of the answers, your medical care coverage may be at risk.

HIPAA: Protecting Against Pre-existing Clauses

Most parents worry excessively about how their insurance payer will react when they add a newly diagnosed baby to their existing health insurance plan, or decide to change plans. Ironically, insurance companies don't always want to insure those who need it most! A pre-existing medical condition may be used to disqualify applicants and save the payer money. People with hemophilia sometimes face this kind of discrimination. But there is good news.

The Health Insurance Portability and Accountability Act was created to protect you against pre-existing condition clauses when you want to change jobs or insurance plans.

HIPAA means that individuals who are eligible for group health plans cannot be denied coverage or have their coverage cancelled if they have pre-existing medical conditions, as long as they meet two criteria:

- They have had at least twelve months of continuous coverage.
- They have had no lapse in coverage for 63 days.

HIPAA also makes it easier for you to switch plans. It provides continued coverage through a group or individual private plan, Medicare, Medicaid, Children's Health Insurance Policy (CHIP), Tricare (military), and any state or local public health plan. And it's especially easy to switch plans during your payer's open enrollment period. Many insurance plans offer open-enrollment periods, usually once a year, when anyone can join, regardless of pre-existing conditions.

If your insurance company has a pre-existing condition clause requiring a waiting period, your child will be eligible for insurance after that time is up. In the meantime, *you* are financially responsible for the monthly premiums and all medical bills. The waiting period could be three months to one year, so be adequately prepared with a stock of factor or alternate forms of coverage.

[6] A preferred drug list (PDL) limits approval to use brand name drugs, usually within a therapeutic class. For example, a plan may have a PDL that lists only one brand of recombinant factor VIII; for approval to use a different brand, you may need prior authorization from your physician. A PDL differs slightly from a formulary: For example, the formulary might allow recombinant factor use, but the PDL restricts your choice to one or two brand names of recombinant product.

Managing Costs

Even when you have a great insurance policy, understand your coverage, and have answers in writing, you still need to contain costs—especially if you have a lifetime maximum. The greatest cost in hemophilia treatment is the cost of factor. *You must know the price per unit of factor.* If you don't, call your factor provider and insurance provider *today*.

If you have to pay some expenses yourself, learn how to estimate out-of-pocket costs well in advance. Be sure you know the following:

- Annual policy premium: For a group plan, multiply the amount taken out of each paycheck by the number of paychecks you receive each year.
- Percentage of copayment: For example, the insurance company pays 80% and you pay 20%.[7]
- Annual deductible per person: How much you pay per person before coverage starts.
- Annual cap per person: Maximum amount the insurance company pays per person per year.
- Annual out-of-pocket maximum: Maximum amount you are required to pay.
- Annual prescription costs: Some plans require a copayment, while others cover 100%.
- Whether prescriptions are applied to the lifetime maximum.

Some costs, such as emergency surgery or hospitalization, are unpredictable. Other costs, such as clinic visits and prophylaxis treatment, or even specific bleeding patterns, are predictable. Try to calculate predictable costs:

Average number of infusions per month	X	Average number of units of factor required per bleed	X	Cost per unit of factor	=	Total factor costs per month

Now you have an estimate of your annual expenses, broken down into 1) what's covered by your medical plan and 2) how much is your financial responsibility. Here are some suggestions to help you manage costs:

Protect the lifetime maximum. Try to negotiate with your factor concentrate provider for a lower factor price. It's not unusual for two hemophilia patients, using the same factor provider and same insurance company, to pay different prices per unit. Consider switching factor providers to get a lower price.

Work with healthcare providers to help ensure that all services are medically necessary. Ask in advance why a procedure is being ordered and how much it costs. Is it medically necessary? Is it being ordered to legally cover the medical team? Should your insurance limitations be considered first?

Investigate the reimbursement programs offered by many home care and pharmaceutical companies. These companies can work with you to contain costs. If you can prove financial hardship, they sometimes waive certain fees or portions of the bill. Some manufacturers offer "compassionate care programs" to provide free product.

Explore home infusion. If you can infuse at home, and the insurance company allows it, you could save thousands of dollars in doctor and ER fees. If you need medical assistance, remember that the ER is expensive. An HTC or urgent care center may be a wiser choice. For some families, the HTC is located far from home. Check with your HTC about alternate, local options for urgent medical assistance.

