PEN

inside

Teaching Your School-Age Child About Hemophilia

Laurie Kelley

One of the biggest challenges we have as parents of children with hemophilia is teaching our children about their disorder. We often use words like hematoma, factor, and deficiency; and concepts like prophy, coagulation, and heredity. But children understand these words and concepts very differently than adults do.

If you don't know how your child's mind works at various stages of his development, then teaching him about hemophilia becomes hit-or-miss. But when you know how he thinks, you can tailor information in a way that he can easily understand. So to teach your child about hemophilia, you need to know how he processes his world in general, and hemophilia concepts in particular.

The School-Age Child's Thinking Tools

Between ages 7 and 11, the school-age child is in a fascinating stage of cognitive development. "Cognitive" refers to *how* he thinks, how he processes incoming information about his world—basically, his ability to think logically. Just as he has a skeletal structure that develops as he grows, he also has a mental structure that develops as he matures, filtering information in a way he can grasp.

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welcome

I've made a career since 1989 of educating families with hemophilia and von Willebrand disease about their disorder. I have an undergraduate degree in child psychology, specializing in children's stages of cognitive (thinking) development. By the time I graduated, I had published research on how children understand concepts of health and illness. When my son with hemophilia was born, I developed a strong interest in learning how children understand the disorder.

I applied the stages of cognitive development founded by Swiss psychologist Jean Piaget, and using my previous research, I interviewed boys with hemophilia at various ages for a book I was writing. Their answers mirrored what Piaget had discovered: children, no matter their culture, exhibit the same characteristics of cognitive development as they mature through different stages. Using these characteristics, you can explore with your child his ideas about death, life, dreams, health, and more. It's fun and fascinating!

We all want to teach our children about their disorder, so they can care for themselves and have better self-esteem. But we can do it more effectively if we first understand how they process their world, and then choose terms and concepts that we're pretty sure they will understand. You wouldn't give a Trek road bike to a preschooler! He doesn't have the ability to handle it. He needs to grow taller, build his muscles, and gain coordination. Similarly, you wouldn't use words like genes, proteins, or chromosomes with a preschooler; but you can use them with an older school-age child, and that's who we examine in our feature article.

You'll also enjoy reading how Helene Zereik learned the best ways to teach her son about hemophilia. And Richard Atwood reviews books for young readers. Don't forget, LA Kelley Communications has the biggest collection of children's books about hemophilia, all age-appropriate and meticulously researched! Best of all? They're free.

Laurie Kelley

inbox

I JUST FINISHED READING YOUR book *Raising a Child with Hemophilia*, on my son's second birthday. Thank you for writing this book and for updating it so diligently. I wish I had known about this book when my son was diagnosed with severe hemophilia at seven months.

I found the book a difficult but necessary read. I cried more reading this book than I have since he was diagnosed, but it allowed me to face some of my fears about the future. It has been a bit like peeking around the side of a curtain to see what the future may hold, and it doesn't seem as bleak as my imagination made it out to be.

I am now recommending your book to all of Christopher's aunties, uncles, and grandparents.

Iain Edgar
SCOTLAND

IN YOUR LATEST COMMUNIQUÉ, YOU mentioned the Facebook group Hemophilia Mother. I'd include a warning that some FB groups are not private, and anyone can join and see what you write, as with this one. I took myself off the group because of an incident about six months ago: people outside of the hemophilia community were taking screenshots of our conversations about hemophilia and circumcision, and these were showing up on an anti-circumcision site. It turned very ugly,

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PARENT EMPOWERMENT NEWSLETTER | AUGUST 2018

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Parents or patients with personal insurance questions should contact their employer's human resource department, Medicaid or Medicare caseworker, payer representative, or HTC social worker.

It's increasingly recognized that women are not just carriers of the hemophilia gene, but also can experience symptoms if they have less than 50% of their factor active, and they should be diagnosed with mild hemophilia. The majority of diagnosed patients are male. For editorial simplicity in PEN articles, whenever we refer to a person with hemophilia, we may alternately use "he" or "she," or just "he."

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Helene Zereik

Learning About Hemophilia: Through the Heart and Head

I closed my eyes for just a moment, and when I opened them again, my baby was five years old and starting kindergarten.

G, born in 2007, is my firstborn and has severe hemophilia B. I left my job in human resources when he was a baby, because it was getting hard to explain all my absences due to his constant hospital visits and injuries. My mother had a home daycare, and I would sometimes leave him there. I never put him in a private daycare, and we never did any extracurricular activities besides swimming when he was a toddler.

I sheltered him, and I sheltered myself. Then school started, and I could no longer shelter him. Short of homeschooling him, there was nothing I could do besides send him to a public school.

I wondered how I could send him to school and not feel anxious, nervous, stressed. I searched for the right school within our district. I decided on a smaller school with fewer than 300 kids. I felt that it was important for all the teachers, administration, and lunch monitors to know who my son was.

I felt better knowing that everyone would identify my son not only by his first name and his heaps of curls, but also because he had a bleeding disorder.

I wondered, how can I prepare him? How can I educate him about his disorder? G was old enough to know that he had something that most kids did not have or had never heard of, that he was different somehow. He knew that he could get "boo-boos" more than other kids. Yet he was still too young to understand that he couldn't pretend to be Batman or Spider-Man and attempt to climb walls. He could not comprehend the dangers of simple things like playing on the monkey bars or climbing the ladders at gym.

I sat him down to tell him that Mommy was going to have a meeting with the district nurse, the hospital nurse, and the school staff to talk about how to deal with his getting hurt sometimes. I showed him his medical kit that I had prepared. It contained popsicles for lip and tongue bleeds. It had superhero band-aids for his scrapes, a Lightning McQueen ice pack, and colorful bandages. I supplied his teacher with children's books on hemophilia. The teachers read short stories such as *All About Me* and the *Bob Goes to School* series given to me by the Canadian Hemophilia Society. G seemed happy with the items but was still a little anxious.

"I don't like hemophilia. I don't like myself. I don't like my body," he would repeat, he would cry.

My heart ached as he spoke those words. As the year progressed, they became weekly sayings.

I simply said, "This is your body and this is how it works." I told G that in order for him to participate in the fun at school, he had to take his special medication that would make him strong. It gave him super-power abilities to be able to play at gym and just be like all the other kids. I told him that he was a regular little boy, but that he just needed an extra special touch to be able to jump around and play.

G was too young for me to explain clotting or severe bleeds. So I made a visual for him. I opened the faucet to let water drip slowly. I explained that the water was like his blood. When he got hurt, his body was like the faucet, and he needed to make it stop running. The only way to do that was to take his medication. And this explanation seemed to work. As G got older, I used kitchen ingredients, adding flour to water to show how a substance can thicken a

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Paul Clement

SIPPET Revisited: The Big Question

Shock waves reverberated through the hemophilia community in November 2015 when the abstract "Study on Inhibitors in Plasma-Product Exposed Toddlers" (SIPPET) was published. What caused the stir? The study found that recombinant factor VIII (rFVIII) products are associated with an 87% increased risk of inhibitor development in toddlers below age six, when compared to plasma-derived factor VIII (pdFVIII) products containing von Willebrand factor (VWF). SIPPET also found a 69% greater risk of the more severe high-titer inhibitors, as compared to pdFVIII products with VWF. Some parents of toddlers worried: Should they switch their toddler from an rFVIII product to a plasma-derived product with VWF?

The full SIPPET report wasn't published until May 2016, six months after the abstract.[1] Now, two years later, the data has been analyzed by researchers, clinicians, regulators, medical advisory boards, and other experts. Regulatory agencies and national hemophilia organizations have issued updated treatment recommendations. Here we'll discuss these recommendations to help you and your hematologist select the right factor product for your child.

