Parent Empowerment Newsletter

LA Kelley Communications, Inc.

PEN

CELEBRATING OUR 25 ANNIVERSARY

inside

Do New Prolonged Half-Life Products Mean the End of Ports?

Christine Bergeon Burns

When my first son Charlie was born with severe hemophilia B in 2011, I quickly learned to count myself lucky. He would likely need prophylactic (preventative) infusions of factor IX "only" twice weekly to maintain a 1% trough level and minimize the chance of spontaneous bleeds. This was a piece of cake compared to hemophilia A parents, who were typically infusing factor VIII prophylactically every other day, or parents of kids with inhibitors, who might be infusing daily or even several times daily.

Christine and family: Will new products change the port decision?

Christine Burgeon Burns

A year later, I felt something akin to guilt as my husband and I, along with my son's hematology team, decided to get a central venous access device (CVAD)–in particular, a port–for our son.[1] I felt a little conflicted about potentially putting my son at some risk, in the name of convenience, comfort, and control. Surely those other families were prime candidates for ports–but were we infusing frequently enough, or were his veins really troublesome enough, to warrant the device?

Thankfully, all of these insecurities quickly melted away as we learned to successfully infuse with a port at home. Then, after a couple of years of twice-weekly prophylactic infusions with almost no breakthrough bleeds, we learned of a new prolonged half-life factor IX product that promised prophylactic infusions as infrequently as once a week![2] Should we switch Charlie, who now had a port, to the new prolonged half-life product? Around the same time, we also learned that our newborn second son, Kenny, also had hemophilia. Decision time again. Will Kenny even need a port, if he uses a prolonged half-life product? How will one decision affect the other?

Prolonged half-life factor products and CVADs such as ports may decrease the burden of infusions for families with bleeding disorders. People with severe hemophilia may be able to infuse less frequently when using prolonged half-life factor, as compared to products with a normal half-life. Ports may increase success, reliability, and ease of administering factor as compared to standard peripheral access. But decisions about whether to move forward with a prolonged half-life product

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1. Central venous access device (CVAD): Medical device used for IV infusions without having to access a vein directly. CVADs include internal devices, called ports, and external devices, called central lines. Ports are surgically implanted. 2. Prolonged half-life factor products are sometimes also called "long-acting" or "extended."

welcome

Laurie Kelley

Twenty-five years ago, a young pharmaceutical product manager named Rob Partridge, working for Armour Pharmaceuticals (now part of CSL Behring), gave a young novice writer (me) an opportunity that changed my life. I had an idea for a book about hemophilia. And with funding approved by Rob's colleague at Armour, Joe Pugliese, an icon in our community, I had my big chance to publish what became *Raising a Child with Hemophilia.*

In turn, I enjoy offering members of our community the chance to publish. This is what PEN is all about: hearing your point of view, helping you put it into words, and publishing it. Almost everything we publish comes from our families. Our feature article, by Christine Bergeon Burns, suggests an unusual idea that made me think twice: What impact *do* new prolonged half-life products have on whether a child gets a port? No one I know has asked that question before. I decided to give Christy the chance to express her ideas in PEN, to our readers' benefit.

And Chris Ingram, a guy with hemophilia from Arizona who I met last December at a meeting, shared with me that he does medical insurance coding for a living: Would we like an article about what families with hemophilia should know from his point of view? Absolutely!

We love it when our readers submit articles. And how else do we involve our community? At a meeting earlier this year, Ray Dattoli, a man with hemophilia and another community icon, told us that author Paul Clement's three-part series on pain medication in Inhibitor Insights was good, but he felt we were not taking the risk of addiction seriously enough. A mom also contributed the same comment. We listened. In this issue of PEN, Paul focuses on addiction: what it is, and is not.

Thanks for sharing your feedback, readers. That feedback has the potential to become an article. Keep your ideas and comments coming!

inbox

The Circumcision Decision (PEN, Aug. 2015)

EXCELLENT ARTICLE! GREAT JOB TACKLING THIS SUBJECT. I hope everyone can read it and let boys make that decision for themselves.

Ashley Webb
California

AGAIN, YOU'VE SERVED THE COMMUNITY WITH A GOOD article, this time on circumcision. I did a lot of circs as a rotating intern on the OB service. We used the clamps and left them on 10 minutes. We were in sterile gowns and gloves and had to sit there with the baby for 10 minutes, doing nothing; there wasn't room to do a second baby while waiting for the first. It felt like a long time, just sit and wait. And more babies to circ after that.

Later, when interviewing people with hemophilia, some of whom had bled with circs and some had not (even some with severe hemophilia), I wondered whether those who'd bled had had impatient doctors who didn't wait the whole 10 minutes.

The gadget that stays on until it falls off sounds like a good choice for a baby with hemophilia.

Thanks for everything you're doing.

Carol Kasper, MD
California

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Insurance 101 Post-ACA

Christopher Ingram

Shopping for health insurance is one of the toughest decisions to make, especially when you have a bleeding condition. How do you know your needs will be covered? There's a lot of confusion when shopping for health insurance. What's a deductible? What's an out-of-pocket max? What is coinsurance?

Understanding some terms and concepts will make the process of purchasing health insurance a little easier.

First, know some key terms:

Premium. This is your "membership fee"–the amount you pay every month to have health insurance. If you don't pay your premium, you don't get the member benefits.

Deductible. This is the amount you pay out-of-pocket *before* your insurance plan will pay for anything. For example, you may have a $1,500 deductible. This means you're responsible for paying the first $1,500 of covered medical expenses[1] in a given year before your insurance will start to pay.

Coinsurance. This is the percentage of covered medical expenses that you share with your insurance *after* you meet your deductible. The most common split is 80/20. Using our $1,500 deductible example, this means that once you've met your deductible, your insurance will pay for 80% of covered medical expenses and you will be responsible for the remaining 20%. Your portion of the coinsurance contributes to your out-of-pocket max.

Out-of-Pocket Max. This is the financial limit of covered medical expenses you'll have to pay in a given year. After you hit this maximum, you will no longer have to pay for any covered medical expenses. Let's say your out-of-pocket max is $6,000, and you meet that amount. At this point, with the exception of your monthly premium, insurance companies will cover 100% of your covered healthcare expenses.

Note that the coinsurance, out-of-pocket max, and deductible each have two differing amounts, one for in-network healthcare providers and one for out-of-network. In-network costs are incurred when you use the healthcare providers listed by your insurance company. In-network providers contract with an insurance company usually by offering lower prices. If you visit an out-of-network provider, the deductible and out-of-pocket max are usually doubled, and coinsurance may go from 80/20 to 60/40.

Under some plans, some out-of-network expenses are not covered at all.

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1. Remember that under some plans, some out-of-network expenses will not be covered.

Ingram family: Summer, Elijah, Chris, and Anjelah (left to right)

sponsored by Novo Nordisk Inc.

Opiate Addiction: Fears and Facts

Paul Clement

The hemophilia community has lost several young men this past year to drug addiction and overdose, sometimes involving painkiller addiction that escalated to more deadly drugs. It's a trend seen nationally. In Utah, prescription painkiller abuse has become epidemic. In Vermont, the governor's 2014 State of the State address was devoted to his state's heroin epidemic, rooted in prescription painkiller abuse.

Our community needs painkillers, especially for people with inhibitors. Not all prescription drug use leads to addiction or heroin use. What are the risks of using painkillers? Because of the rising drug use epidemic, the national media may blur the lines between prescription painkiller use and addiction. But there are distinctions you should understand if you're worried about painkillers.

Side Effects

People with hemophilia and inhibitors typically experience more acute (short-term) pain because of repeated and prolonged bleeding into joints. They're also likely to develop hemophilic arthropathy, in which the joint cartilage is damaged, at an early age. This results in chronic (long-term) joint pain. Pain medication can help.[1]

All drugs have side effects: an overdose of acetaminophen can cause liver failure; and NSAIDs are associated with an increased risk of gastrointestinal (GI) bleeding, and reduce the blood's ability to form a clot. Opiates are the safest of the three classes of analgesics because they don't cause organ damage, as acetaminophen and NSAIDs can. And opiates do not affect the ability of the blood to clot, nor do they cause GI bleeding.[2] But opiates do have side effects, and if they are abused, an overdose can cause respiratory suppression and death.

