November 2009 • Volume 19 • Issue 4

Parent Empowerment Newsletter

**Crossroads**
**Part 3 of a 3-part series**

BY LAURIE KELLEY

# Home Care, 340Bs and Insurers: The Battle Over Your Child's Factor Dollars

This third and final article in the Crossroads series examines how increased pressure from the insurance industry is shifting revenues and power in the factor provider industry. Has cost containment evolved into profit generation? If changes continue unchecked, you may be at the mercy of healthcare decision makers whose main concern is the bottom line – theirs, not yours.

You already know that treating hemophilia is expensive. But have you thought of your child as a valuable commodity? His value is in the "revenue" he generates: if he were a company, he'd be a million-dollar-plus revenue stream. Your child is much more than a patient with a chronic disorder who needs medical care. He's the littlest consumer in a $2 billion industry fueled by the sale of factor. And the dollars he generates each time he is infused are at the heart of massive changes currently affecting every entity that sells factor – your hemophilia treatment center (HTC), specialty pharmacies, home care companies, and even insurance companies.

*Insurance* companies selling factor? Yes, they're the latest newcomers to the factor-selling business. And because they make the final decisions about which factor provider you use, insurance companies can shut out some traditional and local factor providers – maybe even yours. They can switch your home care company or force you to use their pharmacy.

What began about five years ago as cost-cutting measures by payers, including private insurers, may have evolved into profit-generating tactics. Little by little, the community is losing control of where hemophilia dollars go – dollars produced by your child.

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## inside PEN



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# welcome

Sherrell Portrait Design

The healthcare system in our country is at a crossroads, with many reform options to consider. Which road will we take? Town hall meetings, meant to allow citizens a chance to air their concerns, have become frenzied, explosive, and even dangerous. Healthcare – how it is delivered and who pays for it – seems to have become the key issue in America, with people deeply entrenched in their beliefs about how the system should operate.

Hemophilia healthcare is also at a crossroads. Like it or not, it is evolving, and which direction we'll take at the crossroads is unclear. What is clear is that insurance challenges remain our main source of stress. President Obama learned about these challenges for the first time when he listened to Nathan Wilkes, father of a son with hemophilia and inhibitors and husband of *PEN* columnist Sonji Wilkes. Nathan introduced President Obama at the Denver, Colorado, town hall meeting in August. Read about Nathan's thrilling experience in this issue, and watch the video of this event on YouTube.

Our feature article delves deeply into how escalating healthcare costs have led insurance companies to change the way we do business in the hemophilia marketplace. I examine how these changes can affect you, as a person with hemophilia or parent of a child with hemophilia. Because the picture is so complex, I present a history of the industry and how it has evolved. Learn what's happening currently with insurance changes and how they are affecting home care companies, HTC 340B programs, and local nonprofit hemophilia organizations.

This is a watershed time, and many changes will impact us for years to come. Crossroads – which path will we take? Read and learn, ask questions, and be proactive: know your insurance situation, and meet with your local hemophilia organization. A crossroads means we will move forward down one road eventually, one that we all hope will lead to a bright future.

**PARENT EMPOWERMENT NEWSLETTER • NOVEMBER 2009**

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# inbox

## PLASMA-DERIVED AND RECOMBINANT FACTOR

LET ME CONGRATULATE YOU ON YOUR TIMELY ARTICLES IN the August issue. I attended the International Society of Thrombosis and Hemostasis meeting in Boston last month, and the major message from various sources about hemophilia was the increased use of plasma-derived concentrate. I am sure that many parents are confused; they've heard conflicting messages. Your articles are excellent, as usual.

Carol K. Kasper, MD
Emerita Professor of Medicine
University of Southern California, Orthopaedic Hospital
California

*PEN* DOES IT AGAIN: ONE OF THE BEST DISCUSSIONS OF plasma-derived versus recombinant factor. The history of factor development is balanced. The article notes the pioneering work of Dr. Judy Pool; I worked in her lab in the early 1970s (about six years after her discovery of cryo). Families visiting California actually came to the lab to see if she could be interrupted for a moment to thank her. She always came out of her office and graciously met them. Still get goosebumps recalling this.

Richard Lipton, MD
Director, Hemophilia Treatment Center
Long Island Jewish Hospital
New York

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BY NATHAN WILKES

Laura Hoeppner

# *Hemophilia Spotlighted* in White House Fight for Healthcare Reform

My cell phone rang at 10:30 pm on Friday, August 14, with "unknown number" on the caller ID.

We had just gotten home from a late evening hemophilia event and put the kids to bed when I remarked that it was a good thing we didn't have anything planned for Saturday. Rest would be welcome after our hectic week.

Pausing long enough to wonder who would call me this late and block the number, I pressed the green button. "Hello?"

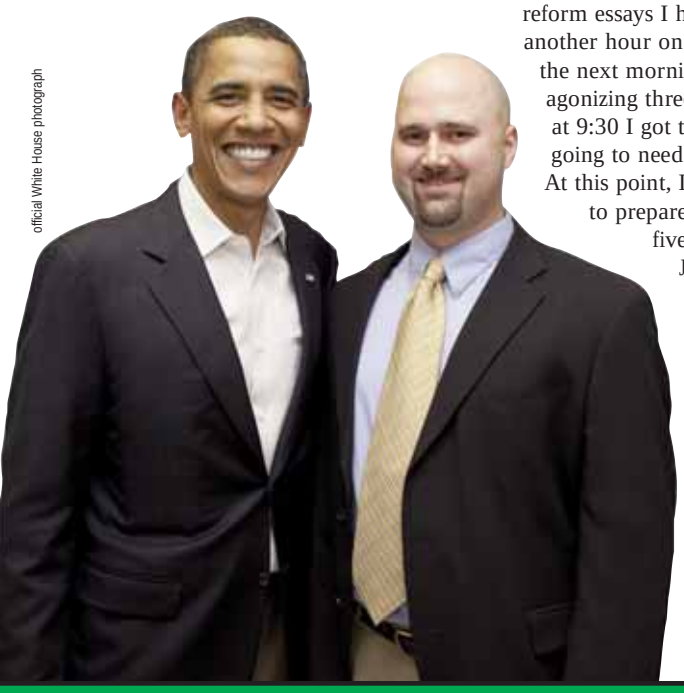
"Is this Nathan?" a pleasant voice asked. "Yes," I responded, still hesitant.

"This is Karen with the White House Office of Public Relations."

"With *who*?" I answered.

She repeated it. I'd heard it the first time – I just couldn't believe it.

President Obama was giving a speech the next day at the town hall meeting in Grand Junction, Colorado, and the White House was looking for someone with a "lifetime cap story" to introduce him.



official White House photograph

As I paced nervously back and forth, Karen and I spoke for nearly an hour about our family's story, healthcare reform policy and what I felt about it, and the potential to introduce President Obama the following day.

The White House Office on Health Reform was intrigued by our family's health insurance horror stories. For years, we'd had "gold-plated" health insurance through my employer. But once our son Thomas was born with hemophilia and later diagnosed with an inhibitor, we saw year after year of unprecedented premium increases, coverage limitations, cost shifting, and outrageous bills. Ultimately, an insurer-imposed $1 million lifetime cap forced Thomas out of our plan and forced me to quit a great job. The state high-risk pool, with its own $1 million cap, gave Thomas only a little over one year more of tenuous coverage before he would be dumped again.

Good story, but the vetting wasn't over. I sent the White House material on my background and some healthcare reform essays I had written. There was another hour on the phone at 5:30 am the next morning, followed by an agonizing three-hour wait. Finally, at 9:30 I got the message: "We're going to need you today. Stand by." At this point, I had only six hours to prepare a speech and drive five hours to Grand Junction to deliver it.

There's something to be said for being prepared, as we had already packed and fueled the car when we got the green light. For the next six hours, as my wife Sonji and I sped across Colorado, I exchanged phone calls and emails with the White House and their advance team. By the time we arrived in Grand Junction, my nerves had been replaced with an adrenaline-fueled euphoria.

For the next 30 minutes, I waited backstage as President Obama arrived and greeted the VIPs. One of my handlers had told me, "He's going to want to talk to you," yet I could hardly believe it.

The president's smile was immediately disarming. Because he is leader of the free world, I had expected an air of pretentiousness, yet detected none. This was not a rock star, but a genuinely concerned everyman. Once the VIP swarm faded away, there was just President Obama, the obligatory security detail, and me. We spoke briefly before I went onstage, and this simultaneously energized me and settled any remaining nerves. My introductory speech was a great success.

By the time the town hall meeting was over, it had begun to sink in: *the President of the United States was talking about hemophilia, to Grand Junction, to the nation.* Not since the Ricky Ray Act of 1998 had hemophilia had such a platform and awareness at the highest levels of government.