Get an MSA (pretax medical savings account). Estimate costs, and put money toward the MSA. The MSA also covers new eyeglasses, ancillaries and over-the-counter medications.

Consider a secondary insurance plan. You can obtain one through your employer or on your own. You can usually submit your copays for coverage and reimbursement through the secondary plan, which will lower your costs. But explore this option only if the premiums will be less overall than your anticipated costs when using a single plan.

Coordinate benefits through insurance plans when possible. If you and your spouse already have separate

[7] If you have a copay each time the prescription for factor is filled, find out if the copay is per dose or per prescription. If per prescription, ask if you can get three months' worth of factor with one prescription, and whether there is a limit on how much you can get per copay.

QUESTIONS TO ASK YOUR INSURANCE COMPANY

- Are there special restrictions or requirements for care at an ER?
- Does this policy cover out-of-state medical services?
- Does this policy cover out-of-country medical services?
- If I'm dissatisfied with the services of my HMO primary care physician, how do I file a grievance?
- If I'm dissatisfied with a decision by the insurance company, how do I appeal?
- Can my HTC write supporting documentation to help determine the rationale for coverage of the products and services in question?
- Do you have an open enrollment period?
- Do you contract with a disease management company?
- Am I entitled to receive EOBs?
- Do you use specialty pharmacies?
- What is the monthly premium or portion of the premium I must pay?
- What is the annual deductible per person?
- Are there any copayments?
- What is the annual coinsurance?
- What is the lifetime maximum?
- Will prescription claims be paid at 100% after meeting annual out-of-pocket expenses?

insurance policies, you may be able to increase your coverage by coordinating benefits through both plans.

As you work to manage your own costs, be aware of the cost-cutting tactics of insurance companies. I once received a presumptuous, jolly letter from a company I'd never heard of: "Greetings, Mrs. Kelley! Your son Thomas, who has hemophilia, is now being supervised by our disease management group…" *What?* How did this company get my son's private data? This disease management company was hired by our insurer to offer hemophilia treatment management–silly me, I thought our HTC did that–and more to the point, to monitor our factor usage and expenses. I was not forewarned about this change and knew nothing about the company. What the insurer did was legal, but I quickly learned that I had to watchdog this watchdog group. Would they question expenses? Regimens? Dosing amounts?[8]

To manage costs, become a watchdog. Ask your payer these questions: Will a third-party disease management group monitor my child? How experienced are they in hemophilia? Who has access to my child's health information? How will this information be used? You may find that a third-party disease management group is a cost-containment wolf in here-to-help-you sheep's clothing, hired more to manage costs than to manage disease. As one mother put it, "It's legal, but it's underhanded and infuriating. Parents need to be warned about these tactics." As a parent, managing costs means *not* allowing anyone to interfere with your child's healthcare by using his factor consumption data against him.

Record, Document, File

Each visit to the doctor or hospital, each lab test, each service, and each factor order generates medical expenses and medical bills. Eventually, the medical service provider will invoice your payer. Your payer will reimburse your factor provider or medical service provider for this. Your payer may send you a copy of the Explanation of Benefits (EOB) showing how much was paid and whether you owe a copayment. If your payer doesn't send it (as mine didn't), then request one. In fact, demand one. You need to keep track of what the factor provider, medical service provider and payer are doing. The medical provider will bill you for any copays, or for any products or services not covered by your insurance policy.

From day one, you must keep comprehensive, accurate records of all your EOBs, your insurance policy and any other relevant records. To track your expenses and services, now is the time to set up a filing system, perhaps in a portable file case or on your computer. You never know when you'll need your records as evidence of coverage and payment, to phone your payer or even to present in court.

The time may come when you and your payer disagree about costs, payments and coverage. Always keep accurate records:

- All medical procedures.
- All phone calls with your payer.
- All correspondence with your payer.

Medical procedures include infusions, hospital trips, services received, and doctor visits. Keep records of everything for at least three years to help you determine whether your deductible has been met, to assess your portion of costs, to decide if you are being billed fairly, and to see how close you are to your lifetime maximum. Consider setting up a chart to track each illness and injury.