Yet opiates are not the most popular painkiller choice of people with hemophilia. In the HERO study of 1,386 people with hemophilia from 11 countries, the most common analgesics used for treating acute or chronic pain were acetaminophen and NSAIDs.[3] Why don't more people use opiates to treat chronic pain? Sometimes, patients and physicians have misconceptions.[4]

Patients may avoid opiates because they fear side effects such as drowsiness, nausea, constipation, and mental cloudiness. Some want to save opiates for "when they really need it." Others say that pain is part of being a person with hemophilia. And many patients fear addiction.

Healthcare providers may avoid prescribing opioids because they lack knowledge of pain management strategies, worry that patients will give their drugs to someone else, or fear criminal prosecution if patients abuse opioids. Healthcare professionals also fear patient addiction.

Addiction Is Different

Misunderstanding about opioids often centers on confusing the meaning of three terms:

- tolerance
- physical dependence
- addiction

Tolerance occurs when the same dose of a drug becomes less effective over time, so the dose must be increased to be effec-

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1. This is the final article in a four-part series on painkillers for people with inibitors. 2. The three classes of analgesics are acetaminophen (brand name Tylenol) for mild to moderate acute pain; non-steroidal anti-inflammatory drugs (NSAIDs) such as ibuprofen (brand name Motrin) for mild to moderate acute pain; opiates for moderate to severe acute and chronic pain. 3. A. L. Forsyth, M. Gregory, D. Nugent, et al., "Haemophilia Experiences, Results and Opportunities (HERO) Study: Survey Methodology and Population Demographics," *Haemophilia* 20, no. 1 (Jan. 2014), 44–51. 4. Michelle Witkop, Angela Lambing, and Rhonda Fritz, *Nurse's Guide to Bleeding Disorders*, https://www.hemophilia.org/sites/default/files/document/files/Nurses-Guide-Chapter-18-Pain.pdf (accessed Oct. 14, 2015).

richard's review

Linda Weaver's Studio

Richard J. Atwood

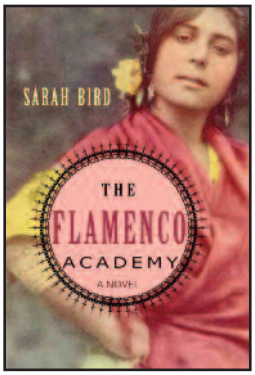
Hemophilia and Music

Who isn't moved by music? I'm a fan for sure, though as a listener not a performer. I also admire authors who accurately describe musical intricacies and sensory experiences. To make their writing more interesting, some authors include hemophilia. Combining the pleasures of literature and music, the following internationally set, musically themed novels either include characters with hemophilia or mention hemophilia.

Tomás Eloy Martínez
The Tango Singer
Bloomsbury, 2004

In 2001 an American doctoral student researching the work of Jorge Luis Borges travels to Buenos Aires, Argentina. There the student is sidetracked by an obsessive quest to hear the tango singer Julio Martel, who sings at unannounced concerts. Martel happens to have hemophilia A. He suffers from intestinal hemorrhages, liver fibrosis and necrosis, nosebleeds, arthrosis, a distended spleen, and damaged veins. His treatment is blood transfusions in a hospital. Tragically, Martel dies.

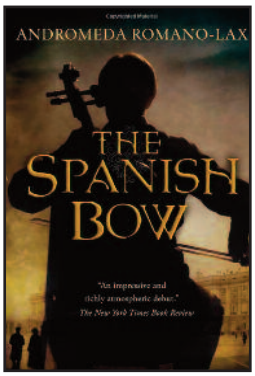
I was captivated by the sensory images of Buenos Aires and the tango in this novel; I felt I was there, listening to the music. Hemophilia is fairly accurately described. The award-winning Argentine-born author is now in exile and teaches at Rutgers University in New Jersey.



Sarah Bird
The Flamenco Academy
Alfred A Knopf, 2006

Cyndi Rae Hrncir (*Hern-shur*) attends high school in Albuquerque, New Mexico. She meets Didi Steinberg at an oncologist's office because both of their fathers are dying. Though opposites in personality, Rae and Didi cement an inseparable bond of friendship. Rae meets Tomás Montenegro, a mysterious guitarist who plays gypsy flamenco music, before he unexpectedly disappears. Didi and Rae study flamenco dancing at the University of New Mexico while Rae obsessively searches for the missing guitarist. After auditioning as a dancer, Rae goes on tour with Tomás, who has unexpectedly returned. Later, Rae discovers that Tomás and Didi are lovers, but Tomás really loves Rae. The love triangle in this coming-of-age novel is intense. Describing his Roma heritage in Spain and America, Tomás says, "Like I had hemophilia, something in my blood that made me special but was a curse."

Although King Alfonso and Queen Eugenia of Spain are mentioned in the novel, I found it odd that their two hemophilic sons are not. The author lives in Texas.



Andromeda Romano-Lax
The Spanish Bow
Harcourt, 2007

Feliu Anibal Delargo Domenech was born in 1892 to musical parents in a Catalan town in Spain. With his hip dysplasia, Feliu suffers lifelong pain and a limp. At age six, after his father dies, Feliu is bequeathed a cello bow. Despite the family's poverty, Feliu is sent to Barcelona to study cello. As a young musical prodigy, Feliu plays his cello for and tutors Queen Victoria Eugenia, or Ena, a hemophilia carrier. He later tours Europe and America as a maestro cellist. When Spain erupts in civil war in the 1930s, Feliu takes a political stand by refusing to play for fascist leaders Franco and Hitler. He narrowly escapes to Cuba in 1940.

This fictional memoir is more about the cello than the protagonist. Hemophilia is not part of the plot, but the first son of King Alfonso XIII

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To Port or Not?

Laurie Kelley

There was a time in the US when we had no prophylaxis for hemophilia…so ports were rarely used except for immune tolerance therapy (ITT) for inhibitors. After a 25-year study in Sweden was published in 1992 demonstrating the positive effect on joints of primary prophylaxis,[1] "prophy" became the standard for hemophilia treatment, aiming to preserve joints by preventing most spontaneous joint bleeds. Prophy's frequent infusions meant more wear and tear on a child's veins and more stress on parents while attempting to hit a vein on the first try. This led HTCs to seek alternatives to peripheral venipuncture. Enter the port.[2]

A port is a surgically implanted device, typically in the chest wall just under the skin, that offers a larger target compared to sticking a small vein. A port consists of a drum-like device (reservoir) with a silicone top into which the needle is inserted. The port is connected by a catheter (tube) to a large vein.

Now, with the advent of prolonged half-life products (see our feature article), parents are wondering, *Do we really still need ports?*

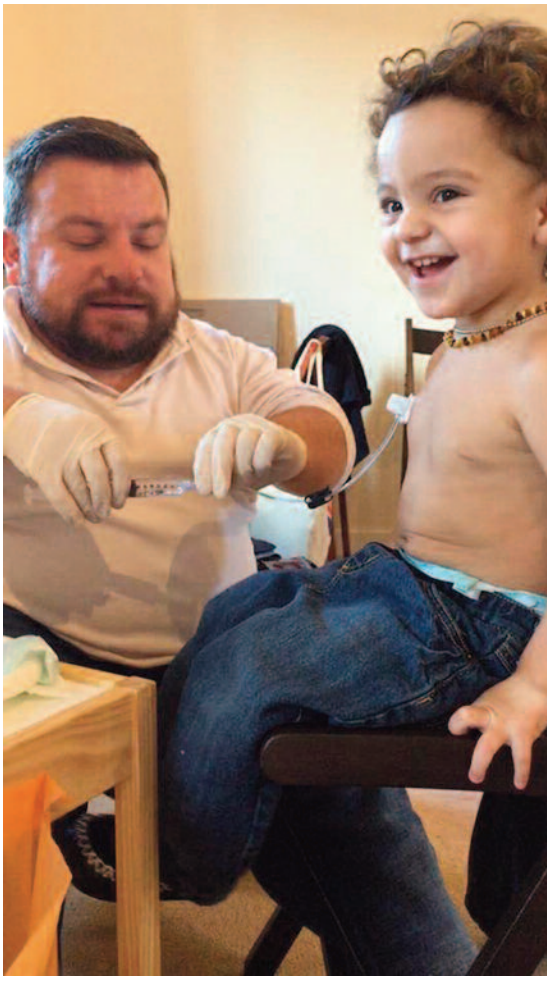
How to Decide?