Many have asked me, "How did you get selected?" Honestly, this story began in January 2007, when I finally decided to share our family's story – and my ideas for comprehensive healthcare reform – with elected officials in Colorado. After that, our story grew legs… a press conference and a *Newsweek* article in 2007; a feature article in *California Nurses* magazine; quotes in a *Washington Post* article on lifetime caps; testifying before Congress on lifetime caps; and many opportunities to speak about healthcare reform, including three

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BY SONJI WILKES

# *Using Distraction* to Help Your Child Heal



Hatsady Chounlamany was exhausted and frustrated. Her two-year-old son, Ryan, had been battling a bleed for two days. Hatsady had been faithfully administering doses of Ryan's bypassing agent around the clock, and the factor was working – Ryan was feeling better. So much better that he attempted to run and play like any two-year-old. Hatsady knew he should rest more. She called another inhibitor mother and asked, "So how in the world do I keep him immobile?"

Inhibitor parents have all been there: cooped up with cabin fever, sleep deprived, armed with instructions from the HTC to rest and limit mobility, and worried about how long this bleed will take to resolve. Parents are thrilled that their child is feeling better, but unsure how to combat the feelings of restlessness, depression and boredom that lie ahead.

You know how to control the bleed with factor and RICE (rest, ice, compression, elevation). But the "rest" part of RICE works only when you can limit your child's mobility or use of the injured limb. What are some practical ways to keep your child occupied or distracted during an acute bleeding episode?

## Distraction: Trick of the Trade

Inhibitor patients experience frequent bleeding episodes. More often than traditional hemophilia patients, they must restrict mobility in response to a bleed. Bleeds require numerous and repeated dosing of bypassing agents to resolve, which means recurrent needle sticks. Often, bleeds take longer to resolve in inhibitor patients – or just as one bleed resolves, another begins. Parents search for ways to combat the redundancy of boredom, bleeds and inactivity. It takes creativity and a bit of tried-and-true experience to determine what works best for your child.

Carrie Nease is the mother of twin preschool boys, one with an inhibitor and one without. She explains, "Families with severe hemophilia may use distraction to keep a kid off an injury for a bit while they wait for their factor product to work, but families with inhibitors have to bump that up. We have to do this for *days*." Carrie likens it to the hemophilia treatment of yesteryear: even though inhibitor patients have bypassing agents to help resolve bleeds, "parents need the patience and persistence of a saint to get through and beyond a bleed." She says, "Inhibitor families are limited to their arsenal in treating bleeds; we have to rely more on RICE, more on distraction, more on everything a regular hemophilia family has – and for longer periods of time."

So what methods of distraction work best for inhibitor families?

Many turn to video games or television to pass time while recovering from a bleed. Kelly Millette remembers watching *Toy Story* over and over when her son Chris was younger. She also believes in the power of comfort food, and feeds Chris his favorite foods during a bleed.

It's also important for a child to maintain social contact, especially when bleeds take so long to resolve. Julie Baker, mom to Austin, age 14, notes, "Austin likes to text, get on Facebook, and talk to his friends. He loves to go out to eat, so depending on his mobility level, we go out for pizza at his favorite place."

Carrie also likes getting out of the house. "Sometimes it helps to bundle them up and go for a walk – with the one having a bleed in the stroller, of course. It helps to change scenery."

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### Tried & True Distraction *Techniques*

- ☑ Play **video games:** include some that will help teach reading and math.
- ☑ Use **drawing tablets:** encourage your child to draw what he's feeling. Then have him explain his feelings using words. Have him play-act with a doll or teddy bear. Lots of information is available on how to interpret a child's drawings.
- ☑ Watch **television, videos, DVDs:** even if it means repeated viewings of a favorite show.
- ☑ Make **a CD of favorite music:** during an infusion or painful procedure, your child will be more relaxed listening to his favorite tunes.
- ☑ Create **a bleed box:** fill it with special or unfamiliar toys, books, and games.
- ☑ Change **the scenery:** depending on mobility, go out to eat or take a walk.
- ☑ Stay **in touch with friends:** text messaging, email, or social networking sites.


*Transitions* is a *PEN* column sponsored by Baxter BioScience

# Operators *Are Standing By*

BY KEVIN CORREA

They're everywhere — teens hammering away at the tiny buttons on an endless array of wireless devices. Sure, they have incredibly nimble fingers and may habitually run over their cell phone minutes, but despite their communication-savvy natures, many teens with hemophilia are intimidated at the prospect of placing their own factor order over the phone.

## Stepping-Stone

The transition — from parent placing factor order to teen doing so — is certainly straightforward. But it's also key because it serves as a stepping-stone when the teen starts to assume increased responsibility for his healthcare.

From a self-esteem perspective, it's a great transition, combining a distinct responsibility (placing the phone call) with a tangible result (receiving the shipment). And a successful transition gives the teen a sense of accomplishment and builds confidence he'll need down the road.

Karla Watkinson, nurse coordinator at University of Iowa's Hemophilia Treatment Center (HTC), explains that this transition holds many teaching opportunities beyond the act of placing the order. "We really want our teens to understand exactly what patient choice means," she says. "'Why do I use this particular factor and what does it cost? How does the cost of factor relate to my insurance?' These are some of the things they really need to understand."

## When to Start

When should your teen start ordering his own factor? Obviously every child is unique, and there is no specific age at which to start the transition. Karla notes that of the teens seen at her HTC, most do not order routinely until they're about age 15 to 18.

Sal Rio, a 23-year-old with hemophilia, recounts his experience ordering factor for the first time.* "I was probably in seventh or eighth grade," he recalls with a chuckle. "My dad called me into the kitchen, handed me the phone and said, 'I'm tired of calling for you. You're a big kid. Here's the number.' I made the call and placed the order. I have ever since."

Sal's transition wasn't quite as abrupt as it may seem. He knew his home care representative personally from hemophilia events, so calling him wasn't too far out of Sal's comfort zone.

Karla encourages parents to help their teens become more comfortable interacting with adults on the phone. She finds that role-play helps. "We expose teens through role-play at hemophilia camp and chapter events," Karla says. "They'll practice placing an order just as they would practice self-infusing."

## It's in the Mail

Your teen's job isn't done once he's hung up the phone. When the factor arrives, he has more tasks to complete. First, he should check the contents of the shipment. Is the shipment correct? Do the receipt and contents match? If not, your teen needs to notify his home care or 340B rep about the discrepancy.

Once the shipment passes inspection, your teen should log all information, from order placement to infusion. In his treatment log, he should record brand name, lot number, expiration date, and assay size (total international units, IU). Some brands include peel-off labels to make this process easier. This is important to know in the event of a product recall. Karla encourages teens to bring this log to their clinic appointments to help the staff better tailor the patient's treatment regimen.

Finally, your teen should store the factor according to manufacturer recommendations. Though all factor can be stored at room temperature, refrigeration can extend shelf life.

## Pitfalls

"As with any other transition, there will be bumps along the road," notes Karla.

One frequent problem is simply forgetting to place an order. "Even now I still forget to order," Sal admits. His reminders often come in the form of a phone call from his home care company.

Sal's is a fairly common oversight, says Karla. "We get frantic calls from

*continued on page 18*

*The patient's name has been changed for anonymity.

BY ZIVA MANN

# Wanted: Advocate Seeks Partner

## *A Tale of Two Preschools*

Periodically, I put on my nicest shirt, walk into a roomful of teachers – and wish I could hide under the table. But I can't advocate for my sons from under the table. So I sit, sheaf of papers in hand, a list of our legal rights rippling through my brain, and hope that someone here will smile. Listen. And above all, work with me. Because we may have rights, but it takes teamwork – and a caring partner – to achieve those rights.

When Shai was two, I met Judi, director of a local preschool. I poured out our story: Shai's hemophilia, the inhibitor, his ballooning list of serious allergies, my fears and hopes. She listened carefully and, astonishingly, smiled. "Every child has needs," said Judi, simply. "Let's figure out how to meet Shai's needs." And she did, while I watched and learned.

Under Judi's eye, I filled out Shai's first individual health plan (IHP), condensing the overwhelming medical mess into a single, clear page. And I learned to build a positive working relationship with the teachers. Until Judi, I'd thought that advocacy was a battle: me against them. But she believed that advocacy means creating a partnership of shared goals, shared effort, and frequent, honest communication. We became friends, and Shai was happy at preschool. Judi's good will traveled with us to kindergarten. "They are good partners," she promised the new school. So that August, I walked into a room packed with smiling, soon-to-be partners – and Shai bounced into kindergarten.

### Seeking a Partner, But Finding One Size Fits All

A year later, Judi had moved out of town, and now my younger son Akiva was ready to start at a different preschool. That September, I strolled confidently into a meeting with preschool staff. I described Akiva's unusual, aggressive allergies. I explained that he needed more accommodations than the average allergic child, more even than most children with multiple allergies. I pulled out Akiva's IHP, which I'd prepared with help from his physician, and asked if we could think together about allergy management. The preschool director held up a hand. "I have a system for allergies," she said, and left.

My smile froze: there was no Judi here. Instead, there was a *system*, built to handle medical oddities like us. A one-size-fits-all system for allergies.

The teachers and I tried teamwork: quick conversations in hallways, phone calls, emails. But agreements were broken or forgotten, and Akiva got sick from his allergens. Holding him as he wept and coughed, I realized that their system didn't fit us. And I couldn't change it.