> Should you switch your toddler from a recombinant factor VIII product to a plasma-derived product with VWF?

Inhibitor Risk Factors

Inhibitors are antibodies—part of the immune system—directed against infused factor. About one-third of people with hemophilia A will develop inhibitors. Inhibitor development results from a complex interaction between a person's immune system and genetic and environmental risk factors. Even after decades of study, we're still unsure why some people develop inhibitors and others don't. But we do know of about a dozen risk factors for inhibitor development, including severity of hemophilia; type of factor VIII gene mutation; family history of inhibitors; having African or Hispanic ethnicity; certain variations in immune-response genes; presence of immune system "danger signals" such as infection, injury, surgery; intensity of treatment; and type of factor concentrate. Having these risk factors increases the chance of an inhibitor, and the more risk factors you have, the greater the risk.

The SIPPET Goal

SIPPET focused on one risk factor for inhibitors: type of factor concentrate. SIPPET researchers wanted to answer this question: Is pdFVIII with VWF less immunogenic—less likely to cause inhibitors—compared to rFVIII without VWF?

This is a long-standing question in hemophilia research. Some inhibitor studies have found a higher inhibitor rate in recombinant products; others have found no difference. And because there are so many "confounding variables" (other influences that can affect inhibitor development), and because of the small number of participants in inhibitor studies, it's been hard to find a definitive answer.

SIPPET researchers designed a study called a prospective randomized controlled trial (RCT). "Prospective" means looking forward, before the patient has developed an inhibitor (unlike retrospective or "observational" studies, which look backward, after an inhibitor has developed). "Controlled" means that there are two groups: (1) an experimental group that will use pdFVIII containing VWF, and (2) a control group that will use rFVIII without VWF. This control group is a standard of comparison against the experimental group. "Randomized" means that no one involved in the study influenced which group a patient was assigned to.

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1. Flora Peyvandi, Pier M. Mannucci, Isabella Garagiola, et. al., "A Randomized Trial of Factor VIII and Neutralizing Antibodies in Hemophilia A," *New England Journal of Medicine* 374 (2016): 2054-64.

richard's review

Linda Weaver's Studio

Richard J. Atwood

Hemophilia Fiction for Younger Readers

Learning to read is a significant milestone in our personal development. As adults, we nudge young readers along in this quest. First we read to them, and later we encourage and supervise for age- and skill-appropriate reading materials.

Our bleeding disorder community is fortunate to include notable authors who write about hemophilia. Their books for young readers are educational and entertaining. Look for short reviews of these books in PEN's Biennial Bleeding Disorder Resource Guide.

Other authors who write for younger readers sometimes include hemophilia. I suggest the following books.

Main Characters with Hemophilia

Starring Peter and Leigh (Delacorte 1979)
The Friendship Pact (Scholastic 1986)

Susan Beth Pfeffer

What could be better than a fictional leading character with hemophilia? Peter Sanders, a 17-year-old with hemophilia on Long Island, becomes stepbrother to 16-year-old Leigh Thorpe. Leigh, a former child actor, wants to be a normal teen, while Peter, often bedridden, wants to attend school. Pfeffer also included hemophilia in her preteen novel *The Friendship Pact*. Tracy Newfield, age 12, has a crush on a young actor, Ross Perlman. Ross is a Dartmouth graduate who gives a benefit concert for National Hemophilia Foundation and dedicates his unreleased song to Tracy. Hemophilia in these novels seems dated, yet the descriptions are accurate for the time period.

Panda Bear Is Critical (Macmillan 1981)
Picture Perfect (Severn House 2000)

Fern Michaels

Michaels wrote *Panda Bear*, a suspense novel, and later retitled it *Picture Perfect* after making some changes. Five-year-old Davey Taylor stays with his aunt and uncle while his scientist parents travel to Florida to testify in a mob-related drug trial. Davey has hemophilia, for which he receives daily "antigen shots." During a camping trip, Davey is kidnapped without his hemophilia treatment. He must cunningly escape, aided by his Yorkshire terrier and his CB radio, so he can be treated before it's too late.

She Died Too Young
All the Days of Her Life
A Season for Goodbye
(Bantam 1994, 1994, 1995)

Lurlene McDaniel

This series of three inspirational novels deals with life-altering situations for children and young adults, ages 10 and up. In the series, teens with various medical conditions attend summer camp at Jenny House in Asheville, North Carolina. One camper is Jeff McKensie, who has hemophilia. Jeff moves from Colorado to Miami to study to be an architect, yet his romantic opportunities at camp are stymied due to his medical condition.

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The Benefits of Physical Therapy: A Customized Approach

Dr. Michael Zolotnitsky

As humans, we get tired of performing the same tasks over and over again, so we switch our routine. One month we like Facebook, then Instagram, then Twitter, then Snapchat. Why do we always switch our social media, diets, pillows, destinations, or fashion trends? Our minds tell us that if we do the same thing repetitively, we'll get bored. The same goes for exercise.

We have to change our physical routines to avoid plateauing. Exercise needs to be consistently modified to help improve our bodies, to keep them from getting "bored." But how do we do that safely to avoid injury? People with bleeding disorders find it hard to exercise for various reasons. I had difficulty when I first began, because I was afraid of causing more damage to my joints. When you start a new routine, you may face challenges: soreness, increased joint pain from overload, increasing low back or neck pain from improper technique, or maybe just not enjoying the workout. I wanted to find workouts that would be fun, so I could see results, not get bored, and improve my overall well-being to reduce my joint bleeds. That's when I decided to pursue a career in physical therapy—to increase my knowledge and to help others.

Attaining my doctorate in physical therapy to assist people with bleeding disorders was a huge accomplishment in my life. I knew what it was like growing up with hemophilia. I endured persistent joint bleeds, had trouble walking, and felt different from my peers. When I learned that with proper exercise, I could greatly improve my joint health, I felt like I had my life back, and I swore never to lose that health. From age 13, I have been running, weight lifting, and playing sports. I haven't had a joint bleed in over 15 years; I attribute this to strength training and living a healthy lifestyle.

I wanted to educate the bleeding disorder community about what exercise can do for us all. I didn't want anyone to feel they couldn't do something because their bleeding disorder prevented them. I had the same concerns at a young age, but with hard work, I was able to overcome adversity and live like a "normal" person.

Exercise is my key to wellness, staying fit, and avoiding joint damage. I believe it's crucial for people with bleeding disorders. Maintaining optimal joint health will increase functional mobility, strength, and endurance; but most important, it will reduce the number of joint bleeds and improve overall quality of life.

And not just any physical therapy, but a personalized approach. No two people are the same, especially when it comes to bleeding disorders. When I sit down with a patient, I ask, "What do YOU want to get back to doing?" From there, we develop a personalized and customized treatment plan that relates to that patient's goals. For example, if someone wants to run, we watch him run on our antigravity treadmill, which uses three camera angles and allows dramatic reductions in impact and gravitational forces, helping the patient increase mobility without pain. This allows the patient to walk, run, squat, and jump in a pain-free environment.

If a goal is more sports-related—soccer, basketball, golf—it's imperative to assess the overall quality of movements: the golf swing, running, jumping, or shooting a basket. We find the root of the pain or dysfunction by assessing all of these movements, and by looking at the ankle, knee, hip, pelvis, and spine to make sure we aren't letting our patients put unnecessary strain on the body. A full-body approach is essential, so that every joint is covered.

Footwear is also important because increasing strain on the feet can affect overall walking, and can cause joint pain from the feet to the low back. As we age, our bodies change; so it's crucial to be reevaluated by a professional experienced in dealing with people with bleeding disorders. Flexibility is important, strength is important, but we need to make sure our bodies as a whole are symmetrical. We can use methods such as kinesio taping to place the joints in improved alignment, for joint support, and for reducing joint inflammation.