The decision to insert a port is not a simple one. Though the surgery itself is outpatient and takes less than an hour, there are also risks associated with ports that should be discussed when deciding whether to get a port. You'll need to learn and then recognize the symptoms of complications, including infection, skin breakdown, and blood clots.[3]

Your HTC team will explain the benefits and risks of having a port. But ultimately, it's YOU who decides whether to place a port in your child. And it often helps to hear about the experiences of other parents. So we asked a group of parents these questions: Who suggested you implant a port? What made you decide to use one? Did it meet your expectations? Were there any complications? And was it removed?

Usually parents decide on a port for specific reasons:

Target joints. These are joints that suffer repeated bleeds. To break the cycle of bleeding, a child may be placed on prophy. Even if your child has great veins, your HTC team may suggest a port for a few years. Melissa Howell reports, "My son was two years old and suffering bleed after bleed from an ankle trauma. Our hematologist suggested a port and gave a small demonstration of how one works. I was on board! My son had his port for nearly five years without one ounce of trouble!" Lindsey Eberhardt agrees. "My oldest son, now eight, wasn't diagnosed until he was five with moderate hemophilia. He was having joint bleed after bleed, probably every other week. Our doctor suggested a port placement and showed us how they work. After a little discussion with my son, we were on board! He has had his port now for a little over a year. It's much easier to access his port for prophy three times a week or when he does have a breakthrough bleed."



Gabriela Blasquez

Ports help save wear and tear on veins from frequent infusions

Inhibitors. People with inhibitors often need many infusions, depending on whether a child is undergoing ITT or using NovoSeven (which requires infusions every two hours). Cristelee Reynolds-Peake recalls, "When Seth was two, he developed an inhibitor, and his hematologist decided that he needed a port. His veins were also very bad. So we really didn't have a choice."

Poor veins. Some children have veins that roll away, are hard to find under plump layers, or are just too tiny. Liz Purvis had her son's port placed at five months to continue the dosing for his

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1. Primary prophylaxis is usually defined as prophylaxis started after the first major joint bleed and before age three. 2. Ports are one type of CVAD. CVADs consist of a small reservoir and flexible tubes (catheters) that are threaded into large veins; they allow for infusions without venipuncture. There are external CVADs and internal CVADs. Internal CVADs are called ports. 3. See Laureen A. Kelley, *Raising a Child with Hemophilia*, ed. 5 (LA Kelley Communications, 2015, in press), chapt. 7, for a complete review of complications of ports.

Marquis family

A port is barely noticeable as a bump on Gage's chest.

and/or a port involve a variety of considerations, because each choice may provide potential challenges as well as benefits. How do you, as a parent, decide? Does the availability of prolonged half-life factor products tilt the scales when it comes to making the port decision for your child? Does already having a port tilt the scales when it comes to deciding whether to switch your child to prolonged half-life factor?

Ports and Prolonged Half-life Factor: The Basics

Most parents of children with hemophilia are aware that prophylaxis is an important part of ensuring the long-term well-being of our children. Prophylactic infusions can decrease spontaneous bleeds, minimize likelihood of long-term joint damage from repeated bleeds, and reduce acute and chronic pain. Prophylaxis also provides some (if often low-level) protection in case of an accident or injury. It can make a world of difference in parents' peace of mind. With fewer visits to the hospital and greater ability to participate in physical activities, kids with hemophilia on "prophy" often lead very normal lives.

But the frequent infusions prophy requires can be demanding for families with hemophilia, and in some cases may even deter people from undertaking prophy. Plus, some children metabolize factor faster than average, and others are battling inhibitors (antibodies produced to destroy factor, which the immune system perceives as foreign). For these families, immune tolerance therapy (ITT) or prophylaxis can mean infusing daily, or even multiple times per day. Even when on prophy, most kids with hemophilia will still have occasional breakthrough bleeding. It's clear that a wide spectrum of infusion patterns exists, making the choices of factor product and route of administration key–and sometimes complicated–decisions for parents.

A port implanted under the skin provides a larger and more resilient target for repeated needlesticks than a peripheral vein. For children with particularly difficult veins, this may be the only way to reliably administer factor. For other families, infusion is possible without a port: the child may have prominent veins, and a parent or nurse is skilled at "sticking." However, a port may still be beneficial because it decreases the trauma of home infusions, offers freedom from after-hours ER visits when help is needed, or simply preserves a young child's veins for when he's older and ready to self-infuse. Risks include port infections, which can be deadly if not treated early; port blockage due to thrombosis (blood clots); and mechanical failure of the port, such as the port catheter pulling out of the port. Whether the benefits of a CVAD such as a port outweigh the known risks is specific to each patient, and should be decided in conjunction with the child's HTC team (see box 1).

Prolonged half-life factor products are relatively new on the hemophilia scene. They're considered an exciting devel-

1 Discuss these questions with your HTC when deciding whether to place a CVAD:

- How hard is peripheral access to learn? Does my child have hard-to-access veins?
- What is a CVAD? Which ones are available? How do they work?
- How often is access required (for bleeds, prophylaxis, inhibitor)? Are we candidates?
- What will surgery involve?
- What is the risk of infection? Risk of thrombosis (blood clots)? Are there surgical risks?
- What does the surgery cost? How much will be covered by insurance? What will our copay be?
- Can we maintain a prophylactic regimen with a port? Will we commit to strict aseptic technique when using a port?

opment by many patients and HTCs, but may not yet be fully trusted, understood, or embraced by those who take a wait-and-see approach. For some families, the difference between infusing factor IX once every 7 to 10 days for prophylaxis, versus twice weekly, can be a true game changer.

Yet not all prolonged half-life products are equal: current prolonged half-life factor VIII products do not prolong the half-life as dramatically as do factor IX products. Also, individual patients may respond differently from the reported averages, and children may need higher or more frequent dosing than adults.

What Influences What?

Today, parents of young children with hemophilia may be in a unique predicament, as they try to formulate a plan to undertake prophylaxis for the first time. One new mom's son is crawling and will soon learn to walk; he's had extensive bruising. Her HTC team did not recommend starting her son on the new prolonged half-life product until he'd had 50 exposures to the standard product the team had been prescribing for years. Yet this mom was beginning to suspect that those 50 exposures might prove difficult to infuse and might warrant a port. Should they postpone implanting a port on the promise of a product they hadn't yet tried? Or get the port now, continue with her son's current standard product, try the prolonged half-life products after the 50 exposure days…and wonder later if he ever needed a port? This decision is likely to disappear if families are offered–and accept–these products from the onset as options for their children's treatment.

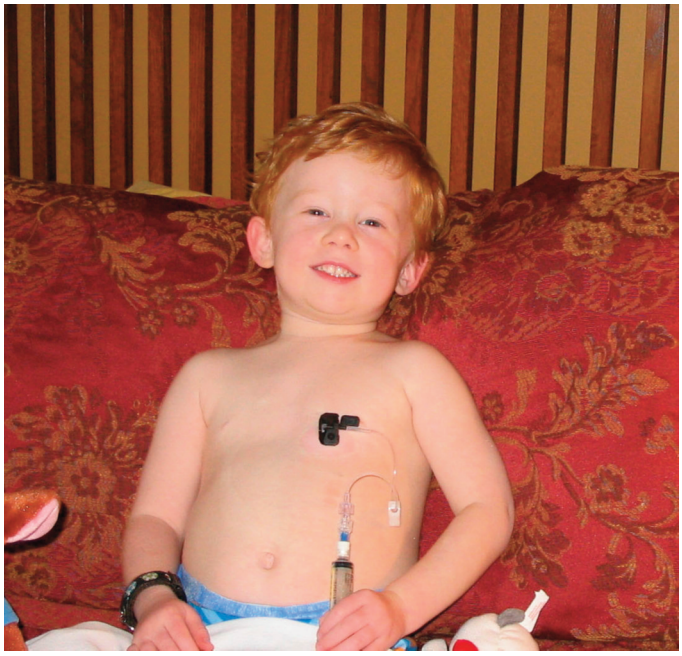
In the age of prolonged half-life products, some parents may feel that the risk presented by a port outweighs the benefits for their child. Mary Dunleavy,* mother of an eight-year-old boy with hemophilia, wishes prolonged half-life had been available when her son began prophy. "While I appreciate how lucky we were to have a [port], I still would have preferred not to need one in the first place. I watched my son go through many port infections, and I think they were actually worse than bleeding episodes." Indeed, a port infusion isn't a walk in the park; it's still a needlestick, and parents must learn aseptic technique and be willing to accept the risks on behalf of their child. Some find it hard to shoulder these responsibilities.