Doggedly, I asked for another meeting. There were no smiles as I described Akiva's latest allergic reaction, and how close we'd come to calling 911. "We need to figure out how to meet Akiva's needs," I urged. "Could I see your allergy management plan?"

I stared at their plan, horrified at the gaping holes: an incomplete list of Akiva's allergies, and inadequate accommodations to help avoid an allergic reaction; no mention of cleaning up allergen-laden foods or communicating with us in the event of a reaction. I compared the school's plan to our detailed, doctor-approved IHP, and shuddered. But the director believed her plan – the system – was fine, and the teachers followed her lead. "We don't understand why

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### Be a Good Advocate

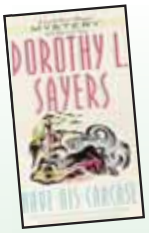
Advocating for your child is a challenge for which few of us are trained, but the skills are simple:

- **Know your rights!** There are many resources – online, print and human – to help you learn.
- **Your Child 101.** Develop an IHP or a similar document to help the school understand your child's needs. Ask your HTC to review the IHP with you, to make sure it's accurate. Invite the school to "slow down" with you; every child is different, even with the same diagnosis.
- **Find a partner.** A school nurse, teacher, or other partner who understands your child's needs, can work with you to use the school's resources to meet those needs. Ask yourself: Is my child's teacher or school a good match for our family?
- **Nurture partner relationships.** Show your appreciation! We bring homemade cookies, and the boys make thank-you cards.
- **Know your resources.** Free legal hotlines, advocates specializing in children with medical needs and in schools, your state board of education, and more.
- **Remember: You have an audience.** Your children don't need you to be the perfect advocate, but they do need to see you try. If something isn't working, dust yourself off and try again.

# Richard's *Holiday Picks*

BY RICHARD J. ATWOOD

Because I like all hemophilia literature (well, almost all), limiting my holiday gift list to four was quite a challenge. For now, I choose for your reading enjoyment these four books about bleeding disorders.




## *Have His Carcase*

**Dorothy L. Sayers**

Everyone should read this classic mystery that set the precedent for all subsequent crime fiction using delayed blood-clotting time due to hemophilia to stymie the murder investigations. The novel has even been recognized in medical literature, in a 1965 editorial in the *Lancet*.

Harriet Vane, a successful writer of detective fiction, and Lord Peter Wimsey, an aristocratic amateur sleuth, investigate the questionable death of a bearded corpse with a slit throat on an English beach. But I don't want to give away the plot!

Sayers, born in 1893, was a scholar in classical and modern languages and is known for her series of novels and stories featuring the elegant amateur detective, Lord Peter, in the years between the two World Wars. The BBC movie with the same title was filmed in 1987 and released on DVD in 2002.

*Harper & Row, 1932*

✦ ✦ ✦

## *'Tis Better to Have Loved and Lost*

**Maxine Bender Segal**

This personal, impassioned, and captivating biography by a fine wordsmith reminds us to be constantly vigilant about blood-borne diseases. In the early 1950s, Maxine Bender's two infant sons, Scott and Douglas, were both diagnosed with severe hemophilia A. Max assumed the roles of nurse, psychologist, teacher, lecturer, organizer and fundraiser. As her son Doug told her, "The crucial and distinctive part of our relationship, what forged us so deeply to each other and what bonded us to you, of course was hemophilia. Although we strived to be like everyone else, we knew, deep down, that we were different from the other boys, but we were not different from each other."

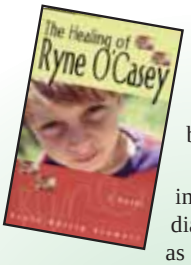
Scott was diagnosed with hepatitis in 1957, and he and Doug were diagnosed with HIV in 1987. In 1988, Max suffered the deaths of both sons from AIDS, the death of her mother, and the not-guilty verdict of their lawsuit against the plasma center and concentrate manufacturer that provided the recalled factor that both of her sons infused.

Max documents the changes in treatment from transfusions in the 1950s, to fresh frozen plasma in 1964, and finally to factor VIII concentrate in 1968. She tells her story in a series of memories, but the facts are precisely documented because she kept a journal.

*Xlibris Corporation, 2003*

## *The Healing of Ryne O'Casey*

**Scott Philip Stewart**




This is the fictional account of a boy born in 1985 in a small town nestled in the Blue Ridge Mountains of eastern Tennessee. Diagnosed with hemophilia A, Ryne leads a normal life while receiving factor VIII "transfusions" at the local county hospital. But in third grade, Ryne is diagnosed with HIV contracted from "contaminated blood." As his condition deteriorates and he becomes homebound, Ryne realizes that his body, his circle of friends, the time he has left, and his whole world are shrinking down to the size of his small bedroom. Using wisdom beyond his years, he wants to experience the miracle of healing, realizing that more than just his body needs healing.

Although the timing of Ryne's HIV diagnosis is doubtful, as are some of the medical details, these criticisms are counterbalanced by the insightful description of having AIDS, and of the uniqueness of this segment of the Southern population. But a few facts shouldn't get in the way of telling a good story. This novel carefully balances tragedy and humor.

Stewart was enrolled in Princeton Theological Seminary in 1986 when his two older brothers were diagnosed with HIV; both brothers died in 1989.

*FaithWalk Publishing, 2004*

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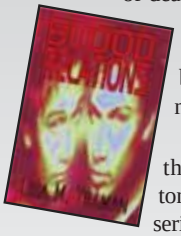
## *Blood Relations*

**Lisa M. Tillman**



This satirical murder mystery takes a jaundiced, yet humorous view of politicians, lawyers, and television reporters while solving the murder of a victim who bleeds to death from internal hemorrhage without major trauma.

Abigail Gardner is enjoying the single life of an investigative journalist until she must return to her hometown to care for her aging father – and also to prove her brother innocent of the murder of his socialite girlfriend. As Abby uses her investigative skills to determine the cause of death, she learns that some of the victim's relatives have also bled to death with no trauma.

The witty names of the characters set the tone for this less-than-serious crime thriller. Despite some misinformation and misspellings, this is a great holiday read: it's fun, and it focuses on the rare bleeding disorders, such as Hereditary Hemorrhagic Telangiectasia, also known as Osler-Rendu-Weber syndrome.

*Hilliard & Harris, 2005*

Terry Rice: This is about making money

In state capitols, corporate boardrooms, and hospital offices, a battle is brewing: who will win the contract to sell your child's factor? Home care companies are merging. HTCs are worried about keeping their doors open. Dollars for community support are shrinking. Terry Rice, person with hemophilia and president of Factor Support Network, a home care company in California, grimly sums up the situation: "This is not about cutting healthcare [costs] or what's good for your child. It's about making money."

Exactly *where* are your child's factor dollars going?

## Background to the Battle

Things weren't always so complicated. From the 1950s through the early 1960s, hemophilia care involved only two players: physician and patient. Patients with bleeding disorders went to the hospital for blood products – fresh frozen plasma or cryoprecipitate. Very few patients administered these treatments in their home.

In 1966, when the first commercially produced factor concentrate, Hemofil, was made by Baxter's Hyland division, a new player appeared: the pharmaceutical company. Following this breakthrough came other manufacturers with new products, and the marketplace grew.

In 1975, a third player emerged: the HTC. These specialized centers of excellence arose from Congressional Public Law 94-63, authorizing federal funds to establish a national network of HTCs to embody the concept of *comprehensive care*, a chronic care model that addresses all the needs of a person with a bleeding disorder.[1] The following year, $3 million was appropriated to fund 26 HTCs. The HTC quickly became the most central player in the life of most people with hemophilia, helping them to be more independent and productive. By preventing many of the costly, debilitating complications of bleeding disorders, HTC comprehensive care has proved highly cost effective.

HTCs were the first factor distributors, distributing much of the nation's factor. In the mid-1970s, plasma-derived commercial factor was extremely low-priced. The resulting low profit margins did not attract commercial providers.

For all they offered, HTCs were initially allocated only $3 million to assist in funding the original 26 centers. This initial federal funding was critical, but limited – enough to fund only one nurse at a small center, or a few mid-level employees at a large one.[2]

## The Innovator: Home Care is Born

In 1978, perhaps the most controversial player entered the hemophilia market, changing the hemophilia industry then and today: the home care company.

Bob Brooks

Innovator Bob Brooks founded Rialto Pharmacy in 1978

Pharmacist Bob Brooks and businessman Hank Stickney worked in a San Bernardino, California, county medical hospital. They both saw firsthand how hard it was for chronically ill patients to visit the hospital regularly to get medicine. They also witnessed the hospital's massive billing errors, which added to patients' woes. Brooks recalls, "Hank and I thought, 'We could apply some basic principles of community retail pharmacy. We can deliver it. We'll give patients the products they want to use, not the products as a result of the county's lowest bid. We could include needles and ancillaries as well as their medicine, to make their lives easier.'" With Brooks and Stickney at the helm, Rialto Family Pharmacy opened in 1978 in San Bernardino – the first home healthcare specialty pharmacy.