This customized approach yielded fantastic results when my first patient with hemophilia came to me for treatment. After developing inhibitors at an older age, being diagnosed with severe osteoarthritis of the hip, having brittle bones and severe weakness due to prior joint bleeds, he was facing total hip replacement. My evaluation showed that he had difficulty walking, balance deficits, poor core stability, and weakness in his knees, hips, ankles, core, and overall upper extremity strength. He used to walk a mile, but now it was hard for him to stand for five minutes with his cane. So his three months of treatment included endurance training on the antigravity treadmill; manual therapy to improve mobility of his hips, knees, and ankles; laser therapy to reduce joint inflammation in his hip; and balance retraining to reduce his risk of falling and improve his overall gait. At the end of treatment, he had stopped using a cane, and his hip surgery

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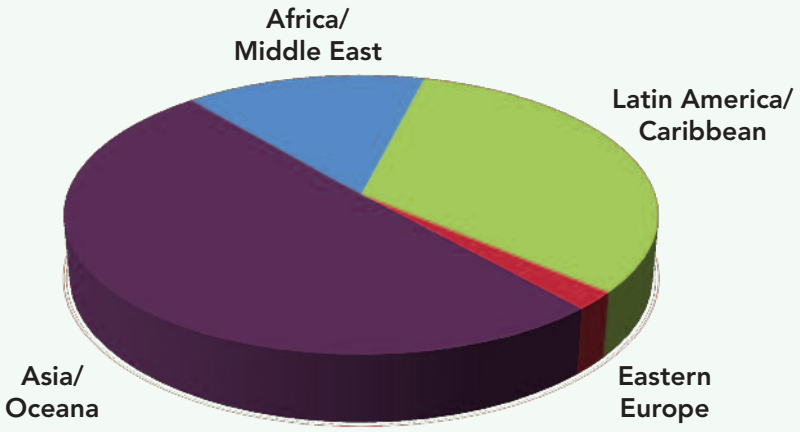
Banner Year for Project SHARE

Project SHARE had a banner year, thanks to a large donation of product from Octapharma, one of SHARE's supporters. About 46 million IU of factor, valued at over $46 million, was donated to 34 developing countries. And 2,258 mg of NovoSeven®, valued at $3.4 million, was donated to 9 developing countries. Some of these donations went to hemophilia camps in the Dominican Republic and Romania, so that children could safely attend camp. LA Kelley Communications president Laurie Kelley also visited Rwanda, Pakistan, and Nepal, to assess needs and visit patients who received product. Download all the Project SHARE annual reports at www.kelleycom.com/projectshare.

2017 Factor Recipients by Region



LA Kelley Communications, Inc.

A young recipient of factor in Pakistan

PROJECT SHARE

Teaching... from cover

Your child's mental structure is characterized by five major thinking tools that are constantly evolving:

Causal thinking: Figuring out when something *causes* something else, using a step-by-step process. A preschooler doesn't typically think step-by-step.

Internalized thinking: Moving from understanding his world mainly through his senses—where things happen *outside* him—to realizing that things can happen *inside* him.

Gradient thinking: Knowing that the world isn't just polar opposites, like good guys and bad guys. There are now shades of gray, degrees of intensity. A good guy might do something bad. Your child can also distinguish parts from the whole.

Empathic thinking: Starting to see the world from another's point of view.

Time: Understanding that he doesn't exist just in the present, but that he has a past and a future.

For understanding hemophilia, the most important of these five thinking tools may be causal thinking. Your child can now try to figure out how one thing causes another. Like…What causes bleeding? A blood clot? What is genetic transmission? It's hard to explain these concepts when your child doesn't understand causality.

These are more sophisticated thinking tools than he had as a preschooler, yet a school-age child, ages 7 to 11, is most comfortable using his new thinking tools on things and places he knows best—the tangible, visible world. So let's see how he uses these thinking tools on various topics in hemophilia, starting with blood.

How He Understands Blood

Unlike a preschooler, your school-age child understands the concept of the whole and its parts. So you can explain blood in terms of what it's made of. Children between ages 7 and 9 believe that blood is a red liquid, but also that it's composed of "stuff—water, food and energy." Children between ages 9 and 11 tend to describe blood in more abstract terms. "It's cells. Little roundish stuff. They're red and blue." A child develops from concrete to more abstract thinking, so this is perfectly acceptable!

Now you can introduce the idea that blood has components: white blood cells, red blood cells, and platelets. While preschoolers focus on things outside the body, mainly what they can see, hear, and feel, a school-age child realizes there are things inside him that he can't see. So he's ready to learn about simple blood components, especially those related to his hemophilia.

How He Understands Hemophilia

Because he understands a whole and its parts, your child can now categorize things. A preschooler might describe hemophilia

as "blood," or "something I have," but a school-age child can classify hemophilia as a "blood disorder," or "when blood doesn't stop bleeding."

He also progresses from describing hemophilia as his own specific injury ("It's when I get a hurt knee") to seeing it as a condition ("It's when *someone* gets hurt and bleeds a lot"). This is the empathic thinking tool: he knows he is not the only one to have hemophilia. He now says that hemophilia is when "*boys* with hemophilia have to go to the hospital sometimes." Compare this to the preschooler reply, "When *I* have to go to the hospital."

Your child also has matured from an external to a more internal focus. A preschooler might say, "Hemophilia is bruises," but a school-age child will say, "My blood doesn't work right." What is it that doesn't work right? Well, he understands the concept of a whole and its parts, and he's ready to know that blood is composed of parts. So he can deduce that hemophilia means "something's missing" in his blood. Some children say that they have "lost" something, or that their blood is "too thin." These answers reflect the "something's missing" idea. For example, "It's when you're missing some factors that help to make it so if you slam your knee against something it doesn't swell up as much. You'll have to replace the factor."

Misconceptions and medical inaccuracies abound as your school-age child struggles to understand hemophilia. "It's a blood disease. You lose part of your blood and you need to get more blood." At this stage, what's important is not so much that his answers are right or wrong, but how he arrives at his interesting conclusions.

So teach your child that hemophilia is a "blood disorder." Teach him that blood is made up of parts, and that he is "missing" a part. There's no need to get too specific at first, for example by mentioning factor and proteins; just stick to general concepts and ideas. To help him visualize, use a concrete example, like the falling dominoes. Remember that a school-age child is increasingly able to understand more abstract terms, but he needs the help of concrete examples.

How He Understand Genetics

Learning about heredity is a great way to exercise the "missing step" concept in a step-by-step sequence. To a preschooler, hemophilia is just something he was born with. To a school-age child, something had to happen to *cause* hemophilia.

What is that something? His parents are usually the missing step. Your child possesses the thinking tool of time, so he may realize that hemophilia could have started in his family many years ago, even centuries ago.

But *how* exactly does hemophilia get from one person to another? Most school-age children name a *causal agent*—the thing or event that caused hemophilia to happen. This can be a parent, blood, a chromosome, sperm, or even "vibes," as one boy phrased it.

Understanding often differs among younger children (ages 7–9) and older children (ages 10–11). When asked how they got hemophilia, younger children may simply reply, "Mom" or "Mom and Dad." Some children name blood. "It streams through your family, through their blood. I got it when I was two or three. My uncle gave it to me." This child is medically incorrect, but he's trying to sort it out logically: a family member had hemophilia, I have hemophilia, and…maybe my uncle gave it to me?

School-age children may also understand or accept some basic heredity rules, such as "mothers are carriers." This makes sense to them, because a "carrier" is a causal agent.

