



## How is von Willebrand Disease Diagnosed?

by Renée Paper, R.N.

### inside PEN

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The following chapter is excerpted from *A Guide to Living With von Willebrand Disease*, the first book devoted exclusively to helping people live with the world's most commonly inherited bleeding disorder. Free copies of this comprehensive resource are available from LA Kelley Communications, Inc. at (800) 249-7977 or [www.kelleycom.com](http://www.kelleycom.com).

Von Willebrand disease (VWD) is a complicated disorder, with different subtypes and symptoms that may vary from person to person and change over time. Because VWD is not very well known, some healthcare providers may not have ever heard of it. If they have heard of it, perhaps they have never seen a VWD patient before. The tests used to diagnose VWD are often complex and may not be available at all labs or testing facilities. For these and other reasons, if you suspect that you have VWD, have a thorough evaluation performed by a **hematologist**, a doctor who specializes in bleeding disorders, to get an accurate diagnosis. This evaluation should include a detailed personal and family medical history, a physical examination, and an accurate laboratory evaluation. If all three of these things are not done—or are not done correctly—it is possible that the diagnosis could be missed.

*Our doctor explained how the diagnosis was made. His report—18 pages long—began with my son's history when he first began to experience bleeding problems. The report continued with our family history and any bleeding problems we had. The next section contained the results from the platelet studies, then plasma coagulation studies, followed by comments based on the laboratory tests.*  
—J.O., Minnesota

Family history is important because the doctor<sup>1</sup> may not be looking for a bleeding disorder. For example, if a woman tells her gynecologist that she has heavy menstrual bleeding, the doctor may not even consider a bleeding disorder. However, if she also says there is a history of heavy bleeding in the family, the doctor might consider VWD as a possible cause. The physical exam is important to help rule out other causes for the symptoms. Finally, if the possibility of a bleeding disorder is considered, the doctor may order general screening tests that are of little or no value in diagnosing VWD. To diagnose VWD, specific tests for VWD must be ordered. Yet, if the doctor does not know about these tests, they won't be ordered.

#### Patient and Family History

The doctor should ask about your family history. Because VWD is most often **inherited**, it is common for many family members to have it. However, because VWD often goes undiagnosed, none of the family members may know they have it until one person is diagnosed and the rest are subsequently tested.

There are also specific questions that the doctor or nurse should ask about your bleeding symptoms. Table 2 lists some of these questions. If you are not asked about your bleeding symptoms, be sure to offer this information.

*continued on page 7*

<sup>1</sup> Not all patients see a doctor; some may see a physician's assistant or a nurse practitioner. The information provided here applies no matter which healthcare provider you see.

# welcome

## You'll notice a big change in

**PEN this issue!** We have a new look, new colors, new column, and a new name. The new look represents the recent changes in our company. We've expanded our services and programs, which we'll describe in a future issue. Our new colors, green and black, reflect our company colors: green represents the Irish heritage of our name, and black represents power, and the unknown—the field of possibilities. Empowerment and possibilities are what we promote, and what we believe in. Our new column is **Insights**, by Paul Clement, father of a child with hemophilia, and our Contributing Editor.



Why the new name? I started *PEN* in 1990, following the release of *Raising a Child With Hemophilia*. The idea for *PEN*, like so many of our ideas at LA Kelley Communications, came from a parent. A mother from North Carolina called to ask, "Now that the book is done, why can't we continue to share stories somehow, parent to parent?" She gave me the idea to start a newsletter *by* and *for* the parents. I envisioned a forum where parents could directly exchange ideas, share stories, and air concerns.

At first, our Mailbox section was the largest part of *PEN*, reflecting the overwhelming need for parents to connect. Now, Mailbox has shrunk, as more and more families connect by email and frequent meetings. While we didn't want to change the well-known acronym *PEN*, we realized that we are less an "Exchange" now; we're more about "Empowerment." We provide you information, we strongly encourage you to use it, and we show you *how*. Our strength now lies in our reporting, from a unique perspective—our contributors are almost all parents, patients and families, and our newsletter is for parents, patients and families. That's the one thing that will never change!

## letters

The May edition of *PEN* was fabulous. I thought I had received the majority of publications—was I wrong! I never knew so many biographies existed.

**Michelle Higgins**  
CALIFORNIA

I know it's tough to list all of the books in the world that relate to hemophilia, but I was surprised that *Blood Saga* by Susan Resnik didn't make the list. It is the most accurate account of the history of hemophilia I've ever read.

**Randall Curtis**  
CALIFORNIA

## PARENT EMPOWERMENT NEWSLETTER AUGUST 2002

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

*PEN* does not promote individual products or companies, and will use brand product names and company names pertaining only to news and education.

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*Funding provided by generous grants from our corporate sponsors (page 15)*

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### Are you interested in submitting articles to *PEN*?

*PEN* is looking for medical professionals, advocates and consumers with good writing skills to submit articles. *PEN* pays \$800 for original feature articles, and \$50 for As I See It. For submission guidelines, contact us at info@kelleycom.com. *PEN* will work with authors on editing and content but cannot guarantee that submissions will be printed. Overseas authors welcome!

I am thrilled to know that the information on Amicar® has been sent to so many people with hemophilia [LA Kelley Communications direct mailing, May 2002]. My grandfather, who had moderate hemophilia A, was a case study for Amicar about 30 years ago. His hematologist in Pontiac, Michigan, actually removed his gall bladder using only Amicar—no blood products or other medications. This operation, and the removal of some moles on my grandfather, appeared in medical journals. Unfortunately, at that time so little was known about hemophilia that other hematologists dismissed the study. Most hematologists to this day do not use Amicar to its potential in hemophilia. I hope this information will cause a spark in the hemophilia community and everyone can benefit from this wonderful drug.

**Nancy Rasch**  
OHIO

# Theme Park Summer Fun

## How to Relax While Weightless

Summer is here, and millions of families will seek adventure and thrills at theme parks like Six Flags, Disney World or Universal Studios. What a wonderful day a family can have at a theme park! Kids and parents love the rides, water slides, entertainment and general excitement. Are there any special safety concerns or precautions for families with a child with hemophilia? Yes!

Think of a theme park as a very big playground, but with an atmosphere favoring less parental control—a setting that can lead to impulsive behavior by children.

Imagine yourself at a typical theme park. You'll have general health concerns. Parks can be crowded, hot and sunny—so apply sunblock and drink plenty of water.

Theme parks have paved surfaces, harder than public playgrounds, and filled with children running. Your child needs to wear appropriate footwear. Flip-flops or “Tevas” might be suitable for water activities, but sneakers are safer for walking and running.