Specialty pharmaceuticals, including factor concentrate, are very expensive, fragile, injectible products that cannot be shipped by regular mail. Specialty pharmacies were equipped to handle and ship these delicate products. The idea of home healthcare was brilliant: patients with chronic disorders had medicine delivered to their doorstep. This saved money by avoiding hospital admittance, allowing immediate treatment, preventing long-term damage, and helping patients lead more normal lives. Rialto even took care of billing, reimbursement, and insurance problems, so patients could focus on caring for

1. NHF's Medical and Scientific Advisory Council (MASAC) identifies seven core team members of an HTC comprehensive care team, and another seven extended team members. The core team includes a program coordinator, hemophilia nurse coordinator, medical director, physical therapist, psychosocial professional, case manager, and administrative assistant. The extended team includes a coagulation laboratory director, pharmacist, dentist, genetics counselor, orthopedist, obstetrician/gynecologist, and other specialists such as HIV/infectious disease experts and nutritionists.

2. Initially, funds were distributed directly to the HTCs by what is now called the Maternal and Child Health Bureau (MCHB), a division of the US Health Resources and Services Administration (HRSA). In the early 1980s, HRSA developed a network of twelve regional HTC "grantees," and gave them general grant oversight responsibilities, including identifying and responding to regional needs for developing new HTCs. Instead of funding each HTC directly, MCHB eventually shifted the responsibility of determining regional grant allocations to these regional grantees.


their disorder, not on paperwork.

Later that year, James M. Sweeney, an executive with McGaw Pharmaceuticals and former executive of pharmaceutical giant Baxter Travenol Laboratories, founded Home Health Care of America in Newport Beach, California. The new company focused on home delivery of intravenous nursing services. By 1985, the company name was changed to Caremark, and Rialto became Western Medical Specialties (WMS).

The burgeoning home healthcare industry was very profitable. In the early 1980s, the home healthcare market grew by approximately 20% a year, and Caremark's revenues climbed to $250 million in 1987. WMS saw rapid growth in 1985, higher than almost any industry, making it an attractive mate for Caremark. In 1987 Caremark purchased WMS, combining its home delivery of factor and ancillaries with Caremark's home IV therapies and nursing services. In 1988 Baxter Hyland bought the combined companies, and along with its Highland Plus Division, created Caremark Therapeutic Services in 1988.[3]

With this consolidation, some executives left the company, including WMS founder Hank Stickney and his son Doug, who had joined the team. Eventually, Doug and three colleagues started Quantum Health Resources, a specialty pharmacy servicing people with chronic disorders. High revenues inspired the creation of more home healthcare companies. Diane Martz, who worked for WMS, resigned and founded Hemophilia Health Services (HHS) in 1990 to service only patients with hemophilia, like her son Kyle, who later became HHS president. HHS now serves the most hemophilia patients in America.

By the 1990s, specialty pharmacies and home healthcare companies were commonly referred to as "home care" companies. About ten medium-sized companies and dozens of smaller home care practices were established. Today, some home care companies operate solely to service hemophilia patients. And some are founded and operated only by people with hemophilia. No other chronic disorder has specialty pharmacies devoted only to it – pharmacies often run by patients.

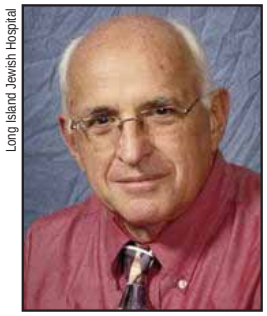
Why? Profits – factor is one of the most expensive drugs. Home care proliferated throughout the 1990s and into the millennium. Private insurance companies, such as Blue Cross Blue Shield and Aetna, and state and federally funded assistance programs, such as Medicaid and Medicare, kept reimbursing the home care companies for factor sales, with few questions asked.

## The Alternative: 340B Programs

Home care was only one way to distribute factor. Another evolved in 1992 and became a way to help HTCs: the 340B program. This program was part of the Veterans Health Care Act (VHCA) of 1992, which allowed federally funded hospitals to purchase prescription outpatient drugs, like factor, at about 15% lower than the lowest manufacturer purchase prices – lower than prices paid by home care companies.[4] The legislation was meant to both preserve scarce federal dollars and allow HTCs to generate a profit by selling factor directly to patients at a markup.

The profit was welcomed. The federal funding for HTCs was considered seed money to assist in establishing treatment centers – it was never intended to cover the full cost of operating an HTC. Government funding for HTCs was low, considering the expansive medical services they offered, especially when HIV hit the community in the 1980s. Although Congress increased the initial funding for HTCs from $3 million to $12 million annually during the AIDS crisis, funding remained flat for many years afterward. At the same time, the number of HTCs grew from 26 to 146, and the patient population increased by more than 85%. As a result, less money was available to each HTC and, in real dollars, funding decreased.

To remain solvent, many HTCs began selling factor, taking advantage of the 340B program. The resulting profits helped fund hemophilia nurses, social workers, and patient services – essentially keeping the HTC doors open. Dr. Richard Lipton, director of the HTC at Long Island Jewish Hospital, makes it clear: "HTCs would probably go away without factor sale revenues."


Long Island Jewish Hospital

Dr. Richard Lipton: Factor revenue is crucial to HTCs

The program was successful. Today, 90 of the nation's 146 HTCs have 340B programs and sell factor to patients. But all HTCs are being hit hard by two economic constraints. First, every year inflation and the cost of medical care escalates. Second, the patient population seen at HTCs has skyrocketed, as more patients are diagnosed with bleeding and clotting disorders, especially von Willebrand disease. As a result, HTC's slice of the now $18.1 million[5] pie gets smaller every year,

3. In 1992 Caremark spun off from Baxter to become independent.

4. In 1992 Congress passed the Veterans Health Care Act (VHCA), which established section 340B of the Public Health Service Act. The PHS Act allows the lowest manufacturer purchase prices on prescription outpatient drugs for certain federally funded entities and public hospitals that treat a disproportionate share of Medicaid and Medicare patients. The intent of Congress was to "enable these entities to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services" (H.R. Rpt.102-384, 102nd Cong., 2d Sess., pt. 2, at 12 [1992]). These covered entities serve special groups of patients, typically the uninsured or low income, those facing catastrophic medical costs, or those underserved in the care of certain diseases, such as AIDS. Federally funded covered entities named in VHCA include community health centers, black lung clinics, family planning centers, Native Hawaiian health centers, and HTCs that receive grants from the Maternal and Child Health Bureau.

5. http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=117&contentid=960 (accessed Oct 1, 2009).

requiring the host hospital to provide more HTC funding. Because of this, some HTCs with 340B programs are being pressured by hospital administration to encourage patients to obtain factor through the 340B – to generate income for the HTC and reduce the financial drain on the host hospital. Lipton admits, "We encourage our patients to use the 340B as their factor provider. The costs are lower, but it's also our primary source of revenue; they need to know that."

Factor sales have now made HTCs with 340B programs direct competitors of home care companies. Indeed, Accredo[6] has about 25% of US hemophilia patients on its service, about the same market share as all 90 HTCs with 340B programs combined. And with competition comes the complaint that 340Bs have an unfair cost advantage: they purchase product below the cost paid by home care. If a 340B charges the same per-unit price for factor as a home care company, the 340B will have a higher profit margin.

Concerns about unfair play exist on both sides. Lipton contends, "Critiquing of 340Bs comes from home care companies, [our competitors]. But I can say that some home care companies are quite predatory."

Despite the competition, home care companies and 340B programs coexisted relatively peacefully for more than 15 years, even finding ways to work together. For example, some home care companies are hired by 340B programs to manage the 340B factor inventory. But a few years ago, a powerful new player arrived and shook the market.

## The Interloper: Pharmacy Benefit Manager

The hemophilia business model of HTC, home care and, more recently, 340B evolved unchallenged for two decades. But things were changing: devastated by HIV and hepatitis C infection, consumers demanded safer products. Improved donor screening methods, better manufacturing facilities, and the advent of recombinant factor led to rising costs for factor and more opportunity for profits – and a proliferation of factor sellers. Healthcare costs were soaring.

So around 2002, another player emerged in the hemophilia marketplace to act as sheriff and chief cost cutter: pharmacy benefit managers (PBMs).

PBMs are companies traditionally hired by insurance agencies to manage the health benefits of their clients, the employers who purchased the insurance plan. For example, a large company might hire a PBM to manage the health benefits of its 250,000 employees. The PBM would examine employees' claims, manage their prescriptions, analyze costs, and find ways to lower premium costs for employers. PBMs also negotiate contracts with pharmaceutical companies for discounted volume drug prices.

From 2000 to 2004, insurers started bringing in PBMs to try to control hemophilia drug costs. The multi-billion-dollar


PBMs were perfectly positioned to enter the hemophilia marketplace. They made their move, and began buying home care companies to establish their own specialty pharmacies. Now, instead of just managing health plans of large employers, they became factor providers too.

Currently, the three dominant factor providers in hemophilia – Medco Health Solutions, CVS/Caremark and Express Scripts – are also the nation's largest PBMs. Express Scripts purchased CuraScript in 2004. Medco purchased Accredo in 2005, and CVS purchased Caremark in 2007.