Records are essential when you have many bleeds, are covered by two insurance policies, or pay a portion of the bills. Healthcare providers and insurance companies do make errors in billing: for example, charging for nonexistent services, billing twice or overbilling. Protect your own finances by tracking errors. Read and file the EOBs you should receive from your insurance company after it pays the medical bill for any treatment.

Always photocopy your claim forms and any correspondence with the hospital or insurance company. You can question and petition errors from a position of strength if you have detailed records to back up your claim.

Take notes on telephone conversations with your insurance company representatives. Some day, you may need to know exactly who spoke with you and what was said. Document full names, titles, time and date of call, subject and conclusion. Ask for direct phone numbers (instead of extensions). You may be assigned a case number when you call. Ask for and record all case numbers.

If you call to ask about alternative insurance policies, *you do not have to identify yourself*. Before you learn all the facts, don't reveal information that may disqualify you or lead to discrimination. You can ask about hemophilia without revealing your name, address or social security number. If questioned, politely explain that you don't wish to be identified. Once you've received satisfactory answers from the insurance company, follow up–get all those answers in writing. My insurance mantra: *Document everything and get everything documented!*

It's a new year, and a new era for Americans with bleeding disorders. Understanding your basic health insurance policy is both necessary and daunting. You need help to navigate this maze, and help is available from many sources. You'll never have to go it alone.

Adapted from *Raising a Child With Hemophilia*, fourth edition, by Laureen A. Kelley. Available in 2007. Provided with a grant by ZLB Behring.

The author wishes to thank Robert G. Dash, manager, Health Care Economics, Baxter BioScience, for reviewing this article for accuracy.

Laureen A. Kelley is the mother of a child with hemophilia. In 1990, she founded LA Kelley Communications, Inc., to provide practical educational materials for families with bleeding disorders. She has a bachelor's degree in child development, and is the author of ten books on bleeding disorders, including *Raising a Child With Hemophilia* and *A Guide to Living With von Willebrand Disease*. Laurie is the founder and editor-in-chief of *PEN*. She also founded Project SHARE, a humanitarian program that donates millions of dollars worth of blood-clotting medicine annually to impoverished patients in developing countries.

[8] While this group stopped contacting me directly, it may still have been retained by my home care company to manage our factor usage and costs.

nonprofit

PSI to Offer Grant for Inhibitor Patients

Patient Services, Inc., has created the Medical Expense Bleeding Disorder Program to offer a $500 grant to patients with inhibitors. The grant helps offset the extra burden carried by people with inhibitors, and can be used toward incidental medical expenses incurred during treatment: travel, childcare, dental evaluations, lodgings.

For more information:
(800) 366-7741 or www.uneedpsi.org

NHF Appoints Kessler *MASAC Chair*

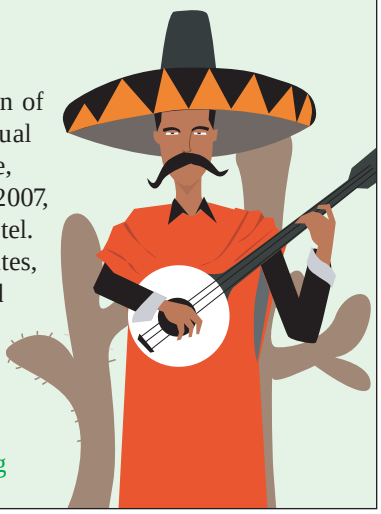
The National Hemophilia Foundation (NHF) has announced the appointment of Craig Kessler, MD, as chair of its Medical and Scientific Advisory Council (MASAC). Dr. Kessler serves on the faculty of Georgetown University Medical Center, where he is chief of the division of hematology-oncology. Over a period of 15 years, Dr. Kessler was director of the adult component of the Washington area Hemophilia Comprehensive Care Center. Currently a MASAC member, Dr. Kessler has served NHF in various capacities, and succeeds W. Keith Hoots, MD.

Source:
IBPN, December 2006
www.marketingresearchbureau.com

HFA *Annual Meeting*

The Hemophilia Federation of America will hold its annual meeting in Albuquerque, New Mexico, March 1–4, 2007, at the Embassy Suites Hotel. Join industry reps, advocates, HTC professionals and consumers at this growing event.