From ages 9 through 11, a child's answers and thought processes become more sophisticated. The causal agent may be chromosomes, which only a few children can discuss at this stage. Remember that school-age children are still very concrete—more comfortable with things that they see, hear, feel, and smell. Chromosomes are abstract. Some children identify an "X thing" as the causal agent, but don't understand the idea in purely scientific terms. To them, X and Y are not parts of the cells. They're more like "germs" that other family members "catch." One boy explained, "Mom's a carrier. She has two little things inside her, little Xs. They're like little eggs. She has a good X and a bad little X in her. My brother got the good carrier and I got the bad carrier."

Ask your child, "Where did your hemophilia come from?" and let him figure it out on his own; don't judge his answer at first or try to answer for him. You can work on the details later, supplying more accurate information once you've listened to his explanation and understood his thinking.

How He Understand Bleeds

Your child is now ready to understand bleeding as a step-by-step process. Although he might reply at first, "When you get cut, you bleed, you put a band-aid on, and you get better," he should begin to offer more detailed answers. School-age children have the thinking tools needed to explain step-by-step how bleeding occurs and how it stops. They can even name a causal agent in the healing process: factor.

Don't forget that school-age children are still most comfortable using tangible, concrete terms and analogies, and may still focus on things *outside* the body. Push them a little to focus on what happens inside.

If you ask, "How does bleeding stop?" your child might reply, "the blood clots," "the skin grows back," "you get stitches," or "the blood dries up and heals." He also might say, "You put a band-aid over it, and you get a scab. It's a blood clot." A causal agent might be named: "There's a *chemical* in the band-aid that makes the bleeding stop." "A scab makes the bleeding stop." Or, "Some little *plates* float up and close the cut." The causal agent might be outside the body, like a band-aid or scab; or inside, like "plates."

Time plays a role in healing now. One 10-year-old child reported, "It takes about an hour to get better. The skin would cover it, and it would turn into a scar." Another said, "It takes two weeks for it to get better."

School-age children grasp the concept of degrees. When asked, "What happens when you get cut?" they often say, "It depends..." What a sophisticated answer! They recognize that it would take longer to "get better" if they have hemophilia. "It takes longer—I don't have that much factor VIII to scare the blood back."

Asking about cuts almost begs a child to focus on the external. To really test your child's logical thinking, ask him what happens *inside* the body when he gets an injury. "What happens if you bang your knee real hard and you get a knee bleed? What happens *inside* your knee?"

Preschoolers will almost exclusively mention the external: swelling, bruising, and pain. School-age children are able to give a step-by-step, internal explanation. They can be very creative, using many concrete examples. "There's blobs of blood in the elbow. The blood tries to go through but can't because of the blob." Or, "I might get a bruise or I might get a bleed. It's filling up with blood. You can't move it, and it gets hard."

School-age children can provide one internal causal link: bleeding causes swelling and pain. They can then associate this idea with the "something's missing" concept. "The blood's rushing in [the knee]. I'm missing my factor to stop the bleeding."

Although your child's ability to understand is maturing, he still can't explain why he bleeds longer. He knows that he lacks something. But just how does that missing something make him bleed longer? He can't say. Blood clotting is still a mystery to most school-age children. "The vein inside your knee breaks, and blood comes out. It's bleeding internally. Factor makes it clot. It stops bleeding." True, but *how* does factor make it clot?

How He Understands Factor

Preschoolers perceive factor concretely, as external to them: it's exactly what you see, a bottle of white powder, their "medicine." Have school-age children progressed in the way they understand factor?

Don't be surprised if your 7- to 11-year-old child still says that factor is "like medicine, little pills that go into your vein and dissolve," or "little balls of white stuff." It's still mainly external medicine to him. Around ages 9 to 11, children may realize that *the body* normally produces factor—and *that's* the "something missing" in hemophilia!

Yet even realizing that factor is made inside them, most school-age children can't describe factor as a *blood protein*. Instead, they rely on a concrete description. It's "what you have and what we don't have. It's a chunk of white stuff that helps you heal." "It's the thing that's missing in your body. You're only missing one. There's supposed to be 10."

One big difference between preschoolers and school-age children is in the way they perceive factor's function. To a preschooler, factor helps him "get better" or helps "my hurt ankle." The first answer is pretty vague; the second is specific to the child's own injury. The school-age child, however, begins to link factor infusions to a particular job—stopping the bleeding. *How* exactly does factor stop the bleeding? Your school-age child may attempt a step-by-step account using concrete, visual imagery. Factor is "my medicine. It helps the blood go back into the vein." Or, "It helps you clot. Clot means to get better, to heal. It looks white." Factor is most often described by school-age children as stopping the bleeding by making a clot. But only a few children can describe a clot, other than as a "plug" or "ball." You can see that their concrete thinking tools (factor as a plug) are battling their more abstract thinking tools (factor as a blood protein).

To explain how bleeding is stopped by factor, your child may resort to a visual answer, using familiar words. Factor *pushes* the blood away, either from the injured site or back into the vein. Factor "mixes with the blood," "boxes with the pain," "eats up the blood," or "scares away the blood." Some children think that the clot means the swelling itself—a bunch of pooled blood. Factor then dissolves the clot, which is just the opposite of what it does!

Your child might also explain that to stop the bleeding, factor "blocks" the vein or the blood. "Factor blocks it so the blood can't come out. It clots the blood first and then puts a little door over the blood so the blood can't get out, and then it clears it out." This is a more medically accurate view of what happens: factor stops the bleeding, rather than removing excess blood. It may be useful for your child to draw pictures of what happens inside a bleeding joint. This will help make a difficult subject more concrete and perceptual, yet more accurate.

The major leap from preschool to school-age is that children now no longer simply say that they get factor *when* they bleed. Children say that they get factor *because* they are bleeding, to stop a bleed. This shows causality, a huge leap in logical thinking.

How He Understands Factor Deficiency Type

A 10-year-old boy once confided to me that he knew of *three* types of hemophilia: A, B, and C. He and his friends had been discussing hemophilia, confusing it with their report card grades. Type A was the "best" and type C was the "worst." He was type A, of course!

There are two major types of hemophilia: hemophilia A and hemophilia B. And there are three levels of severity: severe, moderate, and mild. To understand this, a child must be able to categorize and serialize; and to compare and contrast. Most school-age children have this ability, and are ready to learn about their type of factor deficiency and level. But as you may have guessed, they can easily misinterpret what you tell them as they struggle to fit your information into their thinking structure.

First, ask your child what kind of hemophilia he has. Many children don't know! It's crucial for your child to know his factor deficiency, for his self-esteem and his safety.

Now ask him what that deficiency means. "What does it mean to have hemophilia A?" He may recite "factor VIII." Yet when asked to elaborate, he may revert to a description of his concentrate bottle! Believe it or not, *your child may not understand that his factor deficiency type refers to his medical condition*. It's best to explain this to him in terms he'll understand: "something's missing." He may say, "There are 20 factors. I have all except factor VIII. It's like there's 20 baseballs inside; they have numbers and one is missing." That's a great explanation of factor deficiency!

How He Understands Severity Levels

Many school-age children understand the idea of severity. But they don't see it as the percentage of factor that is active. That's too abstract! Instead, they refer to having "a lot" or "a little" of the something missing. Or, more concretely, they think of severity in terms of having "a lot of bleeds" or "a few bleeds."

Your school-age child is now internalizing his condition. Bleeding depends on *what's* missing and *how much* is missing; and it's measured by how much or how often you bleed. These are very advanced concepts. Ask your child what his severity level is, and whether he knows what that means. Remember to simply listen to his response and accept what he says. Delve deeper into his reasoning, and see how far he can go. If he seems ready to learn more, or asks for clarification, offer it!