In 2007 these three top PBMs had a combined prescription market share of about 45% of all US prescriptions. Together, they wield enormous influence in the hemophilia marketplace, including the purchasing price of factor and choice of factor providers offered by your insurer. They've successfully convinced insurance companies that PBMs provide more value for the dollar as disease management specialists, and have cut more costs – securing their place in the insurer's network of medical service providers and crowding out smaller home care companies.[7]

In their role as hemophilia cost cutters, PBMs negotiate directly with manufacturers *and* make product and brand

6. Accredo purchased Hemophilia Health Services in 1997; its name is now Accredo's Hemophilia Health Services.
7. Factor providers compete with each other to become "in-network" with a payer. An in-network physician, hospital or home care company is one that is preferred by your payer, who contracts lower prices with in-network providers. Usually the insurance policy limits the number of in-network providers, while still offering enough choices of provider. This system preserves choice while eliminating the cost of dealing with dozens of separate companies.

decisions on behalf of their clients. When PBMs began acquiring specialty pharmacies, a potential imbalance was created. Could PBMs overstep their authority and interfere with treatment decisions by the HTCs?

While the community pondered how to deal with PBMs, almost no one foresaw the fourth, most recent player to enter the market.

## Fox in the Henhouse: Payers' Specialty Pharmacies

Until recently, insurers maintained the role of payer, reimbursing the home care companies, 340B programs, and specialty pharmacies that sell factor. They tried to lower drug costs with the help of PBMs. But in 2006, a strange twist in the plot occurred: insurance companies began creating their *own* specialty pharmacies to sell biological drugs, including factor. Aetna created Aetna Specialty Pharmacy. WellPoint Anthem created Precision Rx. In this consolidation of power, not only do these insurance companies sell factor, but they can dictate which company patients will use for their factor provider, sometimes forcing patients to use the insurer's own pharmacy. Patients who were lifelong customers of one home care company suddenly had an unfamiliar factor provider, often giving substandard service, and with insufficient knowledge of hemophilia. Unlike most home care companies, payer specialty pharmacies offer no program or financial support to the community.

Payers are becoming increasingly integrated: they can purchase factor, switch patients to their pharmacy, and sell factor at their own price with no third-party negotiation, all in the name of cost containment. This is radically changing the flow of revenues in our community. Gary Mull, who once worked for a factor manufacturer and recently was acting president of American Homecare Federation, believes, "The patient community is being impacted in a negative way. Insurance companies for the most part are calling the shots now."

"Just on September first," notes Terry Rice, "United Healthcare informed every policyholder that factor would be billed against the pharmacy side, not the medical side. Once factor is on the pharmacy side, the insurer can tier it [which may limit product choice]. They also gave patients three choices of factor provider: Prescription Solutions (United's own specialty pharmacy), the local HTC, or one specific home care company that United chose."

Andy Matthews, a 43-year-old with hemophilia from Texas, received the news on September first. "United is trying to cut out a lot of home care companies," he contends. "There wasn't a bid to the lowest cost provider, which would save money; they just chose one major home care company." Matthews adds, "There's often a longstanding relationship between a single home care company and the insurer who selects it as an option. It's a good ol' boy network. A monopoly like this is the worst thing for our community. There's less charitable funding to chapters."

Bob Brooks, now director of National Cornerstone Healthcare Services, asks, "Should payers really be involved in dispensing of therapy? Should payers have their own pharmacies? Clinics? Infusion suites? I mean, when does it stop?"

## Fractured Industry Needs Healing

The hemophilia industry, especially the factor delivery system, seems fractured. Home care companies acquire each other, PBMs acquire home care companies, while insurers jump into the fray to sell factor. "It's chaotic today," observes Brooks. "There are payers who assert themselves and limit choices. We continue to have home care companies who are large, and smaller ones trying to [survive by] carving out a niche."

Joe Pugliese, president of Hemophilia Alliance, a nonprofit consortium of HTCs with 340B programs, describes the hemophilia community as "in flux." But he adds, "Patients still generally have coverage and get assistance."

If there's one patient in need of help, it's the HTC.

Ann Rogers, executive director of Delaware Valley Chapter of National Hemophilia Foundation (NHF), mother of a son with hemophilia, and well-known advocate, adds, "We're grateful when we have good insurance, but payers, in an effort to control skyrocketing costs, are applying disease management models that work well with other chronic diseases – like asthma, arthritis, and diabetes – to hemophilia. We have 146 federally funded HTCs that have a standard model of treatment. This model works and should not be reinvented, most especially by insurance companies. Insurance companies don't need to establish a disease management model for hemophilia. The HTCs already have it, and it works."

Delaware Valley Chapter NHF

Ann Rogers: Conflict of interest is inherent when a payer owns a pharmacy

This fractured industry has bled factor dollars away from 340B programs. Federal grant funding for 340Bs has remained flat for ten years, at about the same level of funding as the $3 million allocated by Congress in 1976, adjusted for inflation. But instead of being distributed to just 26 HTCs, funding is now shared among 146 HTCs.

Increased competition, or perhaps outright greed, has caused some factor providers to engage in questionable practices. Mull observes, "Some home care companies target high-end users, those on Immune Tolerance [Induction] or with HIV, to bring in more revenue. Some would pay higher commissions to the reps who brought these types of high-end users on board."

It's well known that some home care companies hire patients from the community (some unqualified for the job) and ask them to bring their own personal business – their child's factor dollars – to that company. The Blue Cross Blue

Shield fraud units are aware of this, and they do investigate. Newswires buzzed this summer about two home care companies facing insurance company fraud unit investigations into their hiring and reimbursement practices.

Insurance companies are more aware than ever of spiraling costs and fraud, but how will their cost cutting and investigation into fraud help you, the consumer?

## Cost Containment or Capital Control?

Insurers have pressed for years to contain healthcare costs. The short-term goal of cutting reimbursement for hemophilia treatment is to minimize overall costs within a given fiscal year. This keeps employer premiums down and helps states and federal assistance programs stay within budget.

But is the goal now something else? If costs are lowered, the savings could be passed on either in the form of a lower price per unit of factor (helping to extend your child's lifetime cap) or as lower annual premiums for your employer. Alternatively, cost savings could become *capital*, funneled back to Wall Street in the form of higher dividends for stockholders.

So far, extended lifetime caps or lower premiums resulting from cost-containment methods have not been demonstrated in the hemophilia community.

"We now have a stockholder model of healthcare," says Rice. "We don't see the costs to patients going down. We just want to know where the money is going. The price of factor has not dropped."

Mull adds, "Insurance companies say they want to decrease the outlandish costs of hemophilia service and product, but they aren't doing it. Costs may even have gone up. The profits are going to the insurance companies."

Rogers agrees. "The health insurance industry is not underfunded. Just look at their financial reserves: one Blue Cross of Pennsylvania had third quarter 2008 reserves of $2.2 billion. And that's only the reserves of one Blue Cross."

Bob Charles, former vice president for a major insurer, sees a bigger picture. "Cost containment is still the primary issue. There *are* savings to the healthcare system; that's been proven. [Without intervention of payers] patients may be facing higher premiums or higher co-pays. The fact that there haven't been premium or cost-share increases for those with hemophilia is a testament to the payer's cost-cutting strategy."

Many in the hemophilia community see cost containment this way: patients have been switched without warning from their preferred home care company to another company, or to their insurer's specialty pharmacy, where complaints abound about substandard services. Small home care companies are struggling to survive or have been forced out of business. Charitable dollars have shrunk for scholarships, hemophilia chapter funding, camp support, and other programs. Your child's factor dollars are disappearing into a vortex of powerful companies, run by powerful executives, who seek to maximize shareholder wealth. Your child has become a cash cow, milked for the greatest profits.

## The Future of Factor Distribution

No one doubts that specialty drug costs have spiraled out of control. The *New York Times* reported recently that expensive specialty drug spending grew 15.8% in 2008, up from 12.4% in 2007,[8] and could climb to more than 20% by 2012. Scary forecast.

But cost containment should help extend your child's lifetime cap or allow factor provider choice. Mull notes, "It would be ideal if payers could hold costs down and pass savings on to patients in the form of lower deductibles or lower premiums, but that's not happening."

Charles admits, "There are savings, unquestionably. One large regional Blue insurer covering over five million members created up to a 20% reduction to overall billed charges by using its own specialty pharmacy. Where's the 20% savings going? I'm not sure; it needs to go back to the plan sponsors. Right now it may or may not be."

Rogers believes that costs must be contained and insurers are the ones to do this, but adds, "I think it should be illegal for a payer to own a pharmacy. There is an inherent conflict of interest."

If current trends continue unchecked, what's the future for all factor distributors in this rapidly changing healthcare industry?

HTCs with 340Bs may see revenues slowly decrease as they lose factor sales to PBMs and insurer specialty pharmacies. HTCs currently not offering 340B programs may need to start them to remain financially solvent.

8. Medco's 2009 Drug Trend Report. p. 4. www.medco.mediaroom.com

Mull believes that 340B programs are the next target on the insurers' radar. "Right now you may get to choose a 340B as a factor provider, but once insurers get rid of small home care companies, 340Bs will be next. They may be pushed out."