For more information:
(703) 352-7641
www.hemophiliafed.org



manufacturer

ZLB Behring Introduces NEW Helixate® FS Packaging

Helixate® FS and Mix2Vial™ are now packaged together for convenience, to help improve the management of hemophilia A. The package includes one Mix2Vial needle-free transfer device, one recombinant factor VIII protein vial, one 2.5 ml sterile water diluent vial, and prescribing information.

For more information:
(888) 508-6978
www.hemophiliamoms.com

NEW! Wyeth Self-Infusion Training Kit

Wyeth Pharmaceuticals introduces a new training kit containing synthetic hand and elbow models for practice infusions. The kit will be distributed to US HTCs for staff to use when educating young patients about self-infusion. Includes replaceable skin and veins for practicing butterfly needle insertion, an infusion mat, tourniquet, butterfly needles, biohazard disposal container, and sample of ReFacto® Antihemophilic Factor (Recombinant) R2 kit containing powder (not factor) and water for reconstitution practice.

For more information:
Contact your local Wyeth sales representative or (888) 999-2349

manufacturer *continued*

Bayer HealthCare Hemophilia Leadership Development Program

Do you know a college student who has the potential and interest to become a leader in the hemophilia community? The Bayer HealthCare Hemophilia Leadership Development Program is looking for leaders! This is a tremendous chance to see firsthand how a pharmaceutical product is developed and promoted. The schedule includes a visit to Bayer's manufacturing facility in Berkeley, California; a review of state and federal legislative activities; classes on leadership skills and reimbursement; and an introduction to marketing. Participants will also complete an independent project. The program is open to 2–3 college students, touched by hemophilia, who can demonstrate commitment and interest in being future leaders in the hemophilia community. This is an 8–10 week paid ($15 per hour) internship at the Bayer West Haven, Connecticut office. All travel and lodging expenses are covered. Applications are due Wednesday, February 28, 2007.

For more information: www.kogenatefs.com

Grifols PatientCare Program

Grifols announces two programs that will provide a supply of its products to US hemophilia A or B patients experiencing a lapse in their health insurance coverage. The PatientCare Program can temporarily provide Alphanate®, Profilnine® SD or AphaNine® SD factor concentrates to uninsured individuals who need them and who meet the program's financial eligibility requirements. Through PatientCare, patients can receive Grifols product regardless of whether they used the company's therapies in the past. The Grifols Assurance for Patients (GAP) program will provide a supply of product to eligible individuals who have used the company's products for at least three months prior to enrolling.

Source:
IBPN, December 2006
www.marketingresearchbureau.com

Baxject® II *Needle-less Transfer Device for Advate*

Baxject II is now available for Advate. This innovative needle-less transfer device from Baxter BioScience is designed to make the reconstitution and mixing of hemophilia factor easier, faster and safer. Baxject II is compatible with existing injection ports and butterfly sets. Mixing time is faster: less than a minute. Baxject II can be used with Advate and all other Baxter factor VIII therapies.

For more information:
www.baxject2.com or (866) 4-BIOSCI

community news

Blood *Safety*

Noting that there have been no known cases of variant Creutzfeld-Jakob disease (vCJD) in users of plasma-derived factor VIII (pdFVIII) products anywhere in the world, the US FDA has concluded that risk of acquiring this human form of mad cow disease from pdFVIII is exceedingly low, but possibly not zero. In newly released briefing materials, however, the agency acknowledges that there are still too many uncertainties to allow a precise calculation of theoretical risk.

Source: IBPN, December 2006
www.marketingresearchbureau.com

home care

Hemophilia Health Services (HHS) MEMORIAL SCHOLARSHIP

HHS is now accepting applications for one of multiple scholarships worth $1,500 and more. Fifteen scholarships were awarded in 2006. Open enrollment is from January 1 through May 1, 2007.

For more information:
Sally Johnson, (800) 289-6501 x5175
www.factorcare.com

Letters... continued from page 2

Regional Hospital on Tuesday afternoon. The news was refreshing to both Kurt and me. He started receiving the prescribed dosage on Wednesday and already shows improvement.