But in some cases, infusions may be so challenging that fewer infusions on a prolonged half-life product wouldn't make much difference in the decision to use a port. Bonnie Culver explains, "My son needed a central line due to an inhibitor, and all his veins were blown. So for us, it was necessary. Product half-life was not a concern." For Carmen Langdon, it's all about location. "We would still get ports for

* Names have been changed for anonymity.

2 Discuss these questions with your HTC when deciding whether to switch products:

- What factor products are available? What are the new prolonged half-life products? How do they work? How would they be dosed?
- What is the reported bleeding rate, and the expected recovery?
- What side effects have been reported?
- What does the new product cost per unit? Will insurance cover it?
- How different would our infusion schedule be on one product versus another?
- Will switching products mean fewer or more visits to the HTC? Will it mean recovery studies?



Ports provide great benefits but also have risks

our two boys [regardless of whether prolonged half-life factor were available]. We live in a remote community and can't rely on IV access being achieved here."

Even when vein access is feasible, the risks and benefits of a port may not be so different with the availability of prolonged half-life products. Fewer weekly infusions might mean less need for a port; but fewer port accesses should theoretically mean fewer chances for the port to become infected (though perhaps not for clotting complications). Some healthcare professionals feel that the port simply improves families' quality of life, regardless of frequency of infusion. "It's not just sticking veins that is hard," says Kate Khair, a nurse at Great Ormond Street Hospital for Children in London. "Ports give families their freedom back–and control over hemophilia."

You Have a Port: Would You Try a Prolonged Half-life Product?

Many parents of children who have ports are interested in newly available prolonged half-life products because they want the port removed. Janet Diebley Rider, mother of an eight-year-old with hemophilia, states that her family's goal is "to keep our port until we have comfortably switched to a longer half-life product and know that it is working well." She predicts that they may have the port removed sooner, due to the availability of prolonged half-life products. Yet, she adds, "A part of me likes the port not only as a backup but to save his veins...but the other part wants the port out now."

Similarly, Sara Sotengco, mother of a four-year-old with hemophilia, decided against a second port after her son's first port was removed because of infection. Her decision was based largely on the availability of prolonged half-life products. "I didn't think it was necessary, since he would only have once-a-week infusions. I couldn't justify the surgery." But, she adds, "He had horrible veins when he was a baby. If he were any younger, we would have gotten another port!"

Other parents may see prolonged half-life products as a way to make life a little easier, regardless of their child's port. As Miranda Shafer puts it, "Having a port didn't affect our decision to use longer-lasting factor. Whether we were sticking in a vein or in a port, it would be less sticks with twins who both have severe hemophilia A." Vanessa Stowers Flora, mother of two boys with severe hemophilia B, agrees: "We switched so that our family has more flexibility, less pokes in general. But I still want the port to keep happy veins."

Still, some families are happy with their current factor product, perhaps feeling, "If it's not broken, don't fix it." That is, if the infusion routine is already well established, some parents may not be interested in trying a new factor product, regardless of half-life or port. Judy Miller* falls into this camp. Her son with severe hemophilia A metabolizes factor quickly, minimizing the modest potential benefit of a prolonged half-life factor VIII product. But he has also expe-

rienced past health issues that left Judy wary of unnecessary changes to his care. "We are comfortable with what is working."

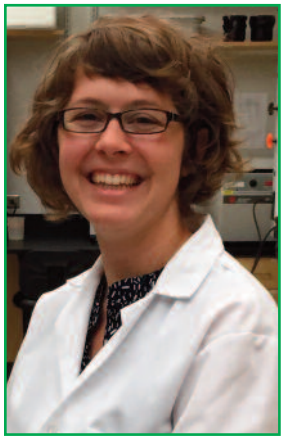
The Verdict?

It's clear that there's no single formula for how to approach prolonged half-life products and port use. These decisions are highly individual, often depending on variables specific to a particular patient, family, or HTC team. Different HTCs may follow differing courses when it comes to prolonged half-life products and ports. Some may take a more conservative approach, preferring peripheral venous access and factor products they have prescribed for years. Others may encourage ports for some patients, or be actively engaged in clinical trials with new factor products in the pipeline.

Significantly, HTCs may vary in how much input they seek from families when it comes to making decisions. Parents shouldn't hesitate to speak up and take an active role in the healthcare of their children. This is crucial if the family is interested in exploring a course of treatment that may not line up with the initial recommendations of their child's HTC team. As I've described in boxes 1 and 2, weighing the risks and benefits of a port or prolonged half-life product often involves awareness of lifestyle variables, in addition to medical considerations. An informed discussion of the reasoning behind the HTC's recommendations, along with the concerns and desires of the family, should result in a personalized care plan that is in the best interest of the child.

And my own family? We approached our port and product decisions with open minds for our second son, choosing prolonged half-life factor to minimize frequency of infusions, and exploring regular peripheral infusions at the HTC first to see whether we could transition to doing them at home. But having a preschooler with a port ultimately weighed heavily on our decision to also have a port placed in our toddler. We were struggling with missed work because of frequent HTC visits, and we were eager to have a unified treatment approach for both boys. We were also familiar with the port infusion routine at home, had never had infection problems, and our HTC team was on board. A port made the most sense for our lifestyle.

At the time of this writing, we're a month post-op and have successfully completed a couple of weeks bleed-free, doing extended half-life prophy at home. We are once again counting ourselves lucky. Still, we look forward to ongoing advances that decrease the burden of frequent infusions for all families affected with bleeding disorders–advances that ultimately point us toward a cure. As Mary puts it, "I am just grateful that we live in an exciting age of innovation and discovery, and that the medical world evolves at the rapid rate that it does."



Christy Bergeon Burns lives in Bloomington, Indiana, with her husband Steve and their two sons, Charlie (4) and Kenny (1), who both have severe hemophilia B. Christy holds an undergraduate degree in biopsychology from the University of Michigan and a doctorate in ecology, evolution, and behavior from Indiana University, where she is currently the director of a core animal behavior research laboratory.

Inhibitor Insights... from page 4

tive. With opioids, this can be done several times because unlike NSAIDs and acetaminophen, opioids have no ceiling dose (an upper dosage limit where no additional benefits are seen). When you stop using an opioid, tolerance will decrease and eventually disappear, but tolerance to that opioid may continue for months to several years after you stop using the drug. *But tolerance is not addiction.*

Physical dependence means you will experience withdrawal symptoms–which often mimic the flu–if the drug is suddenly stopped or the dose is lowered too quickly. Anyone on opioids for more than several weeks will be dependent to some degree. Physical dependence is often confused with addiction, but *dependence isn't addiction.*

Addiction is a medical diagnosis, characterized by behaviors that include impaired control over drug use, compulsive use, continued use despite harm, and craving for the drug. Eventually, addiction always harms quality of life.

Many patients fear opioids because they fear addiction, but several large retrospective studies have determined that opioid addiction among chronic pain sufferers is uncommon, with only about 2% becoming addicted.[5] Of those who become addicted, many share similar characteristics. People are at higher risk of addiction if they have previously abused drugs or alcohol, have a family history of drug or alcohol abuse, suffer from depression, have bipolar disorder, or show symptoms of post-traumatic stress disorder (PTSD, which is believed to often affect children with inhibitors).