When your child begins learning about percentages in school, he's ready to apply this to hemophilia and severity—although he'll still want to resort to concrete explanations. "Let's say I had hemophilia 5% and another boy had 1%. The 1% is worse. They may or may not bleed the same. Maybe one kid got hit in different spots." This understanding of percentages is not a

mathematically correct "1 out of 100." Rather, it reflects a scale that goes from bad to worse. So if you use percentages with your child, be sure that you know how he may interpret them.

How He Understands Hemophilia as a Lifelong Condition

Your school-age child understands time now, and begins to understand permanence: things grow, live, and die. Time, a thinking tool, influences his concept of a cure.

Ask your child what will happen to his hemophilia when he grows up. He might say, "I'll still have it. It stays in my body forever." Or he may say, "I'll still have it because my cousin David is 15 and he still has it." Even younger school-age children may answer, "Unless they get a cure, I would have it for the rest of my life."

What if your child says, "I don't know"? You can help him explore the idea of permanence. Use an analogy to compare something concrete that he can understand. For example, explain that hemophilia is almost as if the body is missing a finger or toe, a part that cannot grow back on its own.

Even when he says that he will still have hemophilia when he grows up, you can check your child's concept of permanence. Ask, "If we put all new blood in you, from someone who didn't have hemophilia, what would happen to your hemophilia?" Some children will then show they don't yet understand permanence. Many school-age children believe their hemophilia will go away. "You wouldn't have hemophilia if you got new blood because there wouldn't be hemophilia in it."

As their thinking matures, older school-age children begin to see that they would still have hemophilia even if they received new blood. Why? Some children recognize that the body depletes factor VIII. Some realize that you would have to replace the liver. Now, rather than view hemophilia as existing solely in the blood, a separate entity that can be "removed," they see hemophilia as part of a *malfunction in a system*. "Hemophilia would take over my new blood. The blood uses up factor VIII real quick and can't make it." "Blood gets into your blood and goes to the liver. Hemophilia comes from the liver."

Several companies are working on a cure for hemophilia. Explore ideas with your child about how a cure might happen. What needs to change for hemophilia to go away forever?

Teach by Listening

As important as teaching your child new concepts and terms is simply asking him questions about hemophilia—and listening. *Listen deeply*. When he offers an answer, encourage him to explain more, and to use causality. Ask him, "What happens when you get a knee injury?" He may say, "It swells." And why does it swell? What happens inside? "Blood is filling the knee." How does that happen? How did the blood get in there? Keep asking questions: And what happens next? Where did that come from? How did that happen? What do you think caused that?

And let him respond.

Encourage him to use his thinking tools with each answer he gives. Ask open-ended questions, like "What do you think hemophilia means?" as opposed to "Hemophilia is a blood disorder, right?" See the difference? Don't bias your questions with extra information. Just ask, "How does…" or "Why is…" or "What happens next…" and see what he says.

Sometimes, your child will figure out the correct answer, just by trying to explain his thoughts to you—because you're willing to take the time, willing to listen, and willing to teach. A child who feels comfortable asking questions and exploring answers will gain confidence. Being able to explain his disorder to others will empower him. And that's the beauty of teaching him about hemophilia, in a way he understands.

headlines

manufacturer

Another Extended Half-Life Product Waits Approval

Novo Nordisk submitted a Biologics License Application (BLA) to the US FDA for N8-GP (turoctocog alfa pegol), an extended half-life recombinant factor VIII for treating hemophilia A. The submission is based on results from the company's "pathfinder" clinical trial program, which investigated the efficacy and safety of N8-GP in more than 250 adults and children with hemophilia A, and patients undergoing surgery. N8-GP is a glycopegylated, B-domain-deleted form of recombinant factor VIII designed to prolong its half-life to about 19 hours (1.6-fold prolongation compared to standard factor VIII). **Why this matters:** N8-GP will give people with hemophilia A another option for treatment.

For info: www.novonordisk-us.com

Break on Through

Genentech was granted Breakthrough Therapy Designation (FDA program designed to accelerate development and review of medicines) for Hemlibra®, for people with hemophilia A and without inhibitors. Data was provided from the phase III HAVEN 3 study in people 12 and older who dosed subcutaneously every week or every two weeks. No new safety signals were observed, and no thrombotic microangiopathy or thrombotic events occurred. **Why this matters:** Hemlibra may provide a more efficient prophylactic treatment option for people with hemophilia A, with advantages over factor VIII prophylaxis.

For info: gene.com

Celebrating the Gift of Life

Bioverativ Inc., a Sanofi company, joined the global hemophilia community in recognizing World Hemophilia Day on April 17, by celebrating its pledge to donate up to 1 billion international units (IU) of clotting factor over 10 years, including up to 500 million IU to the World Federation of Hemophilia Humanitarian Aid Program for up to five years, to help transform hemophilia care delivery in the developing world. Since the Bioverativ donation began in 2014, WFH has nearly tripled the percentage of children receiving treatment; provided prophylactic treatment to about 1,200 people; treated 79,500 acute bleeds; and enabled more than 1,500 surgeries. **Why this matters:** Thanks to Bioverativ's unprecedented factor donation, more than 15,000 people with hemophilia in 40 developing countries have already been treated.

For info: www.wfh.org

global

WFH World Registry

World Federation of Hemophilia announced a new World Bleeding Disorders Registry (WBDR). WFH partnered with the Karolinska Institute and Health Solutions to develop this hemophilia-focused bleeding disorders registry. **Why this matters:** WBDR is the only global registry collecting standardized clinical data on people with hemophilia worldwide and will ultimately improve quality and access to care for all people living with hemophilia.

For info: www.wfh.org

science

One and Done?

Scientists at Salk Institute have combined the CRISPR-Cas9 gene editing system with stem cell technology to generate a one-time, autologous cell therapy for hemophilia B. CRISPR-Cas9 gives scientists the ability to change an organism's DNA without using a viral vector. Autologous cell therapy involves harvesting cells from the person with hemophilia being treated, inserting a functional gene for factor IX into those cells (using CRISPR-Cas9), and injecting them back into the person. Tests showed that gene-edited, stem cell–derived liver cells remained viable and functional in hemophilic mice for nearly a year, after just a single injection. **Why this matters:** Gene therapy using viral vectors has shown promise for long-term therapy, but viral vector–based approaches carry problems, including possible immunogenicity and rejection of the newly inserted gene. Use of cell therapy may avoid these problems and allow for long-term, one-time treatment, although tests in humans are several years away.

For info: www.genengnews.com

nonprofit

NHF 70th Annual Meeting in Orlando!

October 11–13, 2018

National Hemophilia Foundation welcomes all attendees to sunny Orlando for three days of learning, fun, and networking with the national bleeding disorder community. Guest speakers, games, and social events make this a valuable time for new families and regular attendees. **Why this matters:** This is the largest national hemophilia meeting in the world.

For info: www.hemophilia.org

soundbites

French drug maker **Sanofi has acquired Bioverativ**, manufacturer of Eloctate® and Alprolix®, for $11.6 billion.

Novo Nordisk's Rebinyn® is now available in the US. **Rebinyn is an extended half-life factor IX** for treating and controlling bleeding in adults and children with hemophilia B.

China-based Creat Group now holds a **majority interest in Biotest**, manufacturer of two plasma-derived clotting factor concentrates, with about 90% of Biotest AG ordinary shares and voting share capital.

US-based Institute for Clinical and Economic Review (ICER) concluded that **Roche's Hemlibra** is a cost-effective therapy for prophylaxis in people with hemophilia A and inhibitors, despite a price set at $482,000 for the first year of therapy and $448,000 thereafter.

CSL Behring will discontinue the sale of **Monoclate®-P and Helixate® FS**, its plasma-derived and recombinant factor VIII products.