You'll also have concerns specific to the theme park you visit. “Mind Eraser,” “Shockwave,” and “Nitro”—what about these special high-end rollercoaster rides? Riders are frequently subjected to changing speeds that result in “G-forces” similar to those experienced by trained, appropriately suited and restrained combat fighter pilots. Your child becomes “Top Gun” in shorts and a T-shirt! Parents should remember that any person can experience head trauma on these specialized rides.<sup>1</sup>

How is the head affected by a ride like the Mind Eraser? Recall that your brain is surrounded by fluid; it is floating inside your skull. This arrangement cushions the brain, and reduces movement, protecting your brain from direct trauma and sudden shifts in skull position. It works quite well in our daily activities, and in automobiles (as long as we're wearing a seatbelt). Now imagine speeding over the crest in a roller coaster. All of a sudden you're weightless, like an astronaut—this is called a **Negative G-force**. (You'll have no trouble recognizing this moment, because everybody screams!) Then, after the coaster speeds

down and resumes its climb, you feel your backside being pushed into the seat. It feels like the force of gravity has suddenly increased. This is a **Positive G-force**. Although your body is restrained, high G-forces could exceed the protective cushioning of fluid surrounding the brain, and could cause injury.

Interestingly, this year New Jersey became the first state to seek legal restrictions on the maximum allowable G-forces on amusement park rides. The regulations result from concerns raised by physicians about the association between neurologic damage and high G-forces on these rides. Certainly, such injuries occur very infrequently, but serve as a cautionary note to all riders of high-end roller coasters—with hemophilia, or without.

My advice? Take some precautions. Level the playing field by giving your child a prophylactic infusion of factor the morning of your visit to a theme park. Yesterday's dose is not sufficient! Don't wait until your child reports the symptoms of a bleed—it may be too late. Besides, your child is not going to report the flop he took running to the haunted house until you're stuck in traffic on the long, long ride home.

Infuse first, then have a great time!

*Dr. Lipton is the Physician in Charge of the Hemophilia Treatment Center at the Long Island Jewish Medical Center. As a United States Air Force Physician (1966–1968), Dr. Lipton knew several fighter pilots, who took him on “joy rides” (with lots of G-forces) that more than fulfilled his childhood fantasies.*



Testing G-forces for fun: Dr. Lipton in his younger days.

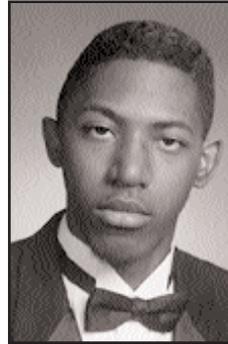
<sup>1</sup> “Study Says Thrill Ride Injuries on the Rise,” Kim Toshino, *LA Times*, February 4, 2002. [www.LATimes.com](http://www.LATimes.com)

# 2002 Eric Dostie Memorial Scholarship



**ANA BALDIVIAS** wants to become a medical coding person in the field of Health Information Technology. She enjoys reading and spending time with her children. "I want to be an example for my kids; I want them to see that anything is possible when one dedicates efforts in whatever one is doing."

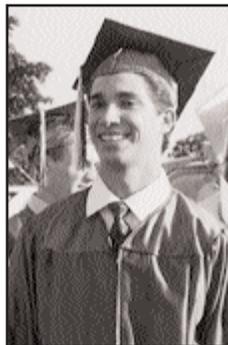
**MICHAEL FITZPATRICK** plans to work in the field of early education. He enjoys theater, playing music, and strategy games. "I learned that having a bleeding disorder does not define who I am or how successful I can be."



**CHAUNCEY FULLER** plans to study biology/pre-medicine. His ultimate goal is to become a pediatric hematologist/oncologist. Chauncey enjoys playing golf, bowling and cycling. "I have an overriding need to help others and to be a role model and spokesperson for children with disabilities."

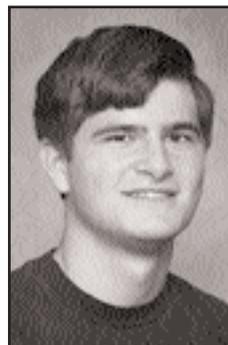
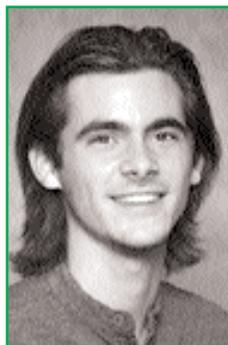
## congratulations winners!

**JOSHUA LUNIOR** plans to pursue a career in education. He enjoys listening to music, collecting baseball cards, and kayaking. "My determination will give me the strength to keep pursuing my dreams, no matter what obstacles come my way."



**BEAU OYLER** plans to graduate from the Academy of Art in San Francisco and start a business that will help ease the path of children with hemophilia and handicaps. Beau enjoys painting, surfing, and teaching handicapped children. "Never has my life been easy, but it has been full of hard work and high rewards."

**BEAU WARD** plans to pursue his master's degree in education. He enjoys swimming, cycling and reading. "As far back as I can remember, I have always been curious and excited about learning."



**TRAVIS WARD** plans to become a special effects artist in the film industry. He enjoys painting, reading, hiking and mountain biking. "My focus has been finding fulfillment in my endeavors, and trying to share all that I enjoy with others."

If you know someone who is eligible to apply for this scholarship, please contact LA Kelley Communications, Inc. after November 1. Also check our website for a list of all scholarships.



# Factor Shortage OVER!

It's official: The shortage is over. Spring of 2002 brought many signs of hope to our community. It will be easier now to obtain recombinant factor VIII and, more important, there will be enough for all. Let's look at three big changes that will directly affect us.

First, this spring **Bayer** announced that its release of Kogenate® FS is steadily increasing. Inventories of the 1000 IU vials of Kogenate FS and KOGENATE® Bayer now stand at a three-to-six-month supply. Releases of 500 IU vials will begin in May; and by the end of this summer, Bayer will begin shipping 250 IU vials.<sup>1</sup>

How does this compare to 2000, Bayer's best supply year? Quarterly rFVIII releases currently exceed 80% of average quarterly releases in 2000. And releases should steadily increase, exceeding their 2000 quarterly average by mid-2003.

Second, **Wyeth Pharmaceuticals** (formerly Genetics Institute) gained approval in May from the European Commission for its ReFacto® manufacturing facilities in St. Louis, Missouri and Madrid, Spain (neither facility yet has U.S. FDA approval). Previously, all ReFacto sold in the U.S. and Europe was produced in Stockholm, Sweden. As the St. Louis and Madrid facilities boost the amount of ReFacto available to the European Union, more product from the Stockholm facility will be available to the U.S. and other countries. Wyeth expects the amount of ReFacto available in the U.S. to triple almost immediately.

Third, **Baxter Bioscience** continues to ramp up its rFVIII production. During the shortage, Baxter Bioscience produced record amounts of RECOMBINATE™ rAHF. It also received FDA approval to produce RECOMBINATE in "Suite C," its third production suite in Thousand Oaks, California. Baxter is now constructing "Suite D," a fourth processing suite, and expects to receive FDA approval by late 2004. With Suite D and its new plant in Neuchâtel, Switzerland, Baxter predicts that it will produce more than *two billion units of rFVIII per year* by 2005, and *2.5 billions units per year* by 2006. That's great news for people in the hemophilia community who were concerned about future supplies of rFVIII.