What Does This Mean for You?

People with chronic medical conditions, such as bleeding disorders, have a higher incidence of depression or anxiety than the general population and may be at higher risk of addiction.

If you have inhibitors, you may need opiates to help manage chronic pain at some time. Opiates will not eliminate *all* pain, nor should that be your goal. Instead, you should aim to reduce pain to a manageable level so you can function normally and be able to work, attend school, or get a good night's sleep. Negotiate a treatment plan with your HTC–ideally with a pain specialist–that balances pain relief, functional improvement, and medication side effects.

Reducing Addiction Risk

There are things you can do to significantly reduce the small chance that you or your child will become addicted to opiates.

1. Know the signs of drug abuse (see box). Several signs overlap with depression. You'll need to determine what are signs of normal teenage angst in your child, what might be depression or bipolar disorder, and what might be drug abuse.
2. Be alert when the risk of opiate abuse is greatest. Ray Dattoli, who has hemophilia, suggests that when teens enter high school or college, the risk of drug abuse is higher. Boys with low self-esteem and few friends may be at greatest risk of abusing drugs because they are willing to do almost anything to feel part of the group, including sharing their drugs or selling them for recreational use.
3. Remember that 70% of children who abuse prescription drugs obtain them from family or friends. Help prevent drug abuse by keeping all prescription medications–especially opioids–in one location in your home. Store them in a combination safe, locked cabinet, or locked drawer–not your bathroom medicine cabinet.
4. Inventory your prescription medications regularly, and more often if you suspect abuse. Dispose of expired meds.
5. Help your child develop positive self-esteem. Matthew Porter, a young man with inhibitors, singles out summer camp as one of the most influential experiences that improved his self-image. He developed a circle of friends who provided support outside of camp. And for him, learning to infuse at camp was empowering.

Drug abuse stemming from pain medication is a huge problem in the US today. But for people with hemophilia and inhibitors, opiates are a valuable tool in managing moderate to severe chronic pain. You now know that drug dependency is not addiction, and that even people at high risk of addiction can use opiates safely with proper safeguards and supervision. Even so, be alert to signs of drug abuse. And don't let misinformation or fear prevent you from getting the pain treatment you need.

Signs of potential drug abuse

- Increasing isolation and withdrawal from family and loved ones
- Behavior changes or extreme alterations in mood, such as frequent hostility, anger, anxiety, or agitation
- Deceitful or illegal behaviors to obtain additional prescriptions or greater quantities of the drug
- Decline in overall performance in work, school, or social life
- Frequently nodding off in inappropriate circumstances
- Frequent complaints of physical aliments: cramping, diarrhea, itchy skin, joint and muscle pain, nausea and vomiting, anxiety, insomnia, or headaches
- Neglect of personal hygiene, or ill-looking appearance
- Changes in eating or sleeping habits
- Ongoing confusion or disorientation
- Low self-esteem or guilt
- Persistent boredom or low energy

5. David A. Fishbain, Brandly Cole, et al., "What Percentage of Chronic Nonmalignant Pain Patients Exposed to Chronic Opioid Analgesic Therapy Develop Abuse/Addiction and/or Aberrant Drug-Related Behaviors? A Structured Evidence-Based Review," *Pain Medicine 9*, no. 4 (May–June 2008), 444–59.

As I See It... from page 3

Don't think that just because you have coverage, it applies to any service, any hospital, any physician. Before you incur a medical expense–drug, medical procedure, or service–make sure it's covered by your insurance policy! Check to see that it's in-network. Otherwise, you could be responsible for 100% of the medical expenses.

Going out-of-network can get really expensive when you order factor, which may be the bulk of your costs. When you're in the market for health insurance, ask the insurance company about its formulary, which will tell you which brands are covered. Then contact the specialty pharmacy you use or prefer–the one that delivers your factor–and ask, "Are you contracted with this health insurance company?" Then ask your insurance company, "Do you have your own specialty pharmacy? Am I allowed to choose one?"

Another way to monitor your out-of-pocket costs is to find out if your claim for factor will be processed under the medical benefit or the pharmacy benefit. This is extremely important. Why? There's an out-of-pocket max on the medical benefit–that's good for you! But there may not be an out-of-pocket max on the pharmacy benefit. Let's say, for example, that for pharmacy services your coinsurance is 50%. This means that when you order factor, you are responsible for 50% of the cost of factor. So if your factor costs $50,000 a month, you'll be responsible for paying $25,000 per month for the whole year! Impossible. But if your factor is processed under the medical benefit (where there is a max), then using the 80/20 split, your factor is $50,000 a month and you're responsible for 20%, or $10,000. But because you have an out-of-pocket max of $6,000, you are only responsible for $6,000. You won't have to pay any more than that for the rest of the year.[2]

You're probably thinking, There's no way I can afford $6,000 out-of-pocket! Most people will have trouble paying that much. Here are the most important things you can do today to keep costs as low as possible and to prepare for paying higher out-of-pocket costs:

1. Know the key health insurance terms.
2. Know which side your factor is covered on: medical or pharmacy.
3. Read your insurance policy carefully every year.
4. Budget your disposable income. Create a budget so you can estimate your expenses ahead of time and not be caught short.

There's also help available. Contact your factor manufacturer and register for its program. Most manufacturers have copay assistance programs that will pay up to $12,000 of your deductible and out-of-pocket max. And patient assistance programs exist to help you obtain free factor when you're between jobs and don't have insurance coverage. There are also free trial offers of various factor brands. For a comprehensive list of assistance programs with contact information, download a copy of National Hemophilia Foundation's "Patient Assistance Programs" PDF.[3]

Or contact Patient Services, Inc. (PSI), which may cover qualified patients' premiums when they lose their insurance.[4]

I hope I've cleared up some of the confusion about shopping for health insurance. It's important to know this information when you have to start a new plan. Insurance companies exist to sell you insurance, and they don't always know your needs. Don't let them pressure you to purchase. Do your research first, and make an informed decision. Check with your HTC social worker before signing up. Make them *sell* to you. Make them *prove* why their coverage will be the best.

If you have questions or would like assistance in talking to your insurance company, feel free to contact me.

Chris Ingram is a certified professional coder (CPC) who currently works at UnisLink. He's worked as a medical biller and coder for the following medical specialties: anesthesiology, urgent care, pain management, gastroenterology, internal medicine, pediatrics, ob/gyn, orthopedics, and trauma surgeons. He has also taught medical billing and coding at two colleges. In his spare time, Chris loves attending car shows and doing car detailing for family and friends. Chris has severe hemophilia A and lives in Arizona. Contact Chris at ichris2001@hotmail.com.

2. More providers are switching from medical to pharmacy to cut costs. According to an article in *Managed Care Magazine*, between 2011 and 2013, the percentage of plans switching from medical to pharmacy rose by 6%, from 43% from 37%. http://www.managedcaremag.com/archives/2015/1/should-specialty-drugs-be-shifted-medical-pharmacy-benefit. 3. http://www.hemophilia.org/Advocacy-Healthcare-Coverage/Key-Advocacy-Issues-and-Materials/Fact-Sheets/NHF-Summary-Of-Patient-Assistance-Programs. 4. www.uneedpsi.org.

YOU... from page 6

head bleed: twice-a-day infusions for two years, and then once a day for two years. "My son will always need a port. He had a stroke. The veins on his left side aren't any good, and he can't use the left hand to infuse the right." Another mom, Joy Stein Fitzgerald, adds, "When it takes the pros 22 sticks plus an ultrasound to get a vein, you place a port."

Quality of life. Hope Thacker's three children with hemophilia had so many bleeds that treating on demand required weekly ER visits. "All three of our sons now have ports, and began prophy at 19, 22, and 14 months, respectively. I just couldn't go through the five to six tries it took for a single infusion on a regular basis. Our HTC encouraged the port placement, but also gave us facts and options so that we could decide." Abby Naumann faced the same decision. "My son was five months old and having regular bleeds. He needed a port to help us infuse at home and empower us to give him factor instead of driving to the hospital two or three times each week." Lori Morgan reports, "My son had only one good vein. We would go to the local ER for infusions. One evening we were on our seventh stick, and still no luck. Kyle and I were both in tears. The ER staff, who are like family, were just as upset and suggested a port. And though the surgery was scary and learning was a little intimidating...it was the best decision! We were able to treat at home. Our life was more normal."