And Rogers warns, "We may see small home care companies go away. In the next ten years we may see continued mergers of PBM with specialty pharmacies." On the heels of the $4.7 billion sale of Precision Rx to Express Scripts, Aetna now is considering selling its own Aetna Specialty Pharmacy. Who could afford such a sticker price? Only a PBM. More consolidation.

If you specialize only in hemophilia, "the small home care company future is bleak," admits Rice. "To specialize in one or two disease states is risky."

But Charles doesn't consider the shrinking choice of factor provider as important as other medical considerations: "If you can save 20% by switching to a different specialty pharmacy, then payers will leave alone expensive treatments like prophylaxis, or next-generation brands."

Even PBMs may be in for a rough ride. The *Wall Street Journal* recently reported that the Senate is encouraging PBMs to be more transparent in how they operate. According to reporter Ann Ziegler in *FierceHealthcare*, this is "something they haven't been good about in the past." She notes that even employers wonder whether the profits PBMs make are worth their cost-cutting, middleman services.[9]

As factor distribution consolidates and funds go to Wall Street, charitable giving, a lifeline to local hemophilia nonprofits, may drop.

## Solutions?

The national debate over healthcare has heightened the need to improve the factor distribution system. "Congress needs to target insurance," suggests Mull. "We need a panel of chronic disorder patients to advise Congress, to give directions of where there are savings."

The hemophilia community offers a variety of solutions. Rogers says, "When a health insurer has an unbelievable financial reserve, from saving money by implementing these cost containments, or in some cases, by denying medicine and treatment altogether, then they should have to return the excess premiums they collected from the people who paid them."

Everyone agrees on one thing: standards of care might be the answer to slow the fracturing of the factor-selling industry. The NHF Pennsylvania chapters were successful in unanimously passing House Bill 620, the Hemophilia Standards of Care Act, in the Pennsylvania House of Representatives on August 5. HB 620 stipulates that insurers must provide options in pharmacy and home supportive services so that as a patient's needs change, the home care pharmacy option may also have to change, to accommodate patient needs. The act, when passed in the Pennsylvania Senate and signed into law, will require each insurer to have a minimum of three full-service providers, which are defined in the bill.[10]

But Rice stresses, "We need to ensure the Standards of Care legislation gets passed in every state."

Rice offers advice to parents whose children with hemophilia fuel the profit fire. "Wake up and be vocal about this. As one insurance company does, the other does. Even if it's not affecting you today, it may tomorrow. You'll start screaming when you find you've been switched to a different home care, when your new specialty pharmacy doesn't know how to deliver your factor properly, when dollars are gone from the community – but by then it's too late."

Pugliese wants HTC programs supported, not only for the expert medical care they provide, but for the support they offer. "When I started with the Hemophilia Alliance, there were criticisms: some say HTC 340Bs don't give back. But we donated $267,000 to chapters and HTCs from the Alliance Foundation, and we're preparing for another cycle of grants in 2010." Insurers with specialty pharmacies have yet to donate to the community.

Even stability won't guarantee where the profits go. Bob Brooks reflects on the 35 years since he started a small, community-based pharmacy that shipped specialty pharmaceuticals to patients' homes, and on the subsequent avalanche that became the home care industry. He never imagined the impact – both positive and negative. "We could do so much more for the patients if we could work together," he says. "The challenge is dealing with corporate America, and realizing that it can't be just a bottom-line focus only."

The bottom line now belongs primarily to shareholder wealth and exorbitant executive pay, and ignores the source of all revenues – your child with hemophilia. Your child needs to preserve his lifetime insurance cap, have access to top-level factor delivery and disease management, and enjoy thriving HTCs. The excellent level of care he receives today was forged from robust competition, innovative ideas to meet unique needs, and compassionate support of the community. Let's not let these values slip away.

9. Zieger, Ann. "Reform should hit pharmacy benefit managers hard." http://www.fiercehealthcare.com/story/reform-should-hit-pharmacy-benefit-managers-hard/2009-08-31
10. Press release. Delaware Valley Chapter of NHF. August 5, 2009.

manufacturer

*Wyeth Pharmaceuticals and Catalyst Biosciences:*

## Agreement to Develop and Commercialize Factor VIIa Products

Wyeth Pharmaceuticals and Catalyst Biosciences, Inc., announced that the two companies have formed an exclusive worldwide collaboration for the discovery, development, and commercialization of factor VIIa products to treat hemophilia and other bleeding conditions. Why this matters: There is currently only one factor VIIa product available worldwide, NovoSeven®RT. Competition may lead to lower product price.

*Source:* Wyeth_News@wyeth.com

## New Self-Infusion Kit

Bayer HealthCare introduces BayCuff™, an adjustable cuff worn on the hand or arm that allows patients to practice self-infusion without actually infusing themselves. Why this matters: Many hemophilia patients who have grown up with ports lack self-infusion skills. A training kit may give them more confidence to self-infuse.

*For information:* www.kogenatefs.com

## Higher Doses for Kogenate® FS and Helixate® FS

The US FDA has approved a 3,000 IU vial size for both Kogenate FS (manufactured by Bayer) and Helixate FS (distributed by CSL Behring). Kogenate FS comes in a conventional vial-to-vial reconstitution system, as well as the Kogenate FS Grab & Go packaging with BIO-SET®. Helixate FS is packaged with the Mix2Vial reconstitution set. Why this matters: Greater convenience; may eliminate the need for combining smaller vials; may allow some patients to achieve more precise dosing.

*For information:* www.kogenatefs.com and www.HelixateFS.com


## How Much Does Your Factor Cost?

If you want to compare factor VIII product costs, visit a new Talecris website and calculate usage. You'll need to know the per-unit price of your product. Why this matters: Talecris obviously wants to show the lower cost per unit of a certain safe and efficacious plasma-derived product, but all of us should know our per-unit cost, important for preserving lifetime insurance maximums.

*Visit the new website* www.factor8cost.com

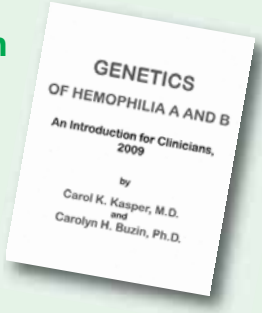
medical

## Royal Disease Identified

British Queen Victoria's family and her descendants, including the Russian royal family, had a severe form of hemophilia B, according to Dr. Evgeny Rogaev of the University of Massachusetts. Rogaev conducted DNA tests on the remains of the Romanovs, including Prince Alexei, who had hemophilia. Victoria's granddaughter Alexandra married Nicholas II, the last Russian tsar. The entire family was assassinated on Lenin's orders in 1917. Why this matters: This settles a decades-old medical mystery.

*Source:* www.sciencemag.org

## Hereditary Transmission Explained




*Genetics of Hemophilia A and B*, by world-renowned Dr. Carol Kasper, is for clinicians primarily, but also for anyone who wants an in-depth, comprehensive look at how hemophilia is transmitted from parent to child. Why this matters: Though it may be technical, this book can come in handy when you visit your child's pediatrician, school nurse, or other medical professional involved in your child's care. No charge, free shipping.

*For information and to order:* carolkasper@hotmail.com

home care

## New Children's Book on Inhibitors


*Quest for Infusion: Nate Goes to Camp* by Chris Perretti Barnes, mother of a child with hemophilia, is third in a series of children's books produced by BioRx and Bayer Healthcare. Nate, a young boy with hemophilia, goes to summer camp, learns to self-infuse for the first time, and makes an unlikely friend. Why this matters: So far, Nate is the only storybook character with an inhibitor. Comic-book style publication is free.

*For information and to order:* www.biorx.net

## New Resources for Inhibitor Patients

Grifols is now offering two new resources:

- Spanish version of *Inhibitors in Hemophilia A*, titled *Inhibidores en la hemofilia A*
- *All About Inhibitors* children's book

Why this matters: More resources in Spanish are always a good thing!

*For information:* Grifols USA, LLC, 888-GRIFOLS www.grifolsusa.com

## New Website and Program for Inhibitor Patients

The website www.myinhibitor.com is now www.changingpossibilities-us.com. Inhibitor patients can apply for financial aid, find educational materials, and receive insurance advice. Novo Nordisk's SevenSECURE® program can help you manage the costs that come with inhibitors. Experts on hemophilia with inhibitors can answer your questions about financial, insurance, and educational support. Confidential and free. Why this matters: Inhibitors can cause insurance problems and disrupt home life. Financial help and expert advice can ease the stress.

*Visit the new website changingpossibilities-us.com*

## First Recombinant Factor VIII Produced From Human Cell Line?

Octapharma, the Swiss-based, third-largest manufacturer of plasma therapies, sponsored a symposium at the International Society of Thrombosis and Hemostasis in Boston in July. The symposium, "From Humans to Humans: Introducing the First Recombinant FVIII Produced From a Human Cell Line," discussed a new recombinant VIII compound recently entered into clinical studies. Instead of existing hamster-derived cell lines, the studies use a human recombinant factor VIII protein expressed in ahuman cell-based protein expression system. Clinical trials are expected to start in the US later this year. Why this matters: It's thought that using a human cell line in recombinant factor VIII products might reduce inhibitor formation.