Does this really mean the shortage is over? Well, the Canadians seem to agree. In May, the **Canadian Hemophilia Society** (CHS) Blood Safety Committee announced an official end to the shortage.

It also recommended completely removing the conservation guidelines it adopted in April 2001. This is unbridled optimism from a country that suffered greatly from the factor shortage—Canada depended on Bayer to supply more than 90% of its FVIII products!

But the U.S. is more cautious. The **National Hemophilia Foundation** is not as eager as the Canadians to completely lift factor conservation guidelines. Believing that many consumers are still rationing factor, the NHF is concerned that the present high inventory levels of rFVIII may give a false impression of the actual factor supply. So, although the NHF has relaxed its conservation guidelines and recommended a 15% increase in the use of rFVIII, it has also adopted a "wait and see" approach before announcing an end to the shortage.<sup>2</sup> Is the NHF overly cautious?

Before you answer, consider this: Neither the FDA nor Bayer BP was forthright about FDA observations at Bayer's Berkeley plant in November 2000. The FDA refused to supply the NHF with a report of its observations—until the NHF requested the report under the Freedom of Information Act. NHF officials believe that *if the bleeding disorders community had been properly informed, proactive measures might have lessened the impact of the factor VIII shortage* when it peaked last summer. The NHF also contends that because the FDA findings were not shared earlier, members of the community were forced to make important decisions, on short notice, about health and lifestyle. Had our community received access to full information from the beginning, the impact of the shortage could have been softened.

Has anything changed? The NHF and FDA have now reaffirmed their common goal of serving the bleeding disorders community, and agreed to continue working together cooperatively.

So what does the news of ample rFVIII supply mean for you? It means that once again, you can use the *product of your choice*. You can start that Immune Tolerance Therapy, or proceed with that postponed elective surgery. You can return to your normal prophylaxis routine, which may have been reduced or eliminated during the shortage. You can get back to life, without worrying about the shortage.

But let's not forget the recent past. Remember to keep your eyes on the horizon of factor production. Educate yourself, read, stay informed... and be prepared to make proactive decisions about the products you use, just in case we're faced with another serious shortage.

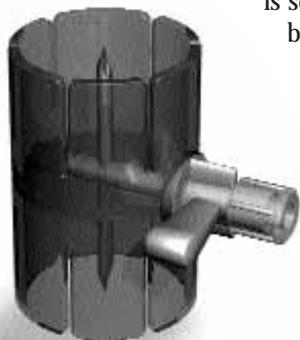
**Baxter predicts that it will produce more than two billion units of rFVIII by 2005.**

<sup>1</sup> Bayer also provides Aventis Behring with rFVIII, which is distributed as Helixate® FS.

<sup>2</sup> For all NHF recommendations regarding the shortage, visit the NHF website at [www.hemophilia.org/home.htm#](http://www.hemophilia.org/home.htm#)

## BAXJECT Device Unveiled

In a survey of 78 U.S. patients with hemophilia A, 30% had stuck themselves with a transfer needle, and 77% expressed concern about transfer needle sticks. To address these concerns, **Baxter Healthcare Corporation** has introduced the **BAXJECT**, the first needleless transfer device. It works with all of Baxter's hemophilia therapeutics, and replaces the metal double-ended transfer needle and the filter needle, thus avoiding needle sticks from these sources. Using BAXJECT is safer than using needles; and faster, because it eliminates some steps in reconstitution. BAXJECT is so safe and easy to use that children can become involved in the reconstitution process earlier.



The BAXJECT

Baxter will provide educational materials for consumers and health care professionals during the transition phase from the needle-based reconstitution system to the BAXJECT. Beginning this summer, Baxter will include **both** needles and the BAXJECT with its factor concentrates. Once Baxter has determined that consumers are comfortable using the BAXJECT, the device will replace needles in the package. For further information, visit [www.hemophiliagalaxy.com](http://www.hemophiliagalaxy.com).

## New Protein-Free rFVIII

**Hemophilia 2002**, the congress of the World Federation of Hemophilia, was held May 19–24 in Seville, Spain. **Baxter Healthcare** presented the first clinical data on its investigational protein-free recombinant factor VIII product, **rAHF/PFM** (recombinant Anti-Hemophilic Factor/Protein Free Method). Now in phase III clinical trials, it will be the first third-generation recombinant factor VIII product.

First-generation recombinant factor products are stabilized with human albumin. Second-generation products have no albumin added as a stabilizer. Third-generation products use no human or animal raw materials in the cell culture process, purification, or final formulation. They have been developed in response to concerns about transmitting unknown viruses, or diseases like CJD (Creutzfeldt-Jakob Disease), through the use of human or animal-derived proteins. Tests indicate that rAHF/PFM is as effective as RECOMBINATE™, Baxter's first-generation rAHF product. Baxter hopes to license rAHF/PFM in 2003.

## Gene Therapy Update

**GenStar Therapeutics** reports that the first severe hemophilia A patient enrolled in the company's U.S. phase I trial continues to sustain a circulating factor VIII expression level of approximately 1%, eight months following a gene therapy treatment. Factor VIII levels less than 1% are characteristic of severe disease. The patient was infused with "MAX AD Factor VIII," a gutted adenoviral vector containing the entire human factor VIII gene sequence, along with a "molecular switch" designed to increase factor VIII expression in the liver. GenStar plans to initiate a phase I clinical trial in Europe in 2002. *Source: IBPN, May 2002*

## Flying With Factor Concentrates?

Since the terrorist attacks of September 11, several people carrying factor concentrates have had problems at North American security checkpoints. At the Ottawa Airport, one Canadian traveler was forced to remove the caps on vials of both his factor VIII concentrate and sterile water—despite the fact that he carried a letter from his doctor certifying his condition, and the need to carry blood products. While these incidents are isolated, you should carry explicit information when traveling. A doctor's letter should include the following:

- Information about your medical condition
- The fact that you need to carry factor concentrates when traveling
- The number of vials
- The lot numbers
- Instructions that the vials should not be opened for any reason
- A prescription for your medication with contact information

Travelers carrying syringes and needles inside hand luggage should have their factor concentrates with them—to explain the purpose of the needles. The U.S. Federal Aviation Administration (FAA) allows syringes and needles on board aircraft when accompanied by medication with a professionally printed label identifying the medication or manufacturer name. Keep factor concentrates in your hand luggage, since checked baggage can be lost. Contact your airline at least two days prior to departure for specific regulations regarding the transport of medications.

*Source: Canadian Hemophilia Society, Hemophilia Today, Spring 2002, Vol. 37, No. 1; FAA news release.*

## Questions Your Doctor Should Ask

### Nosebleeds

- Do you have nosebleeds? If so, how often do they occur? Do you ever need to have your nose packed or cauterized?
- How long does your nosebleed last, what stops it, and how often after it stops does it rebleed?