Falling out of Favor?

Are HTCs distancing themselves from port placements these days? Jane Forbes, registered nurse and mother of four boys with hemophilia, believes that an emerging trend among some HTCs is to avoid central venous access devices (CVADs) and initiate peripheral infusions. Some parents are pleased about this, and some are not.

Cazandra MacDonald says that after five years of having a port, "the HTC seemed to all of a sudden want ports out if not completely necessary. The problem was that they did not prepare our son (or us) for peripheral sticks before the removal. That was a nightmare."

Christa Cecilia Parra chimes in, "My son got his port at 18 months and I had to fight to get it. I didn't want to hear anything about [risk of infection]. All I cared about was making sure we didn't end up in the hospital all the time when my baby was learning to walk."

Lurking Dangers

Understand the risks of ports before you make the decision to get one. Sepsis–a bloodstream infection originating from the port–is among the most serious. According to pediatrician Janna Journeycake, up to 30% of children with ports develop at least one bloodstream infection. Her research indicates the infection rate climbs as high as 50% in those who develop an inhibitor.[4] Parents should be aware of the significant risk of infections, warns Jane Forbes. If not stopped early, port infections can be life-threatening. Also, many port infections can't be cleared without removing the port.

When you make a decision to have a port implanted, be sure your HTC has fully explained all the risks: surgery complications, infection, blood clots, skin breakdown (ports can pop through the skin!), and mechanical failure. To make an informed decision, you should understand the symptoms and signs of each complication before agreeing to a port.

Deena Lipinski has mixed feelings: "We've had three ports implanted. I love them, but hate them. You name the complication, we've had it, including the worst: a massive blood clot formed at the end of the line and sepsis. I don't know how I would have survived without Tyler's [port] when he was a baby, but some of our worst times were as a result."

Steph Reitberger adds, "Our first port had to be replaced two weeks in, when the incision site opened while we were infusing. We were stunned but knew there weren't better options for us, so we opted for a second port."

"Natural" Ports

Are there other options? Jane is a big fan of what she calls "natural" ports: peripheral sticks. One of her children had a port, one did not. "As a nurse, I see two-year-olds sit for peripheral infusions and not bat an eye. A lot of what happens during an infusion has to do with the parent's response: if you show fear, anxiety, it will reflect in your child. If you have to break down, don't do it in front of your child."

She worries about mothers who feel pressured by others to get a port, particularly on Facebook. "So many mothers seem to be using ports, and not peripherals. One mom was getting nervous that she was traumatizing her son by giving him peripherals!"

Jane Forbes

The Forbes brothers grew up using "natural ports"—peripheral veins

4. M. V. Ragni, J. M. Journeycake, and D. J. Brambilla, "Tissue Plasminogen Activator to Prevent Central Venous Access Device Infections: A Systematic Review of Central Venous Access Catheter Thrombosis, Infection and Thromboprophylaxis," *Haemophilia* 14, no.1 (Jan. 2008), 30–38.

To ensure better results with peripherals, advises Jane, prepare your child: Before an infusion, keep your child warm, to increase circulation to the extremities. Have him squeeze a rubber ball to pump up the veins. Make sure he is well hydrated and relaxed (stress causes veins to constrict, making infusions harder). In between infusions, exercises like swimming can improve circulation and muscle development to facilitate venous access. And keep your child's weight down. Jane notes, "It's not uncommon in the hemophilia community to find people who have used the same vein for 10, 20, even 25 years. Some even use the same site multiple times each day."

Outcomes

Despite the risks, many parents report their child has had the same port for years. Eventually, ports will need to be removed. Transitioning to a peripheral needlestick may be difficult for boys who never learned how to stick. So if your child has a port, send him to hemophilia camp where he can learn how to stick himself! Knowing how to infuse is a valuable skill because sooner or later, his port will need to be removed.

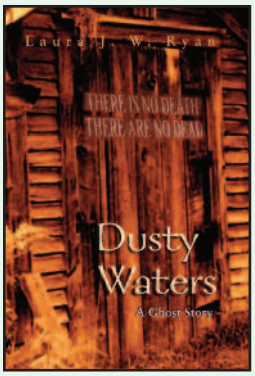
Jessica Hurtado confides, "I've heard horror stories about port infections and malfunctions, but we've had zero complications. I'm glad I get to save my son's veins and joints and keep him bleed-free. That makes it worth it to me." Melissa adds, "As a hematology/oncology nurse and a 'hemo' mom, I am a huge supporter of ports!" Lindsey believes, "This is one of the best decisions we have made for him yet!"

Cristelee notes that not every port story has a happy ending: "After 17 months Seth got a very bad infection that we couldn't clear, and during treatment he became allergic to most antibiotics. Ports are now too dangerous for Seth." But Abby sums up the overall response of most parents: "It was a great decision. I would never go back if I had a choice. Instead of [our] holding him down and listening to screaming, he giggles and helps us push the factor in. It was life changing for our son and whole family."

In making your decision, remember that there are options. You can do peripheral sticks; a whole generation of men with hemophilia are living proof of that, because they didn't have the choice. But don't sacrifice your child's joints or your mental health if you can't do a peripheral. Ports are a great option when you need them. It's up to YOU to decide!

Richard's Review... from page 5

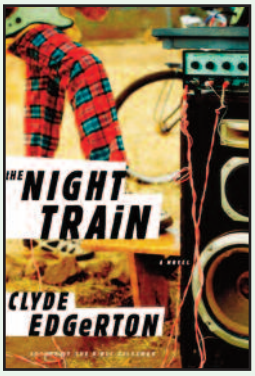
and Queen Ena is mentioned for nearly dying from his circumcision due to his hemophilia. The author, who listens to cello music while writing, lives in Alaska.



Laura J. W. Ryan
Dusty Waters: A Ghost Story
Field Stone Press, 2007

Dusty Waters sings her own political and personal songs that tell stories. After 20 years on tour, Dusty returns in 2004 to her supposedly haunted homestead in upstate New York, where she tapes her biography. Like other women in her family, Dusty can sense, see, hear, and even talk to familial restless spirits. When she was in second grade, Dusty met Emmett James, or Em, who has hemophilia. Dusty and Em were inseparable for the next 10 years, and their friendship turned into first love. Tragically, Em died in the summer of 1978 before their senior year of high school, but his ghost remains. Dusty interacts with Em's presence because of their eternal love.

Told in memory flashbacks, this coming-of-age first-love story is tragic, but hemophilia is accurately described for the 1970s with foot bleeds, hematuria, and hospitalizations. The text includes lyrics for several of Dusty's songs. The author lives in upstate New York.



Clyde Edgerton
The Night Train
Little, Brown and Company, 2011

In the summer of 1963 in eastern North Carolina, the passion for music dissolves the racial divide for two teenagers. One of them, 16-year-old Larry Nolan, known as Larry Lime, has a mentor, a jazz musician called the Bleeder because he has hemophilia. Bleeder teaches Larry how to listen to musical notes by seeing their colors and feeling their shapes.

This coming-of-age novel has a simple agenda for the adolescent protagonists: discovering music that will continually influence their lives. The award-winning author teaches at University of North Carolina at Wilmington. (See PEN, Feb. 2012, for a full review of this novel.)

It's hard to find any novel that combines a musical story and hemophilia, though unfortunately, not all of these novels fully develop characters with hemophilia. I wish that these music-themed novels included soundtracks because I was curious about the different musical styles. Still, the stories are worth reading to appreciate and enjoy the universality of music and to learn a little about hemophilia.

headlines

science

Gene Therapy News

The US FDA accepted Dimension Therapeutics, Inc.'s investigational new drug application and granted orphan drug designation for DTX101 for treating hemophilia B. Created in collaboration with Bayer HealthCare, DTX101 is designed to deliver factor IX gene expression in patients with hemophilia B. Preclinical studies completed to date indicate DTX101 has the potential to be a well-tolerated, effective therapy for hemophilia B. Clinical trials of DTX101 are scheduled to begin by the end of 2015. Why this matters: Dimension Therapeutics has given no specifics about its investigational gene therapy treatment for hemophilia B, but FDA approval of this therapy represents another step in our search for a cure for hemophilia.