*For information*: www.octapharma.com

## Helixate® FS Receives FDA Approval for Routine Prophylaxis in Children with Hemophilia A

CSL Behring announced on August 17 that Helixate® FS has been approved by the US FDA for routine prophylaxis in children with hemophilia A who are age 16 or younger and do not have preexisting joint damage. Why this matters: Parents might be able to get insurance reimbursement for prophylaxis more easily with an FDA indication.

*For information:* www.HelixateFS.com

nonprofit

## Cash Contest for Girls with Bleeding Disorders

MyGirlsBlood is a nonprofit with a mission to connect women and girls with bleeding disorders worldwide, for mutual support and education. It also offers an essay contest with cash prizes: any girl in the world with a bleeding disorder can submit an essay. Why this matters: Here's an easy and positive way to get recognition and empower women with bleeding disorders worldwide.

*Visit* www.mygirlsblood.com

international

## Poland to Purchase Factor

For the first time ever, Poland's National Health Fund has purchased 40 million IU of factor VIII for prophylactic therapy of all children aged 2–18 with hemophilia A. Reportedly, Octapharma was awarded the tender. The Polish Ministry of Health purchases clotting factors for adults in Poland; this year it purchased approximately 100 million IU of factor VIII concentrate. Why this matters: We should keep in perspective that even developed countries may lack standard treatment protocols.

*Source:* IBPN, September 2009

*Inbox... from page 2*

I LIKE HAVING A HARD COPY OF *PEN* and keep most, if not all, issues in a file for future reference. Thank you for your remarkable work! The hemophilia community is fortunate to have you.

Martha Liuzzi
Georgia

## PROJECT SHARE

GOD BLESS YOU RICHLY FOR THE donation. Our children were safe for many months of 2009, for they have immediate treatment with factor VIII whenever they get injured. Thank you indeed.

Tadesse Belay
Ethiopia

THANK YOU ALL SO MUCH! I DO NOT know how to put in words the joy I am experiencing now, thinking my son Matt will be fine. We thank God for you all!

Orlando Legson
Philippines

MY SON AND I WANT TO SAY THANKS TO you and everybody else who made it possible for him to get the factor. Because of Project SHARE, Hartnel and many others like himself can get their surgery and go on with their lives. As I write to you my eyes are full with tears, tears of joy knowing that my son will be able to get his surgery and finish school. I will always remember what you have done for us and continue to pray for you and the many others that help. I will keep in touch and keep you up to date about Hartnel's health.

Utris Murphy
Jamaica

*Ed. note: Hartnel died on August 15, 2009, despite a donation of factor.*

THANK YOU ALWAYS, AND GOD BLESS you. Ethan is 15 months now. Recently he cut his finger and it bled. We went to the hospital with the factor you donated, and he was done in 30 minutes! Thank you guys for taking us this far.

Maureen Miruka
Kenya

THE FACTOR ARRIVED ON TIME SAFELY on September 10. It took only two days. Adrian of Romania started the therapy and is feeling better. He can now find a comfortable position to sleep in. I will keep you informed of his improvement. Many thanks for saving this man's life.

Adriana Henderson
President
S.T.A.R. Children Relief

I WANT YOU TO RECEIVE ALL MY gratefulness for all you do for me, and for the factor you donated. Once again, many thanks.

Franck Hermann Ohouo
Ivory Coast Hemophilia Society

THANK YOU SO MUCH FOR ALL THE support you are giving to our patients with hemophilia. More power to you and Project SHARE.

Marietta D. Charvet
President
HAPLOS Inc.
Philippines

## A SPECIAL ADOPTION

*LA Kelley Communications learned from Melissa Penn at the New York City Hemophilia Chapter about Lu Feng, an eight-year-old orphan in China with hemophilia, available for adoption. Laurie sent an email to her contacts and within one week, a family with a son with hemophilia decided to adopt Lu Feng. Project SHARE supplied factor for Lu Feng, and through our contacts, within one week, we raised $17,000 to help pay for adoption fees.*

THE POWER OF COMMUNITY NEVER ceases to amaze me. The power of the individual, to make a true difference, also leaves me awestruck. Now the forever family has the money to adopt Lu Feng. He will have factor, health, and a family to love him. And this incredible family will have a new son, a new brother. Their family is now complete. A life has been saved, a family formed. It is wonderful.

This leads the way to help even more people. As Lu Feng's doctor mentioned, there are other boys with hemophilia in orphanages in China. Perhaps other people may come forward to express interest in adopting, and perhaps the system could be in place to get these boys factor.

The power of the bleeding disorder community and the effective, quick and resounding responses are amazing, humbling and leave me awestruck. Thank you, everybody.

Melissa Penn
Executive Director
New York City Hemophilia Chapter (NYCHC)

WHAT A MIRACLE, AND A GREAT outpouring of generosity and caring! People are moved by the plight of an individual child with a name and a face. We can't thank you enough for arranging for Lu Feng to receive his medication, and for helping the family with the adoption expenses. He'll be coming home soon!

Pam Thomas
Executive Director
Homeland Adoption Services

THIS COMMUNITY, WHICH WE ARE blessed to be part of, has amazed us with its quick response to help our family bring Lu Feng home. When we began this journey, we so appreciated your support and encouragement of our decision as a family to bring Lu Feng into our home as son and brother to our three other children. Beginning with your initial email about this eight-year-old boy in China, then your efforts to get factor to him, and your pledge to get the word out to the hemophilia community that our family needed financial help to make this dream come true, you never wavered in your commitment to our little boy in China. For that we will be forever grateful. When we began the fundraiser and the first donations began pouring in, we sat at the computer with tears of relief rolling down our faces. We'd never imagined that in less than three days, we would not only meet our goal but surpass it by more than 50%. With the funds raised, we'll

Luckey family awaits its newest member

be able to pay all of our remaining fees for the adoption, purchase our airfare; and we'll have enough left to cover the majority of our living expenses while in China. Thank you does not seem sufficient to express our feelings of appreciation for those who donated and made this a reality.

Currently it has been 63 days since China logged in our adoption paperwork. Typically it takes around 90 days to process this paperwork. Once we receive the official travel approval from China, we have some more paperwork here in the US, and then we arrange our travel plans. We will keep you posted as the journey unfolds.

We are busy at home preparing for Lu Feng's arrival. Bedrooms have been reassigned and redecoration efforts are underway. Two weeks ago, we purchased a bedroom set for Lu Feng with bed, armoire, desk and chair. We are excited for him to see his new home and his very own room!

When he is able to understand, we will tell him how loved he was from the very beginning of his life: loved by his birth parents, who had the courage when he was only a year old to leave him at the one place they knew could help him, the hospital; cared for by the Chinese doctors who gave him the best treatments they could with the resources available to them; nurtured by the caregivers at the orphanage every day; and cared for by the hemophilia community around the world, who united to get him medication and help bring him to America. And finally, he was loved by his forever family in Michigan, before we ever even met him. He has a mother, father, big brother and two big sisters, three grandparents, aunts, uncles, and nine cousins waiting for him here in Michigan to love and support him from the day he arrives home and forever.

We thank you all for your support and will keep you updated as we move forward.

Dave, Shari, and the four Luckey children
Michigan

## ON FACEBOOK

MY SON, DARIAN, HAS SEVERE hemophilia. I cannot say thank you enough for the informative materials you provide! He's 13 now and doing wonderful. The first year was very hard: the not-knowing stage. Your books really helped during this period and continue to help now.

Candida Ross
Massachusetts

WHEN MY SON WAS LITTLE, I WAS ALONE in the hemophilia world for a long time. Your writings were helpful and educational. Now, 24 years later, here we are! Thanks for all you do!

Kathe Gusler
Illinois

## *PEN'S INSURANCE PULSE*

THANK YOU FOR THE TIME, EFFORT AND initiative in putting together *PEN's Insurance Pulse*. As the parent of a four-year-old with severe hemophilia B, one of my biggest concerns for my son's long-term health is insurance coverage. Thankfully, I currently have a stable job with an insurance plan. However, my insurer recently forced me to switch factor providers, and as a result, I lost payment coverage for home nursing care and the wonderful nurse who has been visiting my home for the past two years so my son could receive prophylactic infusions.

Your inaugural newsletter provided very valuable insight into keeping an eye on lifetime maximums, and for monitoring my insurance policy. It's nice to know that there are so many like me who feel concerned over the slow contraction of coverage and benefits for hemophilia patients.

Thank you for all the work that you put into the terrific LA Kelley publications.

Dean Grayson
Maryland

*PULSE* IS A GREAT RESOURCE! Thank you for sharing.

Melinda Cadena
Hemophilia Association
Arizona

PLEASE CONTINUE TO PUBLISH *PULSE*. I have a two-year-old grandson with hemophilia and inhibitors, and I'm worried about his insurance coverage in the future. With insurance coverage dependent upon employment, and with the economy depressed, at any time his parents could lose their insurance – plus the worries about reaching the caps. This publication is very valuable.