### Bruising and Bleeding

- Do you have a history of excessive bruising?
- Did you have significant bleeding when you lost your baby teeth?
- Do you experience prolonged bleeding after cutting yourself while shaving?
- Have you ever had excessive or prolonged bleeding following surgery, dental work, childbirth, or injury?
- Have you ever received a blood transfusion? If yes, for what reason?

### Menstrual Bleeding

- How many days does your menstrual period last?
- How many pads or tampons per day do you use? How often do you change them?
- Do you use a tampon and a pad together or more than two pads at a time?
- Do you miss time from work or school due to heavy periods?
- Do you frequently stain through your underclothes?
- Have you ever been diagnosed with anemia?

Many family members do not know the “normal” length of time for bleeding to stop following surgery, dental work, or even childbirth. They often define normal by comparing themselves to other family members, who may also suffer from VWD. Other affected family members may have blurred ideas of what is normal. This is especially true for menstrual bleeding. If your mother and her mother also had heavy periods, you and your family may think that abnormally heavy menstrual bleeding is normal.

*Frequent heavy nosebleeds and menorrhagia were my clues to seek medical help. My children were 16 and 13 when they were diagnosed. When the parent is also affected, norms for bruising and menorrhagia are difficult to identify.*

*My daughters and I have exceptionally heavy periods. Until my mother was diagnosed with von Willebrand disease in 1997, I had no concept of how to gauge a normal menstrual flow. I now know that I hemorrhaged for three weeks after the delivery of one of my babies, but at the time, I did not know how to relate the information to the OB by phone. The doctors couldn't understand why I was so anemic for the next few months. When many large bath towels are needed, there is indeed a problem! —J.C.W., Iowa*

### Physical Examination

After gathering information about your bleeding and family history, the doctor should perform a thorough physical examination. Although the exam is not likely to shed much light on VWD

as the cause of bleeding, it will help rule out other possible causes, such as benign or malignant growths or other problems in your body. For example, heavy or prolonged menstrual bleeding may also be a sign of cervical or uterine cancer or uterine fibroids (noncancerous growths in the uterus). Therefore, a thorough pelvic examination is imperative to rule out these other causes of excess menstrual bleeding. However, it is certainly possible for a person with any of these other causes of bleeding to also have VWD. Sadly, many women have been told that the cause of their heavy menstrual bleeding was uterine fibroids and have had a **hysterectomy** (removal of the uterus)—only to learn later that they also had VWD. The VWD was likely the cause of heavy bleeding, and the hysterectomy could have been avoided.

If both fibroids and VWD can cause heavy bleeding, how does the doctor determine the real cause? Only a thorough history, physical examination, and appropriate laboratory tests can help pinpoint the problem. However, unless the doctor suspects VWD and knows which tests to order, where to send them, and how to interpret them, the actual diagnosis may be missed.

A similar scenario can occur with other types of bleeding, such as nosebleeds. Nosebleeds can be caused by benign nasal polyps (growths inside the nose) or by a deviated septum (problem with the bony cartilage inside the nose). Again, it is possible for someone to have VWD along with polyps or a deviated septum. If VWD is not diagnosed and surgery is performed, excess bleeding (sometimes called a hemorrhage) during or after surgery can be a tragic consequence. If VWD is diagnosed and surgery is still recommended, proper treatment before surgery can prevent hemorrhage.

### Laboratory Testing

Obtaining an accurate laboratory diagnosis can be a daunting task for several reasons. First, there are many subtypes of VWD—some involve a shortage of **von Willebrand factor (VWF)**, others involve abnormal VWF structure or function. It is a complex process to test for all of the different subtypes. Second, the tests currently used for making a VWD diagnosis are not readily available in most community-based labs. Some tests are sent out to reference labs that do not have the specialized equipment or trained staff to perform the complex testing. Third, many primary care doctors are unfamiliar with the tests required to diagnose VWD and which labs to use. Finally, many healthcare insurance plans have contracts with certain labs that may not specialize in complex coagulation (blood clotting) testing.

Consult with a **Hemophilia Treatment Center (HTC)**<sup>2</sup> (a specialized medical center or clinic for people with bleeding disorders) in your area to find out which labs perform these specialized tests accurately and reliably. Even if your insurance policy does not allow an HTC to perform the testing, the HTC will still help you find a reliable lab. Ideally, laboratory analysis should be done on site and where your blood was drawn. Shipping is often discouraged because of the risk of improper handling of your blood sample, which

<sup>2</sup> To locate the nearest HTC, call the National Hemophilia Foundation at (800) 42-HANDI or visit its website at [www.hemophilia.org](http://www.hemophilia.org).

can interfere with results. If it is impossible for you to be seen by the HTC lab, at least request that your blood sample be shipped there for analysis.

*The local hospital didn't have equipment for processing factor VIII tests, so the blood would be drawn, put in a cup of ice, and transported to another local hospital via taxi. This is the method they used for one year whenever my daughter's doctor ordered a factor VIII count (which was twice a week every week for more than a year). They didn't know anything about von Willebrand disease. —S.H., Pennsylvania*

Even proper tests done in the best labs may be inconclusive because of the nature of the disorder. For example, in type 1 VWD, the levels of factor VIII and VWF fluctuate and may sometimes appear normal. Repeated testing, once or even twice, is recommended before the diagnosis of VWD can be ruled out.

### Influences That Affect VWD Test Results

After recording your family and personal medical history and performing a physical exam, the doctor will order laboratory tests. When having VWD laboratory tests done, it is important to know that many things can affect VWF levels. Consider the following when you, or your child, undergo coagulation testing.

#### Influences That Raise Clotting Factor Levels

The following medicines and conditions may mask VWD on laboratory testing because they temporarily raise VWF and factor VIII levels.

*Crying, exercise, stressful events, recent surgery, and infection.* All of these stimulate the release of a chemical called *adrenaline* (a hormone; also known as *epinephrine*). Increased amounts of adrenaline in the body stimulate the release of VWF and factor VIII from storage sites in the lining of the blood vessels. This may cause VWF and factor VIII levels to *rise into the normal range* on the day of testing and may produce a negative test result. This scenario usually occurs in people with **type 1 VWD**. Although factor VIII and VWF levels may also rise in people with **type 2 VWD**, the abnormal functioning of the VWF does not change. People with **type 3 VWD** have little or no change in VWF levels, as they make and store almost no VWF.

Any stressful situations that occur in the hours preceding the testing can affect the test results. Walking a long distance from your car to the lab, an argument with your spouse the night before, even crying during the blood test (as children often do) can raise VWF and factor VIII levels into the normal range on test day, making the test results unreliable. It is also advisable to avoid strenuous exercise before being tested and to delay testing until any active infection has subsided. Also, try to keep children calm during a blood test. Ask the doctor to use a topical numbing cream, such as **EMLA®** (lidocaine 2.5% and prilocaine 2.5%), to decrease pain. If testing is inconclusive for any reason, repeat testing may be necessary at a later date.