For info: www.dimensiontx.com

payer

Land of the Giants

Insurer Anthem has agreed to acquire Cigna in a $54 billion deal, scheduled in the second half of 2016 if it passes state regulatory approvals. The new merged company would cover 53 million members. The merger would leave only three major players in the insurance industry. Aetna struck a deal to buy Humana for $37 billion, which would cover 33 million members. United Health just completed its own $12.8 billion acquisition of Catamaran, a pharmacy benefits manager (PBM). Why this matters: Continued consolidation of insurers could give them more negotiating power over hospitals and pharmacies, and could mean fewer healthcare provider and prescription options for consumers.

For info: cigna.com

patient resources

Job Hunting 101

Novo Nordisk is offering free career counseling to people aged 18 to 65 with hemophilia and other bleeding disorders, and their caregivers. The program is a two-hour time commitment split up over three phone calls/Skype sessions with a career counselor to help with resumes and interview skills. Why this matters: Your chances of securing a job are better with assistance from experts who can help you focus and refine your search and skills.

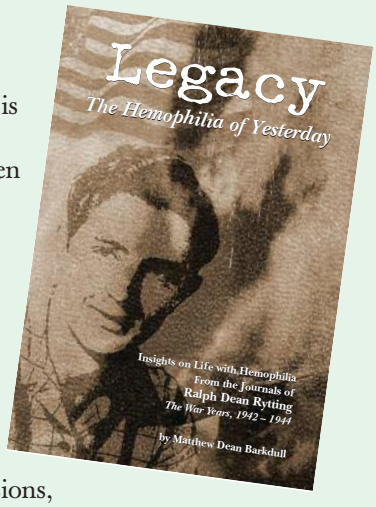
For info: www.mynovosecure.com/support/set_your_goals.html

Play Therapy with Pups!

The Nava Dog Therapeutic Play Kit is a free educational kit that lets kids with hemophilia pretend they're special agents assigned to Mission Infusion: Operation Stop Bleeds. The kit includes activity books with puzzles and games, an infusion log with stickers, a stuffed Nava dog, and play medical supplies like a port for pretend infusions. Why this matters: Children often learn best by playing with hands-on materials.

For info: nava.baxalta.com

Leaving a Legacy

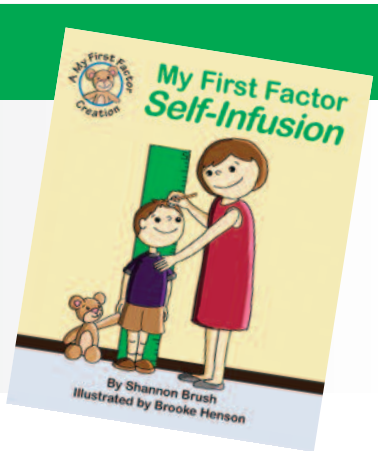


Legacy: The Hemophilia of Yesterday is a compilation of the published journals of Ralph Rytting between 1942 and 1944, at the height of World War II. Rytting was diagnosed with hemophilia at age 16 in 1943. Cryoprecipitate, the first successful treatment for hemophilia, would not be developed until 1964. In an effort to treat his frequent bleeds, Rytting received over 3,500 blood and plasma transfusions, which were mostly ineffective and carried the risk of circulatory overload and death. The exquisitely written journal entries illustrate Rytting's sufferings, his perspectives on a war-torn world, his unwavering patriotism, and his brilliant mind, but also his desire to persevere, love, dream, have a family, and make a difference. Why this matters: *Legacy* has historical significance as possibly the earliest journal by a person with hemophilia.

For info: amazon.com

news from LA Kelley Communications

My First Factor: Self-Infusion



The 10th toddler book in the My First Factor series helps a toddler know that one day, just as he will tie his shoes and dress himself, he will also self-infuse. Colorful illustrations in a chunky board book make this an attractive addition to this series–your child's first set of hemophilia books.

For info: www.kelleycom.com

soundbites

Belgian biotechnology firm **Apitope** received orphan drug designation from the FDA for its preclinical-stage product candidate, ATX-F8-117, for preventing and treating inhibitors in hemophilia A patients with inhibitors or at risk for developing inhibitors.

The FDA has approved revised product labeling for Octapharma's **wilate** (von Willebrand Factor/Coagulation Factor VIII Complex [Human]) to include prevention of excessive bleeding during and after minor and major surgery in adult and pediatric VWD patients.

Emergent BioSolutions announced that it will pursue a tax-free spinoff of the company's biosciences business into a separate, publicly traded company whose product portfolio will include its recombinant factor IX product **Ixinity**.

CSL Behring enrolled the first patient in its phase II/III clinical study evaluating the company's recombinant fusion protein linking coagulation **factor VIIa** with albumin for on-demand treatment in patients with congenital hemophilia A or B who have developed an inhibitor to factor VIII or factor IX replacement therapy.

New long-term data published in *Haemophilia*, the official journal of World Federation of Hemophilia, European Association for Haemophilia and Allied Disorders, and Hemostasis and Thrombosis Research Society, shows a favorable safety and efficacy profile with extended-interval prophylaxis for Biogen's **Eloctate** (recombinant FVIII with prolonged half-life).

manufacturer

First Human Cell Line Product

Octapharma's Nuwiq® (*Nu*-veek) is the first B-domain–deleted recombinant factor VIII derived from a human cell line for treating bleeding in hemophilia A. In clinical studies, patients switched from a routine prophylaxis regimen to personalized prophylaxis based on analysis of individual pharmacokinetic (PK) data. Why this matters: Personalized prophylaxis resulted in lower factor VIII consumption for more than half of study patients with severe hemophilia A, according to results of clinical trials of Nuwiq.

For info: www.octapharmausa.com

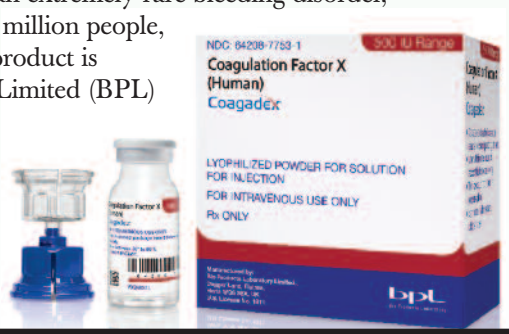
Getting Under Our Skin

The US FDA has granted breakthrough therapy designation to ACE910 for the prophylactic subcutaneous treatment of people 12 years or older with hemophilia A with inhibitors. ACE910 is an investigational monoclonal antibody engineered to simultaneously bind factors IXa and X, which is normally done by factor VIII, to allow a blood clot to form without factor VIII. ACE910 is administered subcutaneously once weekly. Why this matters: ACE 910 will provide a much-needed alternative treatment for people with inhibitors to factor VIII; and because it's structurally different from factor VIII, ACE910 is not expected to lead to the formation of inhibitors against factor VIII.

For info: www.genentech.com

It's a 10

In October the FDA approved Coagadex, a plasma-derived factor X therapy. Factor X deficiency is an extremely rare bleeding disorder, affecting only 1 in 500,000 to 1 million people, men and women equally. The product is manufactured by Bio Products Limited (BPL) of the UK. Why this matters: This is the first time a factor X concentrate is available for patients with factor X deficiency.



For info: www.bpl-us.com

nonprofit

We All Need Somebody to Lean On

In 2014, Hemophilia Federation of America's Helping Hands program assisted 275 households and distributed over $124,000 in direct aid to families in the bleeding disorder community. Of those 275 households helped in 2014, HFA assisted 95 families or individuals with housing expenses. Why this matters: Escalating out-of-pocket costs have left many families in our community needing financial assistance.