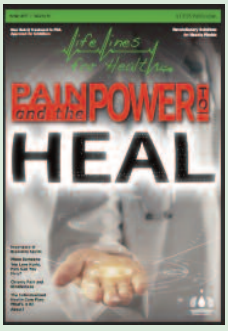
Canadian Blood Services will provide Shire's Adynovate, an extended half-life recombinant factor VIII product, and will phase out Bioverativ's Eloctate, an extended half-life recombinant factor VIII product by the last quarter of 2018.

The US FDA has approved **Shire's Vonvendi** (recombinant VWF) for perioperative management of bleeding in adults 18 and older with von Willebrand disease.

patient programs

Listen Up!

BloodStream podcasts are a unique way to stay in touch with current events in the bleeding disorder community. Podcasts are hosted by Patrick James Lynch, who has hemophilia, and his wife Natalie. Episode 23 welcomes Dr. Steven Pipe to discuss Hemlibra, the recently reported patient deaths, and what this means for our community. And special guest Bill Patsakos, lieutenant with the New York Fire Department, pharmacist for CVS, FEMA volunteer, and father of three sons with hemophilia, discusses his rescue efforts work in Puerto Rico following Hurricane Maria. **Why this matters:** Podcasts offer a great way to learn while you're driving or working out, or if you prefer audio to reading.



Only for Inhibitors!

Subscribe to *Lifelines for Health*™, the first-ever national publication for people with hemophilia and an inhibitor. Sponsored by Shire, the newsletter provides valuable, educational, inspirational articles and news for families and treaters. Published twice yearly and distributed electronically and in print. **Why this matters:** This is the only newsletter dedicated to patients with inhibitors.

For info: ches.education/newsletter

Very Campy

Friday, September 28–Monday, October 1
Painted Turtle, Lake Hughes, California

Inhibitor Family Camp offers a weekend of education, support, and fun designed for children ages 6–18 with hemophilia and active inhibitors, but geared toward the entire immediate family. **Why this matters:** This is the only camp dedicated to patients with inhibitors.

For info: ches.education

Inhibitor Family Camp

As I See It... from page 3

liquid. He seemed satisfied with those visuals.

I kept it simple. There is so much emotion when your child starts school, and it wouldn't have done G any good to have more information than necessary. When he started school, I gave him a little necklace with a heart on it. I told him that it was my heart he was taking to school, and that when he felt nervous, he could take it from his bag and hold it for a minute. This reassured both of us.

The important thing for kids with hemophilia to learn as they grow is that they need to tell someone when they get hurt. There's a fine line when determining a serious injury. Does it require Mom to come and assess, or getting out of class? That fine line is a slow and long learning process for the child and the parent. In the beginning, I would run to the school daily. G would get a paper cut, and the teacher would be nervous and call me. I tended to go as often as possible to lessen the stress for the staff. I instructed staff to call me anytime he fell, hit himself, cut himself, or was in any pain. This was simpler, and though it was stressful for me, it reassured G to see me and to hear me tell him he was okay.

I also joined the PTA, and I volunteer regularly, attending all the school outings. It makes G feel safe and takes a load of stress off the staff. This helped, but it still has not made him understand that he has to be extra careful.

I told him that he needed to always tell me if he felt pain anywhere in his body. I spent much time talking, repeating, showing him books, explaining things to him. Kids live in the moment, and my job was to repeat myself over and over, hoping that as he got older, he would be less likely to throw himself from the top of the monkey bars, and that he would think twice about pulling dangerous stunts. With each passing year, G has slowly started to understand that he is a little different from other kids. I'm hoping he is realizing that although he has different needs, he can still do most everything.

So how can you explain hemophilia to your children? You do it simply, in small words, and regularly. You talk to them. You continuously ask them questions and let them be part of the conversation. Does my son understand everything? No. Does he listen to everything I say? Definitely no! But I'm hoping that with each passing year, he will learn to love his body, own his disorder, and know that it does not define who he is as a person. I'm hoping he'll understand that he has options and opportunities just like everyone else, and that the sky's the limit for him.

Helene Zereik has three children and lives in Canada. She spends her days stepping on Lego pieces and running to the school to assess her son's injuries. Before she had children, she used to do her nails and hair regularly. She worked in human resources, where she would engage with other professionals, who never sounded like a cartoon or Darth Vader. Sometimes, she misses those days.

Randomization is one of the methods used to reduce the effects of confounding variables. If the sample size is large enough, and patients are randomly assigned to two groups, then each group should have about the same number of patients—with the same confounding variables, so their influence will be canceled. Problem is, the more confounding variables you have, the larger your study sample size must be to cancel out the effect of multiple variables—perhaps several thousand patients. And many variables affect inhibitor development, including some we don't yet understand. If the study sample size is too small to cancel the effects of confounding variables, and they can't be resolved by other methods, then the study's conclusions are questionable.[2]

RCT studies, when properly designed, are considered the gold standard in experimental research, thought to produce more reliable data than observational studies. This is not to say that the conclusions of an RCT are always correct, just that they have fewer biases than observational studies, so they're more likely to produce reliable results.

This is why the SIPPET results caused such a stir. As an RCT, SIPPET's conclusions carried more weight than any single previous inhibitor study, all of which were observational studies. But a single study isn't an end-all. When an RCT produces results that differ from previous studies, then researchers usually wait for the results of further RCTs to confirm those conclusions before making changes—such as modifying treatment protocols.

But RCTs are expensive, resource-intensive, and difficult to conduct—especially when the pool of available patients is extremely small, for example when finding previously untreated patients (PUPs) with hemophilia A. It's highly unlikely that another RCT studying how classes of factor affect inhibitor development will be done anytime soon. So here's the problem: Do clinicians change treatment protocols based on the results of a single study, a study funded by manufacturers of plasma-derived factor VIII products? And how much weight should be given to previous observational studies and registries, some of which, such as the European PedNet Registry, track almost four times the 251 patients studied in SIPPET?[3]

Finally, although an RCT can show relationships between variables being studied, it can't prove causality. In other words, SIPPET can't prove that the presence or absence of VWF in factor VIII caused the observed results. SIPPET just shows that there is an association between the two, and not necessarily that a lack of VWF caused the high inhibitor rates seen in SIPPET.

SIPPET's conclusions carried more weight than any single previous inhibitor study, but a single study is not an end-all.

What Do We Know?

• VWF attaches itself to factor VIII and chauffeurs it through the blood, significantly extending its half-life. VWF also physically covers several *epitopes* on the factor VIII molecule, blocking the attachment of inhibitors. Epitopes are points on proteins (such as factor) recognized by antibodies (inhibitors), where the antibodies attach. Experiments have confirmed that factor VIII bound to VWF has a protective effect against the formation of inhibitors, as compared to factor VIII without VWF.

• Of pdFVIII products, only intermediate/high-purity products contain VWF (ultra-high purity monoclonal pdFVIII products have no VWF). The three pdFVIII products with VWF available in the US are Humate-P® (CSL Behring), Alphanate® (Grifols), and Koate®-DVI (Grifols). Of these three, only Alphanate was used in SIPPET.

• No rFVIII products contain VWF.

• When infused, most rFVIII binds, almost instantly, to VWF circulating in the bloodstream. Presumably, this combination of rFVIII and VWF would have a protective effect against inhibitor formation similar to pdFVIII with VWF. But for most brands of rFVIII, not all the factor binds to VWF. Most rFVIII brands use hamster cell lines to produce the factor.[4] These animal cells have a tough time "finishing" or making the final modifications to the human factor VIII protein they produce. So as much as 20% of rFVIII produced by animal cells is not properly modified. This factor is not only non-functional; it also doesn't allow for the attachment of VWF, and has exposed epitopes that may trigger inhibitor formation.