*Recent transfusion.* As obvious as this may seem, you should not be tested for VWD if you have recently received a transfusion of another person's blood or a component of blood such as plasma. The blood donor's clotting factors will interfere with the evaluation of your own levels.

*Hormones.* The hormones estrogen and progesterone, like those found in birth control pills, raise the level of VWF and factor VIII in the blood. This is one of the reasons that hormones, usually birth control pills, are prescribed to women with VWD. They may help raise levels and prevent excessive bleeding. A woman should refrain from taking these medicines three to four weeks prior to undergoing coagulation testing. However, always check with the doctor first because stopping the hormones may cause bleeding. Also, if you are of reproductive age, sexually active, and do not wish to become pregnant, you need to consider another form of birth control when you are not taking these hormones.

*Menstrual cycle.* As when using hormone supplements, a woman's body experiences hormonal ups and downs with the changes of her menstrual cycle. At the time of ovulation, these levels are naturally at their highest in the body. During menstruation they are at their lowest. Because of this, if a woman is of child-bearing age (usually age 12 to 50), it is advisable to *have coagulation testing performed during menstruation* when levels are at their lowest. Recent studies show the lowest levels of VWF and factor VIII occur on days five to seven of the menstrual period.<sup>3</sup>

*Pregnancy, childbirth, and breast-feeding.* These conditions and activities raise estrogen levels in the body and, subsequently, VWF and factor VIII levels. For women with type 1 VWD, the elevation of VWF and factor VIII levels may actually protect the mother from excessive bleeding at the time of delivery. Elevation of factor VIII and VWF levels makes it nearly impossible to make a correct type 1 VWD diagnosis in a woman who is pregnant, has recently delivered a baby, or is breast-feeding. However, for women with type 2 or type 3 VWD, diagnosis during pregnancy is possible. The increased hormones do not alter the abnormal structure or function of VWF in type 2 or stimulate production of VWF in type 3. Women with VWD are at significant risk for excessive bleeding one to two weeks after childbirth. This happens because hormone levels, and therefore factor VIII and VWF levels, return to prepregnancy levels after delivery.

#### Influences That Lower Clotting Factor Levels

The following conditions may affect the diagnosis of VWD in laboratory testing because they *lower* VWF and factor VIII levels.

*Hypothyroidism.* The thyroid gland secretes a hormone needed for proper metabolism. Hypothyroidism (abnormally low thyroid function) sometimes can cause a low level of VWF. This is one example of **acquired VWD**. Correction of the thyroid disorder should correct the VWF level. If it does not, then you may also have an inherited form of VWD.

*Blood type.* Your blood type is determined by the presence or absence of certain proteins in the **red blood cells**. The major blood types are type O, type A, type B, and type AB. People with type O blood have naturally lower levels of VWF than people with the other types, yet they *may not* have VWD. If you do not know your blood type, make sure the doctor tests your blood type at the same time you have the other blood tests. Most coagulation disorder specialists will consider your blood type when interpreting the results.

<sup>3</sup> Kouides PA. Von Willebrand's disease: recognition and management. *The Female Patient*. 1999; 24:86-94.

## Influences That Interfere with Platelet Function

The following medicines and conditions interfere with **platelet** or blood vessel function and may interfere with laboratory evaluation of VWD.

**Pain medicines.** Certain medicines such as **aspirin** and most nonsteroidal anti-inflammatory drugs (NSAIDs) decrease platelet “stickiness,” prolong bleeding time, and interfere with platelet function studies. *People with bleeding disorders are advised to avoid these medicines* because they can increase the likelihood of bleeding. It is especially important to stop these medicines three to four weeks before testing. In 1999, a new class of NSAIDs, called Cox-2 inhibitors, was approved by the FDA. Cox-2 inhibitors (e.g., Celebrex® [celecoxib capsules] and Vioxx® [rofecoxib tablets]) as well as two of the older NSAIDs (Trilisate® [choline magnesium trisalicylate] and Disalcid® [salsalate]) are reported not to interfere with platelet function.

**Other medicines.** Other medicines that have similar effects and can interfere with testing include quinine, guaifenesin, and penicillin. Quinine is a medicine used for leg cramps, malaria prevention, and heart rhythm disturbances. It is also an ingredient in tonic water, a mixer used with alcoholic beverages. Guaifenesin is an ingredient in many cough syrups, such as Robitussin®. Penicillin and related antibiotics can also interfere with platelet function. Before the tests, review with the doctor all of the medicines—prescription and over the counter—that you are taking. To ensure accurate results, these medicines need to be stopped prior to testing.

**Alcohol.** Alcohol in any form may interfere with normal blood clotting and platelet function. Avoid alcohol for several days prior to testing.

**Foods.** Certain foods, such as fish, contain high amounts of fats, called omega-3 fatty acids, that are known to decrease platelet stickiness. Eating a normal amount during a meal should not cause a problem. However, if you have or suspect a bleeding disorder, *do not take capsules containing these fish oils.*

**Vitamins.** Certain vitamins, such as vitamin E, decrease platelet stickiness and can interfere with testing. They can also increase the chance of bleeding if you have a bleeding disorder. The amount of vitamin E in a normal meal, or even in a daily multiple vitamin, should not cause a problem. However, taking extra vitamin E supplements may interfere with platelet function studies and should be avoided.

**Herbs.** Herbs and herbal supplements, including ginkgo biloba, feverfew, Pau d’Arco, ginger, ginseng, and echinacea, may affect platelet stickiness and interfere with platelet function testing. Before taking any herb you should familiarize yourself with its actions and understand the precautions for its use. It is advisable to stop all nonessential vitamin and herb supplements at least one week prior to testing to avoid possible interference with test accuracy.

## Laboratory Tests (Assays)

As you may have already noted, testing for VWD is not a perfect science, but it remains the only way to confirm or rule out a VWD diagnosis. An **assay** is a laboratory test that measures the quantity and/or quality of a substance. A clotting factor

assay measures the quantity of a specific clotting factor in blood. Your doctor may order two types of blood tests to see if you have VWD: **primary screening tests** are used to discover or rule out other disorders; **secondary screening tests** are used to confirm or rule out VWD.

Primary screening tests are not specific for VWD. These blood tests will usually be normal in people with VWD. These are the most common tests used by primary care doctors to rule out a bleeding disorder. Your doctor may not know that these tests are not specific enough to determine the presence or absence of VWD. When primary screening tests come back normal, many doctors assume that the patient does not have a bleeding disorder. This is a potentially dangerous assumption. More specific secondary testing should always be done, especially if there is *suspicion* of VWD. Secondary tests have a higher degree of sensitivity for diagnosing VWD. Specialized coagulation labs should perform these secondary tests.

## Primary Screening Tests

**Complete blood count (CBC).** This test examines the white and red blood cell content of the blood. It also includes a measurement of **hemoglobin**. Hemoglobin is an iron-containing pigment that gives blood its red color and transports oxygen to body tissues. Low hemoglobin can signify blood loss or **anemia**. Some women with VWD have chronic anemia because of the excessive blood loss that can occur during menstruation. Unless you have recently bled excessively, your hemoglobin should be normal.