Kurt is a 25-year-old teacher. Despite his affliction, he has been able to contribute to society through teaching. Kurt's two younger brothers also have hemophilia. The unavailability and the gigantic cost of the drugs has made life very worrying for us.

Project SHARE, [we feel that] Jehovah God has been using you to ease some of the sufferings of mankind. Thank you so very much, and please keep up the good work.

Beverley Parkinson
Jamaica

God bless your whole team and each of your family members for this beautiful cause of helping others less fortunate.

Maria Lanzas, cousin of Yader Velasquez, Nicaragua
Florida

STORM WATCH

My husband and I have eight biological children. Four of them have severe hemophilia. They are ages 28, 18, 14 and 7. Our 14-year-old was diagnosed with type 1 diabetes last April. Coincidentally, he had inhibitors as an infant.

I could go on and on, but right now our main concern is that our insurance company has designated Precision RX as our sole source provider. With the three youngest sons still on

Insights... continued from page 6

production after being exposed to collagen in the damaged blood vessel. This leads to a significant overall increase in fibrin production.

The Clotting Cascade: One of nature's marvels

Why do we need all these clotting factors and other chemicals in the clotting cascade just to form fibrin fibers? The clotting cascade performs several functions, one of which is to amplify the activation of clotting factors and the production of fibrin. Why? So a fibrin clot can be formed quickly and bleeding can be controlled within minutes. For example, factor VIII, working with factor IXa, increases the activation of factor X by more than a thousand-fold; this amplifies the formation of fibrin and the production of a fibrin clot. A second function of the clotting cascade is to keep the process of hemostasis in balance, so blood doesn't clot too quickly or too slowly. This balancing act is accomplished by many feedback loops that allow the clotting process to be either accelerated or quickly shut down, keeping hemostasis in balance.

Bypassing Agents: Taking advantage of the clotting cascade

As explained above, the body has multiple pathways for initiating clotting. The potential for bypassing the intrinsic pathway involving factors VIII and IX was the driving force for the creation of products like NovoSeven®. However, if TFPI quickly shuts down the extrinsic pathway, then why does the bypassing agent NovoSeven® (recombinant factor VIIa) work? NovoSeven works because it is given in large amounts, compared to the natural levels of factor VII. This helps overcome the inhibitory action of TFPI, allowing the cascade to proceed and form cross-linked fibrin fibers. FEIBA® is another bypassing agent, containing mainly non-activated factors II, IX, and X, and factor VIIa. FEIBA is believed to work primarily through the *common pathway*–the point where the intrinsic and extrinsic pathways come together to activate factor X.

But this process does have risks. Since hemostasis requires balance and control, the infusion of large quantities of any factor can disrupt this balance and cause complications such as unwanted clotting. Today, the use of bypassing agents in hemophilia is usually limited to patients with severe inhibitors, or to patients with life-threatening bleeds that can't be controlled by more conservative means.

The clotting cascade is complex, and most researchers agree that we still have a lot to learn. By studying hemostasis and the clotting cascade, we can shed light on another marvel of the human body. We can also illuminate ways to help people with bleeding disorders who suffer from complications like unwanted clotting. Ultimately, research can lead to more effective and safe treatments for both hemophilia and inhibitors.

our policy, the impact will be $600 monthly out-of-pocket. I have read all the information in the *Storm Log* packet. I did not see any suggestions regarding the immediate financial impact this is having on families like ours. What have other families done? I already work three jobs, homeschool our four youngest, and have to do all the other traditional "housewife" things.

Thanks for all you suggestions. I really enjoy your publications and look forward to them each month.

Kathleen and Dwight George
Virginia

Editor's note: PEN has contacted the George family regarding their situation. Readers are welcome to write in and share their comments and suggestions.

We are delighted to get your email updates here. You are so clear in your communications, such a good teacher, and you choose really salient topics for families to learn about all issues. We have admired your educational-advocacy skills for a long time, so keep your emails coming.

Douglas J. and Maureen J. Anderson
New York

Editor's note: Contact us at info@kelleycom.com to receive our free email updates.

Thank you for all the hard work you always put forth for the bleeding community. I read your newsletters and emails whenever they arrive.

Bonnie Genoshe
Colorado

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