This is one reason that two rFVIII products available in the US—Eloctate® (Bioverativ) and Nuwiq® (Octapharma)—use human cell lines for producing factor. It's assumed that human cell lines would allow for complete, correct synthesis of factor VIII. Whether these products have an inhibitor risk similar to pdFVIII with VWF hasn't been determined.

• Extended half-life rFVIII products may have a lower risk of inhibitor formation, compared to standard half-life recombinant factor. Some extended half-life products have proteins added to them, which cover some epitopes on the factor molecule. Or, in products using PEG molecules to extend the half-life, the long strands of PEG may physically keep antibodies away from the factor, like a horse's tail keeps flies away. Whether extended half-life rFVIII products have an inhibitor risk similar to pdFVIII with VWF has not been determined.

Obviously, there are many variables to weigh when choosing a factor product! Some clinicians have questioned whether the SIPPET results even apply to most of their patients, who are Caucasians. The patients in the SIPPET trial were mostly from India, Africa, Egypt, and Iran, ethnic groups that differed from those in the previous studies. Because some ethnicities have different forms of factor VIII, their immune systems may see a variation from another ethnicity as being "foreign," and develop inhibitors against it.

2. Although randomization is the most powerful method of reducing the effects of confounding variables, other methods can also be employed, some after-the-fact. In addition to randomization, SIPPET used matching, blocking, and statistical methods to help reduce the effects of confounding variables. 3. A patient registry collects information about patients affected by a particular condition. The US Centers for Disease Control and Prevention's Division of Blood Disorders operates Community Counts, a patient registry program of people with hemophilia. 4. Hamster cell lines used for the production of clotting factor include Chinese hamster ovary cells (CHO) and baby hamster kidney cells (BHK).

And the majority of patients in the US, Canada, and Europe receive prophylactic infusions, which have been associated with a lower incidence of inhibitors as compared to the on-demand treatment protocol used in most developing countries.

Another variable is that most US, Canadian, and European patients with hemophilia A use third-generation or extended half-life recombinant factor products, unlike the second-generation recombinant products (associated with a higher risk of inhibitors) used by 84% of the patients who received recombinant product in SIPPET.

National Recommendations

What do the experts say? The European Medicines Agency (EMA) is the European equivalent to the US FDA. EMA does not recommend any change, concluding that "there is no clear and consistent evidence of a difference in the incidence of inhibitor development between the two classes of factor VIII medicines: those derived from plasma and those made by recombinant DNA technology."[5]

But national patient advocacy organizations worldwide recommend considering SIPPET conclusions in treatment options for PUPs in countries where rFVIII is the standard treatment. Their recommendations include

- Continued treatment with rFVIII
- Treatment of PUPs with a third-generation or extended half-life rFVIII
- Treatment of PUPs with pdFVIII containing VWF
- Treatment of PUPs with pdFVIII containing VWF for the first 50 exposure days (days on which they receive factor) followed by treatment with rFVIII
- Treatment of PUPs with pdFVIII containing VWF for the initial 50 exposure days only in PUPs perceived to be at significantly increased risk of inhibitor development (if you don't have multiple risk factors, start treatment with rFVIII)

But these recommended treatment options only involve PUPs. National Hemophilia Foundation's Medical and Scientific Advisory Council (NHF's MASAC) recommends that people with greater than 50 exposure days to any recombinant product should consider remaining on their current product, since multiple clinical studies have shown that the risk for inhibitor development with any factor VIII product diminishes after 50 exposure days. MASAC also recommends that patients with more than zero and less than 50 exposure days consider staying on their current rFVIII product.

The Big Question

This brings us back to the big question: Should you choose a pdFVIII product containing VWF for your toddler?

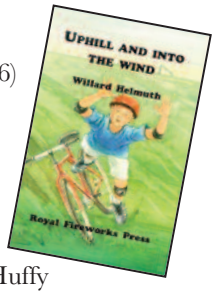
It's not easy to answer, and requires a risk/benefit analysis that is best done in close consultation with your hemophilia treatment center (HTC) hematologist. For some parents, this will be an agonizing decision to make, but remember, a single study is not an end-all. The SIPPET results are not a bombshell, but a piece of the puzzle that is inhibitors. It will take years, and several additional studies, before we have a definitive answer on how factor product type affects inhibitor formation. In the meantime, use the information at hand—with the help of your hematologist—to make the best decision for you.

5. For the EMA conclusion on SIPPET and factor VIII medicines, search for WC500234820 on www.ema.europa.eu/ema/ (November 10, 2017).

Richard's Review... from page 5

Diving for the Moon (Macmillan 1995)
Lee F. Bantle

Bantle, a lawyer, tackles issues of puppy love, adolescence, hemophilia, and HIV in this novel for ages 9–12. Josh Charkey, age 12, has hemophilia and AIDS. Spending the summer at a Minnesota lake, Josh shocks his close friend Carolina Birdsong with his medical revelations, while still fostering their budding romance.



Uphill and Into the Wind (Royal Fireworks 1996)
Willard Helmuth

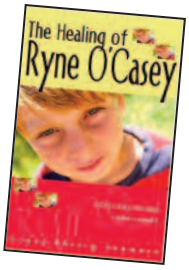
In this young adult novel, 12-year-old Eric Kenton, who has hemophilia, wants to be normal. While his parents worry about hemophilia and HIV, Eric dreams of, then successfully accomplishes, riding his fat-tired Huffy bicycle almost 400 miles to a summer camp in Ohio.

Kinetic (DC Comics 2005)

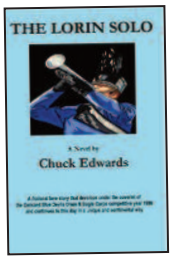
Kelley Puckett and Warren Pleece

Puckett and Pleece wrote and illustrated this story-book comic fantasy. Tom Morrell is a high school senior who has hemophilia, plus about a dozen other syndromes. Tom gains superpowers when he is hit by a truck, and the absorbed energy amazingly "cures" his medical conditions.

The Healing of Ryne O'Casey (FaithWalk 2004)
Scott Philip Stewart



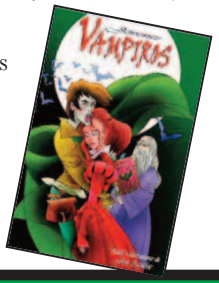
Ryne O'Casey, a 10-year-old from Tynbee, Tennessee, is first diagnosed with "hemophilia type A" when just over a year old, and then diagnosed with HIV at age eight. Some of the medical details are questionable for 1995, but the social reactions are truly believable.



The Lorin Solo (self-published 2006)
Chuck Edwards

This young adult romance novel follows Lorin Lenki, a music student at San Francisco State who has mild hemophilia. Lorin meets Tracy Martin, a nursing student at Saint Rita's Hospital. Tracy has severe hemophilia, as did her identical twin sister who died. The budding romance tragically ends when Tracy dies from a brain bleed in 1986.

Jovenes vampiros. El codice secreto (versos y trazos 2007)
Jose Aguilar



This illustrated novel, written in Spanish, tells the tragic love story of young Marco Tulio and Isabel, who has hemophilia. The setting is ancient Italy, and includes vampires and Leonardo da Vinci.

Supporting Characters with Hemophilia

Something Different in the Bank (E. J. Arnold 1989)
Grace Moulton

Something Different in the Bank is one of a series of books about children with medical conditions. The narrator describes his cousin Michael, who has hemophilia. The treatment of hemophilia seems out-of-date even for 1989, though some of the behavioral concerns are timeless.

Monkey Island (Bantam 1991)
Paula Fox

This teen novel's awards include an American Library Association Best Book for Young Adults, and a Horn Book Fanfare Selection. Clay Garrity, age 11, is homeless in New York City. When Clay is hospitalized for pneumonia, his roommate, an experienced hospital patient with hemophilia, provides sage advice.