**Platelet count.** This test determines the number of platelets in the blood. The majority of people with VWD have a normal platelet count. In the rare VWD subtype 2B, the platelet count may be low.

**Bleeding time (BT).** This test measures how long it takes for the body to form a **platelet plug** at the site of injury. A BT test is done by applying a blood pressure cuff to the upper arm, pumping it up to maintain a specific pressure, making one or two small cuts on the inner forearm with a small surgical blade, then timing how long it takes for bleeding to stop.

Unfortunately, the BT test is poorly standardized: some labs use one incision, some use two; some use commercially manufactured blades, others do not; the amount of pressure applied by technicians when making the incisions varies. Other influences, such as the room temperature, can also interfere with the test’s accuracy. Because of these variables, the usefulness of a bleeding time test is limited. Many HTC’s no longer perform the test. An *abnormal* bleeding time test may point the doctor toward a possible diagnosis of VWD, but a *normal* bleeding time should *not* rule it out. Some doctors may assume that a normal bleeding time means the patient does not have VWD. This can be a dangerous assumption.

*My first son was diagnosed at age two and a half. When he was one, I learned that I had von Willebrand disease and asked my pediatrician if we should test my son. He said yes but, like most pediatricians, thought that since Andrew’s bleeding time was normal, he must not have the disorder. When my second son was born, we had both sons tested, and both were positive. My bleeding times have been good and bad, as have my children’s. I don’t feel that bleeding times were crucial in making the diagnosis. —A.S., Missouri*

**Prothrombin time (PT).** This test evaluates the level of **plasma clotting factors** I, II, V, VII, and X. Because VWD does not involve any of these clotting factors, a PT test is *always* normal in someone with VWD.

**Partial thromboplastin time (PTT).** This test evaluates the level of clotting factors I, II, V, VIII, IX, X, XI, and XII. Only factor VIII is sometimes reduced in VWD. In the majority of VWD cases, the level of VWF is not reduced *enough* to cause a significant deficiency of factor VIII, and therefore, the PTT is normal. The PTT test is prolonged when factor VIII levels fall below 30% (depending on the laboratory method). *Like the BT test, an abnormal result may point the doctor toward a possible diagnosis of VWD, but a normal test should not rule it out.* Unfortunately, a normal PTT may mislead an inexperienced doctor to assume that the patient does not have VWD.

*Our family doctor ordered a PTT to see if I had a bleeding disorder. It came back normal. I asked for more tests because I just knew I had von Willebrand disease—my bleeding episodes were numerous, some serious, and very real to me. My doctor was reluctant, but scheduled a bleeding time test. The results were normal, and he said, “You don’t have it.” He pointed out that I had had my tonsils and wisdom teeth removed and had borne four children. I became angry. I told him I had significant bleeding problems every month and insisted on more tests. He finally referred me to a hematologist—who properly diagnosed me with type 1 von Willebrand disease.  
—K.M., Connecticut*

## Secondary Screening Tests

**Von Willebrand factor antigen (VWF:Ag).** This test determines how much VWF is in the plasma. The result will be low if you have type 1 VWD and very low if you have type 3. It may be low or normal if you have type 2.

**Ristocetin cofactor (RCo) or VWF activity.** This test determines how well VWF is working, by examining its ability to clump platelets together or “stickiness.” Results are abnormal in all three main types of VWD. It may be normal in some people with subtype 2N. For the majority of patients, this is probably the best test available to diagnose VWD.

**Factor VIII activity.** This test measures the amount of factor VIII in the plasma. Factor VIII is always low in type 3 VWD, where there is an inadequate amount of VWF to act as the carrier of factor VIII. Because type 1 VWD also involves a deficiency of VWF, the factor VIII may be a little low in type 1. In type 2 VWD it can be low or normal, depending on the subtype.

**Von Willebrand factor multimer analysis.** This test examines the size and structure of the VWF molecule, and is helpful in diagnosing type 2 VWD and its subtypes.

**Ristocetin induced platelet aggregation (RIPA).** This is another test that evaluates the ability of VWF to cause platelet aggregation, or stickiness. This test is not very useful in making an initial VWD diagnosis. However, this test together with the VWF multimer analysis can be very useful in distinguishing type 2B VWD from type 2A VWD.

There is no perfect test to diagnose VWD with certainty. Researchers are trying to develop new tests to diagnose VWD more accurately and easily. This is a difficult task. Such a test

will have to be highly accurate, readily adaptable to community-based labs, easy to interpret, inexpensive to perform, and require little technical expertise. No such test currently exists.

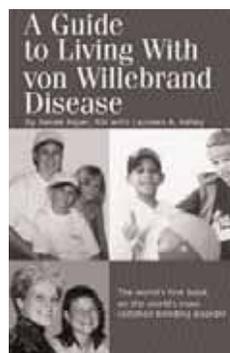
When testing for VWD, *repeated testing is the norm.* To help decrease the need for repeated testing, avoid the medicines and other influences discussed earlier in this chapter that could interfere with the accuracy of test results. Make sure that the lab performing the testing specializes in bleeding disorders. Also be sure that the doctor has expertise in diagnosing bleeding disorders and can interpret test results accurately. Doctors who specialize in bleeding disorders are usually associated with an HTC.

## Getting an Accurate Diagnosis

Keep in mind that the three most important elements of a VWD diagnosis are: (1) personal and family history of bleeding, (2) physical exam, and (3) specialized laboratory testing. If symptoms are present and you have a “gut instinct” that you or your child may have a bleeding disorder, don’t be afraid to ask to be tested.

*My daughter’s father has type 2A von Willebrand disease, and when my baby girl bled when her teeth came in at five months, I asked her pediatrician about von Willebrand disease. He told me to purchase a humidifier. At her nine-month checkup, I asked again; her pediatrician refused to speak to our hematologist and refused to order the tests. He thought I was imagining things. When her nosebleeds and bruising had become more frequent, I asked again, at her one-year checkup. No—not until she was old enough not to cry during the blood tests. When she was two, she had pneumonia. Every day she awoke with blood on her pillow. It broke my heart, and then I would not accept no for an answer. I demanded a referral and got one. She was diagnosed with type 2A von Willebrand disease. —C.D., Maine*

Also, if the tests come back normal, be persistent and request repeat testing. It should be clear now that a VWD diagnosis can be missed because of problems with testing. Knowing this may help you feel more comfortable asking the doctor to repeat the tests. Finally, if you feel that you or your child has a bleeding disorder that is not being recognized, don’t be afraid to ask for a referral to a hematologist or bleeding disorder specialist.



Contact LA Kelley Communications, Inc. for your **free** copy of *A Guide to Living With von Willebrand Disease*.