For info: www.hemophiliafed.org

The Force Awakens

The Generation IX Community Leaders Project is a call to action for young adults who live with hemophilia B and want to test their skills in an immersive leadership program. Sponsored by the Coalition for Hemophilia B with funding from Emergent BioSolutions, the project is open to anyone aged 16–30 with a hemophilia B diagnosis. Past participants of Generation IX programs are welcome to attend. The instructor team will guide participants through several days of experiential training in interpersonal skill development, finding a personal leadership style, and group dynamics with other motivated young adults. Applications due December 5. The program takes place in Florida, January 6–10, 2016. Why this matters: As the current hemophilia community leaderships ages, young community members must be trained and ready to be leaders one day.

For info: generationixproject.com

global

Biogen's Gift to the Developing World

The first shipments of Biogen's historic donation of factor have started arriving at treatment centers in developing countries. The donation will provide up to 500 million units of hemophilia therapy over five years to the World Federation of Hemophilia. Why this matters: Up to 75% of the world's population with hemophilia receives little or no treatment.

For info: www.wfh.org

After Nepal's Earthquake

As president of Save One Life, Laurie Kelley traveled to Nepal in August 2015 to assess the needs of the hemophilia community in the wake of the April 25 earthquake. This was the first visit by someone from the international hemophilia community. Laurie met with the Nepal Hemophilia Society, a well-run NGO that has partnered with Save One Life to sponsor 86 children, one of our biggest programs. About 53 families lost their homes in the earthquake; two mothers and one eight-year-old died. Relief funds were provided to help families recover. Why this matters: Save One Life will now start examining disaster relief needs and programs for the hemophilia community in developing countries.

For photos: lakelley.smugmug.com

Inbox... from page 2

Bravo! As an OB RN and the parent of two boys with hemophilia, I abhor the practice of circumcision. After assisting with and watching them in my early years as a nurse, I came to the realization that it was wrong. I am so thankful for that experience because it led me to research more, and I had already decided against it before we had our first boy. I had no family history of hemophilia, so when the boys came (twins, one with, one without hemophilia), it was sheer luck that our boys were protected from this surgery.

Over the years, I have become more baffled by the American cultural blindness that encourages circumcision. I watch moms go against their maternal instinct. I watch pediatricians try to convince parents to do it. I watch things that make absolutely no sense in a society with access to all the information necessary to see it for what it really is. It has been eye-opening for me to understand how incredibly strong cultural bias is.

So I was delighted to see an article that spoke the truth and presents circumcision for what it is. When I was told by my family that there was a circumcision article in PEN, I said it's probably an article telling parents how they can have it done safely, so no worries. I can't express how happy I was once I started reading it.

Fantastic job, excellent presentation of the facts, and myths debunked. Way to go after a hard topic and confront it head on!
Stacey Eason, RN
Kansas

Educational Resources

I was so scared when I found out that my newborn had severe hemophilia A. I didn't know what it meant, but I was really sad and worried. The hematologist gave me your book, and since then I have been Googling your company to get more information. I am so thankful for you and the good deeds you have done for our community around the world. I am now trying to go to nursing school to become a hemophilia nurse. I would like to express my appreciation for your work. Your book has helped me through so much!
Hathairat "Joy" Desnoyers
Virginia

I cannot wait to dig into *Raising a Child with Hemophilia*! Thank you for not only gifting us with your book, but going the extra mile with the toddler and children's books. I was really touched by your generosity. These acts of kindness make this journey seem a little less lonely. I hope to pay it forward one day once I become a pro "hemo" mom!
Marci Brown
Michigan

I read your book *Raising a Child with Hemophilia in Latin America*. I have two children with severe hemophilia A. Fede is 16 and has had a simpler life than Fran, who is six and has had inhibitors since age one after treatment for a head bleed.

I had Fede when I was 22, and I didn't know anything about the illness or how he got it. I began to investigate and research it to have a better understanding.

It's difficult when you don't have anything and don't know how to manage the situation. Thank God for the children who have hemophilia in Argentina today because things have changed. Fede wasn't on prophy, since he didn't have many serious bleeds until age three, when he complained of a painful headache. It was a head bleed.

At age three, Fran had another head bleed. When he was age four, we decided to move to Spain so the boys could receive medical care at the university hospital; Argentina is not authorized to do immune tolerance therapy for inhibitors.

Fran has been on ITI treatment for two years, and the inhibitor went up to 1861 BU, lowered to 37 BU, and then began to rise again. Because he is getting braces now, it goes up and down. So we are going to do a more drastic treatment with Rituximab and corticosteroids to bring his inhibitor down.

I send you a strong hug and I am happy to know that people like you exist. You teach people and help the ones going through the same experience of the adventure of raising a child with hemophilia because it's not just that child with hemophilia, but it's also his brothers, parents, family, friends, and the society in which he needs to live tomorrow.
Karen Lemos
Argentina

Project SHARE

I give my sincerest and deepest thanks for your endless support to our dear patients. Thank you so much for the donated factor concentrates you send us.
Mayette D. Charvet
The Philippines

Thanks so much for all the factor my son Paul Daniel received from you. You're always there for all the life-threatening situations, saving the lives of patients with hemophilia.
Irene Cantona Ardoña
The Philippines

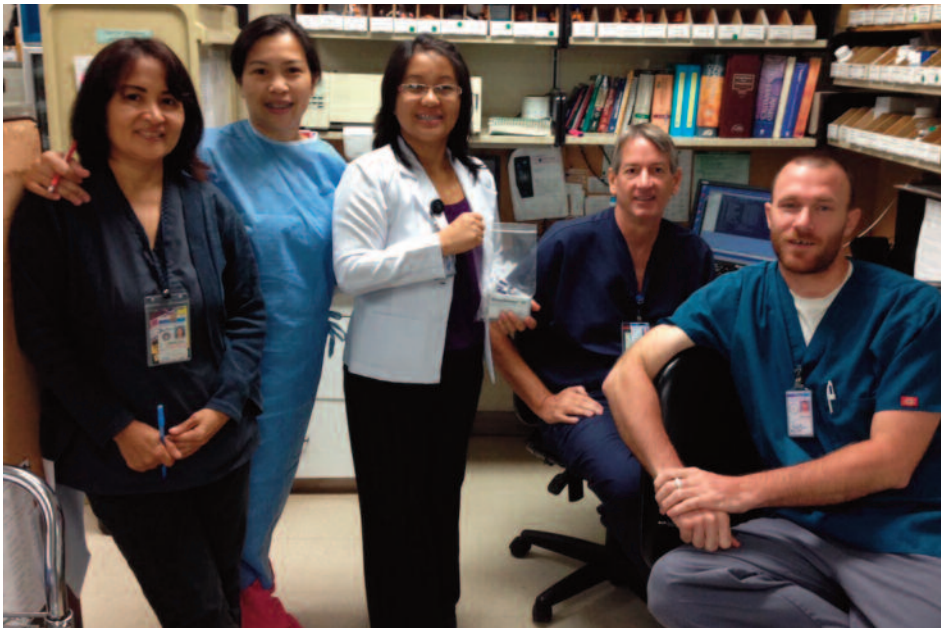
My family is very grateful to God and to Project SHARE for the factor VIII vials that were donated for my two sons, Marcus and Matthew, not just once but twice. The first donation six years ago greatly helped in saving my then five-month-old son's life!

Maricris Galang-Pammit
The Philippines

Thank you for your donation to Saipan's only hospital. We have had a number of motor vehicle accidents after the typhoon because of a persistent lack of street lamps (no power except generators for about 85% of the island) and one was a hemophilia patient with an inhibitor, so the NovoSeven was a godsend.

Here is a picture of our pharmacy team, super thankful for your help!

Tiffany Lin, Pediatric Department Chair
Commonwealth Healthcare Corporation
Saipan



Dr. Tiffany Lin (center) and team

Parenting Moment

We must remember that intelligence is not enough. Intelligence plus character–that is the goal of true education. — *Martin Luther King Jr.*

Take care of your family first. But then reach out to your neighbor, your block, your city, your country. Everybody wants change, but they want it to come by way of somebody else…If you wait for the government, you'll wait a long time. — *Edward James Olmos*

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