Bonita Carlson
Illinois

I RECEIVED THIS FIRST ISSUE AND DIDN'T stop reading until I finished the whole document. It is very informative, written in interesting style, and it alerts the community to potential problems. We should each individually, and through our associations, take action to prevent reduced access to factor and medications. I hope you will continue producing *Pulse* on a quarterly schedule.

Dan Chaij
Tennessee

*PULSE* WAS VERY INFORMATIVE. I REALLY learned important information. I saved it, and filed it under "insurance" so I could reference it as needed. Keep it coming!

Shelley Jajeh
California

I RECEIVED *PULSE* FROM HEMOPHILIA OF North Carolina. I found it interesting and informative and will keep it as a reference. I was especially interested in the Medicare section of the feature article, "From Cradle to Medicare," since I am now 63. For future topics I would be interested in knowing if any of the many available Medicare Supplement options (A–L) are particularly suitable for someone with hemophilia. I look forward to future issues.

Jim Jarratt
North Carolina

I LIKED *PULSE*. IN DECEMBER, when my husband lost his job, we thought we would be okay because we could get COBRA. We were wrong! Your state and the number of employees in your company determine if the company offers COBRA. Ours didn't have to offer it, and they didn't. That left us stranded and struggling to find insurance for our children. From that experience, and with the help of a friend at Farmers Insurance, we were able to get a HIPPA eligible plan through Blue Cross Blue Shield. We met all of the qualifications, and for $263 per month, we have an individual policy that covers just our son, and also covers all of his hemophilia needs. I have even referred this plan to other families about to lose coverage.

It was so exciting to have you at the Hemophilia Association [Arizona] annual meeting in August. You opened many eyes to the needs of the world. The problems we have here, locally, now seem pretty insignificant in comparison.

Thank you for publishing this valuable information, and keep it coming.

Elizabeth and Ken Pulley
Arizona

## JUST A BOY


I WANT TO LET you know that I used your new publication, *Just A Boy*, with one of our young families. The outcome was great because both mom and the child got the idea after reading it. The child "rooooared" with much gusto. We all laughed. Congratulations on another valuable publication.

Elizabeth Fung, LCSW, PhD
Children's Memorial Hospital, Chicago
Illinois

*inbox*

*Transitions... from page 5*

patients saying, 'I forgot to order my factor and I'm on my last dose!' Sometimes you have to let them learn what the consequences are when they forget. And in some cases (if the pharmacy is local) they may have to get in the car and go pick it up."

It's best to have a reserve of factor on hand. As every patient's treatment regimen is unique, consult your HTC to determine your ideal reserve quantity.

Your teen should add the phone numbers for his home care company or HTC 340B program to his cell phone. Place these numbers in prominent locations – on his computer or refrigerator – to help serve as visual reminders.

Misplacing the paperwork for a previous order can also lead to headaches. It's difficult to argue with a pharmacy or insurance carrier over wrongful billing if you don't have the documentation to make your case. Keep those shipping order statements!

## Next Steps

In addition to teaching a necessary life skill, this transition can introduce topics like patient choice and lifetime insurance caps, which will follow your teen for the rest of his life.

A good way to initiate the process is to speak with your HTC or home care team to develop a plan. Once your teen has begun the transition, praise him for his efforts to take charge of his healthcare.

Recalling his own experience, Sal offers this insight to hemophilic teens: "Ordering your own factor is one simple thing you can do to take some of the workload off your parents." And it's a great way to transition to independence.

*Homefront... from page 6*

we're here," a teacher admitted. "Will Akiva be at school tomorrow?" Slowly, painfully, I shook my head.

And swung into action. I called the state board of education and a free legal hotline to ask about the preschool's legal responsibilities. I studied resources like Wrightslaw,[1] pestered an advocate specializing in disability and education, and contacted the national food allergy association (FAAN).[2] "Schools can assume that they know enough about allergies," a FAAN senior staffer warned me, "and don't slow down to learn more."

Akiva's preschool didn't just fail to slow down – they badly underestimated his needs. Later, we learned that this preschool director had believed that Akiva couldn't be as allergic as I'd claimed; that may explain why she chose their system over Akiva's IHP. I knew our rights: we could sue for discrimination or breach of contract, or file a formal complaint. But a lawsuit wouldn't get Akiva back into preschool – only a partnership would. And I didn't have one.

## Seeking a Happy Ending, and Finding an Advocate

For weeks, I'd shake my head when I spoke to Akiva's preschool director. No, I wouldn't sign a waiver promising not to sue if Akiva got sick. No, I wouldn't change my mind about the accommodations he needed. And no, Akiva couldn't come back.

At home, Akiva wandered around wearing his backpack. "Am I going to peeschool today?"

"No, not today," I said, and he crumpled.

Shai was indignant. "They should *listen* to you," he insisted, "and be doing teams with you."

I heard Judi's echo in Shai's words, and hugged my little hemophilia advocate. Then I found my nicest shirt, my sheaf of papers, and walked into yet another room, in another preschool. "Let me tell you about my son, Akiva," I told the staff in that room. And oh, but they listened.

1. Wrightslaw (http://wrightslaw.com) is a resource for families, teachers and advocates working with children with disabilities.

2. FAAN: Food Allergy and Anaphylaxis Network (www.foodallergy.org) is a national organization for families and individuals with food allergies.

*As I See It... from page 3*

consecutive National Hemophilia Foundation Washington Days.

Since August 15, President Obama has referred to me by name several times in his speeches. The White House even sent a crew to do a short (yet highly rated and frequently viewed) video profile of our story, which can be seen on both YouTube and www.whitehouse.gov. I was also invited to sit with the first lady during the president's healthcare speech to the joint session of Congress on September 9. To this day, I maintain a dialogue on the progress of reform with high-level White House personnel, who are committed to completely eliminating the coverage barriers we face when dealing with a high-cost chronic illness.

Ultimately, my Obama introduction was a combination of "right place, right time," and having relatively few degrees of separation from the decision makers as a result of our family's previous advocacy work. I stressed the points that private insurance does not work well; that the more you need it, the less likely you are able to count on it; and that high-cost chronic conditions not only affect the immediate families, but ultimately everyone else under the same company's insurance plan.

Parts of the reform proposals are great for our community. But there are still some troublesome details. In my numerous calls and emails with White House staff since the Colorado town hall meeting, I have never pulled any punches when explaining what I don't like. The good news is that they are listening and working to improve the legislation for us. The bad news is that this will not be over in 2009, even if reform passes. We will still have a long row to hoe.

I once read that every person you talk to is a chance to change the world. Now I believe it. Each of us has a powerful story to tell. It's up to all of us to tell ours.

Nathan Wilkes is an independent consultant on data network engineering and business management, and father of Thomas, age six, who has hemophilia; Nora, age seven; and Natalie, age three. Nathan and his wife Sonji operate Headstorms, Inc., their consulting business, from Denver, Colorado.

*Inhibitor Insights... from page 4*

Another alternative is to keep a "bleed box." Parents can gather small toys, videos, and activity books to be kept separate from other toys that are played with daily. Because these items aren't often used, playing with them is a new activity that can help distract a child longer. One family gathers toys from fast-food kids' meals for the bleed box, and scours department store toy sections after the holidays for Legos, electronic handheld games, and arts and craft supplies.

Laura-Jean Siggens, clinical nurse coordinator at the University of Michigan Health System Hemophilia & Coagulation Disorders Program, urges parents to try "reading, playing music, having the child watch their favorite TV program, singing, talking; anything that works. The key is to know what your child gets mesmerized with."

## Learning to Cope

As lead nurse in a pediatrics cancer center for several years, Siggens has seen firsthand that distraction methods – murals, music, videos, bubbles – work to hold children's attention long enough to endure painful procedures. "Children are resilient and oh, so smart," says Siggens. "They are such troupers and deserve a lot of credit for learning these techniques and using them to help during treatments. Parents, too, can use them to help their children learn good coping mechanisms that they will use throughout their lives."

With a bleed successfully resolved, Hatsady has now identified things that Ryan likes to do. "He loves the Internet, especially music. Now that he's older and knows what he likes, he just points at it and we supply his desire," she laughs. Keeping the big picture in perspective, Hatsady notes, "As you live on with a child with this condition, it gets a little bit easier. It's a determination to adjust life's daily routine to something else for a bit."

Virginia Satir, noted American author and psychotherapist, summed it up: "Life is not what it's supposed to be. It's what it is. The way you cope with it is what makes the difference."[1] Inhibitor patients and families learn this lesson and reinforce it with every bleed.

1. Qtd. in Foster, Rick, Greg Hicks, MD, and Jen Seda. *Choosing Brilliant Health: 9 Choices That Redefine What It Takes to Create Lifelong Vitality and Well-Being.* p. 134.

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*the hemophilia newsletter by families & for families*

**Inside:**
Home Care, 340Bs and Insurers:
The Battle Over Your Child's Factor Dollars

## Visit Your HTC Annually!

# Parenting Moment

*There is always a moment in childhood when the door opens and lets the future in.*

GRAHAM GREENE

*There are only two lasting bequests we can hope to give our children. One of these is roots, the other, wings.*

HODDING CARTER

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