1-800-249-7977  
info@kelleycom.com  
www.kelleycom.com

Q

*A Guide to Living With Von Willebrand Disease* has helped me a lot. I was diagnosed with VWD after I gave birth to my son. While I was pregnant, I had serious and lengthy nosebleeds; I had to go to the hospital several times to stop them. I also bled from various parts of my body for no apparent reason. After my son was born, I bled non-stop for about nine months, and nobody knew why. I had to drive for six hours to another state to be diagnosed and helped. The doctors here just wanted to give me a hysterectomy, at age 20. I finally

**A** What you describe is known as von Willebrand Syndrome. The only way to be entirely sure which form you have is to have actual DNA analysis. The analysis would look for a defect, consistent with your form of vWd, on the genes within chromosome 12. Currently, there are very few labs in the world that can do this analysis. The only one in the U.S. with which I'm familiar is at the Blood Center of Southeast Wisconsin (Dr. Bob Montgomery). This test would be considered experimental, and would likely not be covered by health insurance.

As for your other family members testing negative, unfortunately, as you've read in the book, a negative test doesn't always mean someone does not have VWD. The testing is not yet perfect, and because VWF (von Willebrand factor) levels fluctuate so easily (due to stress, adrenaline and estrogen) the test results can also fluctuate. If the people tested *also* have no bleeding problems, I would give more weight to the "negative results." If, however, those folks have obvious bleeding symptoms—prolonged nose-bleeds, bruising, prolonged bleeding after surgery, injury, dental work or childbirth—I would not give much credence to the test results.

Were you and your family evaluated at a Hemophilia Treatment Center? Was the lab used indeed a specialty lab? Obviously, the more experienced the clinician and lab at evaluating people for VWD, the more likely the results are accurate.

As for your son, if you have an inherited form of VWD, it is possible to transmit VWD to him. If you have the very rare *acquired VW syndrome*, it is not possible to pass it to your son, since yours would involve not a genetic defect, but an acquired problem in which your body alters its own VWF levels or function. Differentiating which you have will certainly require an expert diagnostician and lab.

**Renée Paper, R.N., CCRN**

EXECUTIVE DIRECTOR  
HEMOPHILIA FOUNDATION OF NEVADA

found an excellent hematologist, two hours away. About six months after I was diagnosed with vWd, I was diagnosed with lupus (SLE). I had my family checked for VWD, but all tests came back negative. I thought I must have inherited VWD until I read Renée Paper's book.

Is it possible that I got lupus while I was pregnant, then got VWD because of that? My doctors said there can't be any connection. I don't see how I have gotten this, since I've never had any trouble before. Is it possible that my son could have VWD, when no other family member does?

**Michelle Brooks**

**Q** My husband and I moved from Belize to South Florida in 1987, for a better life and medical care for our two boys, now 17 and 16. They have hemophilia, factor VIII severe, and are doing well.

I was tested as a carrier about ten years ago, because we wanted to have another child. We have a beautiful eight-year-old girl, who has not yet been carrier tested. I was not told when I was tested that my factor levels were low. A few weeks ago, I started to have symptoms of prolonged bleeding. My test came back showing mild hemophilia. Since I have been caretaker for my boys, and for my husband who has diabetes, I am comfortable with this.

The problem is that my father that still lives in Belize, and has had for many years symptoms of prolong bleeding. He has been so emotional about his grandsons, and now his only child also has hemophilia. I need help encouraging him to get tested, so that if we have an emergency in the future, we would know what to do. I can't bring myself to ask him to get tested. We are very, very close but I always just tell him positive things, and I'm afraid that he would not take this well.

**Michelle Longworth**

FLORIDA

**Readers, please mail or email your answers to Michelle's question to LA Kelley Communications. Please indicate if you would like your response published in the next issue.**



*continued on next page*

My friends' two-and-a-half-year-old son is being treated every second day as part of Immune Tolerance Therapy (ITT) for an inhibitor.

He has had a port for a year, and is now in the hospital with a port infection. He has been treated with IV antibiotics aggressively for three weeks, and is on his second course, as the first course was unsuccessful. It looks like the port will have to go.

My friends are understandably reluctant to get another port, but the doctors believe that prodding his veins at such a young age could badly damage them. What are the chances of success with venous access at this age?

**Marelle Taylor**

AUSTRALIA

**A** My four-year-old son Simon has received factor through IV (peripheral) sticks three times a week all his life. I hope this didn't ruin his veins—we were never told it was a possibility. We didn't want a port for several reasons, mainly that we feared infection.

It depends on whether the boy's veins are relatively easy to access. If they are, you need someone who can do it. After driving to and from the hospital three times a week, we started home treatment a year ago. But the child has to cooperate; Simon cooperates at home better than he did in the hospital.

Do your friends want to administer the factor themselves, or could they could get a nurse to do it? I think the person should be able to do it in one stick. Everybody has bad days, but if it can't be done in one shot, you risk damaging the veins.

**Femke Meijer**  
THE NETHERLANDS

**A** Our son is also two-and-a-half, and if he had his port removed because of a stubborn port infection, we would like to consider venous access. At a hemophilia conference in New York City, we were told that if you use venous access, try to do it each time at one spot with one vein. Scar tissue will form, harden the vein, and make access easier with less chance of missing in the future. That surprised me, because I'd also heard the opposite—that scar tissue building up can hurt children's veins. This may be a matter of choosing between the risks: which is worse, scar tissue or risking a port infection?

**Elizaveta Temidis**  
NEW YORK

**A** My son was two when we had to remove his Broviac® because he was outgrowing it. We chose not to replace it. We learned to do venous access, usually in the back of his hand, sometimes in his arm. It was scary at first, but was one of our best decisions. All the complications surrounding the venous access device are gone, which is wonderful. We poke him at home with no problems.

Two things are unusual about our situation. First, we knew he'd outgrow the Broviac eventually, so we started learning to poke him while he still had it. If we had trouble, we had the Broviac as a back-up. Second, our son needs factor only once every four days instead of every two days, which gives him more time to recuperate after we stick him. If your friend's son has a few good veins, they might be able to alternate spots. We are able to use the same spot each time.

Your friends could ask an IV nurse to examine their son's veins, to determine whether they are "good." When the doctors poke him for blood tests, do they normally find a vein easily? If yes, and if your friends feel confident and competent, they should consider going without a port.

**John Vergara**  
NEW YORK

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**A** My son is factor VIII severe, started prophylaxis at age four, and gets infused every other day. He has never had a port. Before he started prophylaxis, he had frequent shots because he was so active. We have had no problems with venous access. He has pretty good veins, and we rotate four or five veins in his hands and arms.

One particularly good vein was used often by the ER doctors, and I thought it was becoming harder to get the butterfly through the scar tissue. We talked to the doctors, and tried to avoid that vein. Eventually, this vein worked back into the rotation, but now it doesn't feel tough. We discussed ports, but they terrify me. Our venous access has gone well, so why change? My son was also against the port. He is now ten, and doing well with prophylaxis and venous access.