Play to the Angel (Scholastic 2000)
Maureen F. Dahlberg

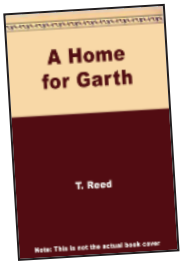
Greta, age 12, has an older brother named Kurt who dies due to his hemophilia. Set in 1938 in Vienna, this motivational teen novel with historical detail involves children from a musically gifted family who fulfill their dreams, even using the death of a sibling with hemophilia for inspiration.

Angel of the Square (HarperTrophy 2001)
Maureen F. Dahlberg

This novel for ages 10 and up tells the story of Ekaterina Ivanova, called Katya, the 12-year-old daughter of a lady-in-waiting to Empress Alexandra in Russia before 1918. Katya is a playmate of Anastasia and learns what is wrong with Alexei, who has hemophilia. Although Katya is fictional, her perspective on the Russian Revolution is believable.

A Home for Garth (self-published 2010)

T. Reed



Jake Stevens, age 11, finds a lost dog that belongs to Robert Higgins, a 10-year-old with hemophilia. Jake's moral dilemma is deciding whether to lie to keep the lost dog, or to be truthful and return the dog to its owner. Jake learns about hemophilia and how it limits what Robert can do.

Hardy Boys Undercover Brothers: Private Killer (#32)
(Aladdin 2010)

Franklin W. Dixon

This novel for ages 8–12 features teens acting as detectives to solve mysteries, and includes a female with hemophilia. Destiny Darity is the troubled daughter of the headmaster at an exclusive private school near Boston. Destiny has a rare blood type that she inherits from her mother and a mild case of hemophilia that she inherits from her father. The Hardy brothers, Frank and Joe, may not completely understand the genetics of bleeding disorders, but they determine who plays pranks on Destiny.

Blood Ties: A Blood Coven Vampire Novel (Berkley 2011)

Mari Mancusi

This young adult novel includes a main character named Sunshine McDonald, a 17-year-old fairy princess living in Las Vegas. She loves Jayden, a mortal with hemophilia. When Jayden is bitten by a vampire, he needs blood transfusions—not for his hemophilia, but for his turning. Sunshine and Jayden travel to London and Tokyo to find a cure for vampirism by drinking from the Holy Grail, and by doing so, cause Jayden's hemophilia to disappear.

I Hunt Killers
Game
Blood of My Blood
(Little, Brown 2012, 2013, 2013)

Barry Lyga

This trilogy of young adult mystery thrillers delves into the world of serial killers, and the vivid carnage may be inappropriate for young readers, or for any readers. Jasper Dent, age 17 and called Jazz, is the son of an imprisoned serial killer. With the help of his best friend Howie, Jazz helps police identify serial killers. Howie is a "type-A hemophiliac" with an overprotective mother, so his assistance is sometimes limited when extracting Jazz from dangerous situations. Thankfully, Howie provides some comic relief to an otherwise serious topic.

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Reading should be a lifelong endeavor and source of pleasure. Take advantage of these books to help young readers learn something about bleeding disorders. And keep reading good books to your child!

YOU... from page 6

Photo courtesy of Michael Zolotnitsky

was canceled. This was unbelievable: a person with hemophilia could overcome his pain and even cancel his surgery!

I can't emphasize it enough: *Physical activity is key.* And it's not too late to begin a program. It's essential to make sure the movements are performed correctly, to improve your joint function and not be detrimental. When you're being assessed by a physical therapist, make sure the approach is customized and personalized, and that your program will constantly be updated based on your progress. I have lived by this approach for over a decade, and have lived life to the fullest. I overcame feeling weak and unable to do the things I wanted to do. It's my mission to help others overcome obstacles to return to their lifestyle. A bleeding disorder is just another bump in the road; don't let it control your future. My favorite phrase is "easy PT, hard life; hard PT, easy life!"

Michael Zolotnitsky, PT, DPT, who has hemophilia, is director of physical therapy at New Jersey Spine and Wellness in Old Bridge, New Jersey. Trained as an orthopedic and neurological physical therapist, Mike ensures his patients are offered a customized approach, including personalized aquatic therapy programs in indoor and outdoor pools. Mike is a national speaker for the hemophilia community, and lectures on safe exercises and alternatives to pain management, demonstrates kinesio taping, and runs aquatic therapy sessions. Mike has run three marathons in one year, and enjoys traveling and hanging out with his family, including his two nephews and his girlfriend. He is fluent in Russian.

Inbox... from page 2

really fast. They started attacking individuals personally by mentioning things on their own personal FB pages.

There's no doubt these FB groups help people, like the mom you mentioned. There is no value you can place on that. The internet is a great resource, but can also be a terrible place. I'd include a warning that some groups are Open and some are Closed, so add yourself with that in mind. More people should inform themselves and make careful choices.

Anonymous

PEN, May 2018

Excellent issue! I love that you put a spotlight on "fake news" and the dangers of social media, and the Hemlibra article was outstanding. Well done, to you and your team! Really outstanding work, and such a valuable resource to our community.

Ann Marie Minicheiello
Massachusetts

I advise everyone to keep to publicly available information regarding Hemlibra, and any complications experienced by those using it. "I heard from so-and-so" or "I suspect this-or-that" hearsay in this matter is not helping anyone, especially when it involves the further dissemination of conjecture, alarmism, or unsubstantiated opinions. Hemlibra presents a radically new paradigm and monumental change for people with hemophilia. For some, such change is scary and daunting, and they handle it poorly by contributing to rumor mills and innuendo, or outright personal attacks.

A good strategy when it comes to Hemlibra, or any emerging therapy, is to stick to facts that can be quickly and easily verified by checking scholarly, not community, resources. Look for press releases from pharma, the FDA, or clinicians involved in the study and publishing their findings legitimately. Read legit medical journals. Fact-check, verify, and most of all, if someone makes an accusation of risk or ill effect, make them provide legitimate proof.

Jeff Johnson
Washington

Excellent article about Hemlibra! Another thing you may want to address is the change in the hemophilia support programs. I've heard from several places that pharma is cutting back on the funds it's sharing and donating to the community. I don't think change is bad—it means less support services, but that's because the support isn't needed. Something the community wanted, to be normal, is on the very near horizon. I'm totally taking PEN with me to the Inhibitor Summit!

Carri Nease
Maryland

Project SHARE

We are thankful and indebted to you for the ever-timely donations. We have been able to treat so many bleeds that would otherwise pose danger to our boys. We would never be this happy and confident about hemophilia care for our boys without your kindness and help.

Sarah Mumbi
Jose Memorial Haemophilia Association–Kenya

Thanks for your great support during my bad times, and for funding my lab diagnosis. I would like to remember such support throughout my life, and if I get a chance to repay in any other means, then I would make every possible effort to do that for you. Thanks once again!

Nehaal Sashi
Fiji

My deep gratitude for extending your timely help in treating my father by sending the inhibitor medicine, which improves his health. Thanks for your valuable support once again.

S. Harish
India

We are extremely thankful to you for the amazing support in making this donation happen, and for spreading happiness in our lives.

Muhammad Yousaf Awan
Pakistan

On May 21, Anjho Quinalayo had a subdural hemorrhage. I received a call from his brother requesting factor IX for the brain bleed. We are lucky that we still have reserve factor IX for emergencies like this. On the same day, I immediately sent 18,000 IU, all of our high-dosage factor, for him. We thank Laurie Kelley, Zoraida Rosado, and the Project SHARE team for your support, and also Father Don Kill. God bless!

Raymund Ñaños
The Philippines

Anjho Quinalayo of the Philppines

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