Your friends should talk to a nurse or someone who knows about veins; get some opinions, and try to build up their confidence. Veins sometimes develop scar tissue, but they also heal.

**Anonymous reader**

**A** It's hard to make this decision under such circumstances. We are now transitioning to peripheral sticks after using a port for five years. We rotate veins and stick an arm every other poke. Our son is infused only twice a week, and this has worked well for us. We've had difficult days when we had to do multiple sticks, but we had the port for back-up. Some families have done peripheral sticks with great success. If your friends are committed to not having another port placed, it's worth trying peripheral sticks, despite a doctor's urging to place a second port. It won't hurt to try it themselves for a few weeks. The decision to have a port placed (or not placed) takes total commitment, so if they are reluctant, it's better to wait until they are convinced of their decision.

If they have another port placed, your friends should be reassured that following strict sterile technique will reduce the risk of infection. We've been told that if our child should develop an infection, it's due to a flaw in our procedure. Have your friends determined the cause of the infection? Did they leave the port accessed for any length of time? Have they examined their technique with their doctors? Some families are taught improper technique, so it would be helpful to check their technique with another family, perhaps from another HTC.

*Jill Lathrop*  
WISCONSIN

**A** Our sons are 21 and 19, so when they were babies, ports weren't an option. Starting at age six months, they've each had over a thousand IVs with bleeds, prophylaxis, and blood work—and never damaged a vein. They like to use certain veins, so they have thicker skin at those sites, but no vein problems.

We started doing the shots ourselves as they reached ages four and two. But before that, the nurses did lots of pokes—trying to find veins, or blowing veins—so our sons had lots of “prodding” at a young age, with no vein damage.

We had to take our sons to the ER often, so it was never the same person sticking them, nor someone with much experience finding veins in babies and toddlers. I think that makes a difference in successful venous access.

*Melinda Ward*  
TEXAS

**A** I am 43, with severe hemophilia. My mother started using fresh frozen plasma on me when I was four. We used cryoprecipitate briefly, then started factor in 1970. I have been sticking myself since I was a teenager. I've used the same vein, in the same spot, for over 30 years. I am on prophylaxis; when I was a teenager, I had almost daily injections.

I use the vein on the inside crook of the elbow for several reasons. First: it is less visible. As a child I was conscious of having needle marks. I can stick with either hand. Second: I can push the syringe with the same hand that I am sticking, leaving my other hand free to type on the computer, talk on the phone, brush my teeth, or drink coffee! I never let the process of infusion interfere with my life. The more matter-of-fact you can make it for your child, the more it will seem like a usual part of the day. Third: good vein care and ease of use. When I was a child, my mother made me wrap a piece of tape around the site, all the way around my arm, and leave it for at least 30 minutes—she couldn't trust me to hold it. As an adult, I hold the site tightly for at least ten minutes, by the clock, after I inject. This produces minimal scar tissue around the site, and insures that it won't be sore from repeated sticks.

I have huge veins, but am fairly thin and very active. Venous access in another place is not an issue. I am used to this site, and want to take care of it, because I still don't want needle marks all over my arms.

*John Reed*  
OKLAHOMA

## UPDATE From Marelle

The port was removed (after two infections and a blockage), and my friend has been learning venous access. She practiced on me a few times, and successfully got her son's vein on the first attempt. We're taking it one step at a time, but so far it's all positive.

➔ The information provided in Parent-to-Parent should **not** be construed as medical advice. *It is advice from one parent to another. Please consult your HTC for information on any medically related questions.*

## From Sue Schwarz

CALIFORNIA

"In March, we reached a milestone in the life of our nine-year old son, Carl: he received his thousandth infusion of clotting factor.

Carl was born with severe hemophilia A. He was diagnosed at age eight months, and we started prophylactic treatment when he was two years old. He receives his clotting factor three times a week at home. This has become as routine to Carl as brushing his teeth or feeding the dog.

"After the initial shock, sadness and anger at the diagnosis had passed, my husband and I got busy learning all we could about hemophilia. With knowledge came a sense of control over this inherited condition. When Carl started walking at age one, he faced the world armed with his blue helmet, and the kneepads made by his Grandma. He looked adorable!

"The infusion process became more complicated as Carl began to have more bleeds. He would bleed into his left elbow one to two times a week. We lived in Sequoia National Park, in central California—one hour away from a hospital, and five hours from our treatment center in Oakland, California. Our friend and park ranger Eric Morey, also a park medic, helped us immensely during this time. Carl called him 'Dr. Eric.'

"As time went on, and my husband and I learned the infusion process, life became more normal. Not having to rely on others or the medical community to see us through every bleed was a big step toward Carl's independence, and ours. We traveled a lot with Carl when he was young; we've seen Emergency Rooms in Bullhead City, Arizona, Cleveland, Ohio, Denver, Colorado, and Maui, Hawaii.

"In February, Carl started to self-infuse. Nothing that he accomplishes in his life can make me more proud than the self-assured, matter-of-fact way he pokes a needle into his vein. My husband, Paul, logs all of Carl's infusions on our home computer, so we

were anticipating the thousandth stick. We keep a log noting the factor lot number, number of IUs given, who infused him, the infusion site, and the history. We have a complete record of all infusions.

"When I think of this small boy having been stuck with a needle 1,000 times at only age nine, my heart aches. But when I consider what a difference clotting factor and prophylactic treatments have made in his life, I am so grateful.

"Five years after Carl was born, his brother Eric arrived. Eric is not affected with hemophilia. But now, at age four, he watches with keen interest his brother infusing himself. Last

summer, Eric fell and broke his collarbone. I was driving him to our local ER, and he turned to me and said, 'Mom, just give me some clotting factor.' I had to laugh, but I was intrigued that this young child had an understanding of why his big brother gets clotting factor.

"So as I thought about what to do to mark the grand occasion of the thousandth stick, somehow a cake, a new game, or cash did not fully capture this accomplishment. It seemed bigger than

these things—this incredible journey our family has traveled together. I thought, Carl will handle this infusion as he does all the others: with a kind of ho-hum attitude that only a kid can exude. Although he was not expecting anything special on that day, I felt the need to celebrate this event.

"Perhaps I am applauding the fact that *Paul and I* have survived 1,000 pokes. I remember the really tough ones, when Carl was a toddler and it took two people to hold him while a third person found the vein. I was so glad when that stage was over, and life with hemophilia became almost simple. Now that Carl is self-infusing, a new sense of independence has arrived. So we will continue on with the next 1,000 pokes, or until the promise of gene therapy becomes reality and we can kiss those needles good-bye."



## From Joel Gresham

"I've been diagnosed with severe hemophilia A, but it's never stopped me. The diagnosis came two days after my birth, and the symptoms have followed ever since. I don't mind my situation; it's made me humble, yet powerful with words where others may need force. I've become a leader among my acquaintances, because people with hemophilia must make healthy decisions, and peer pressure is something I have never liked.

"It was hard explaining to my friends why I couldn't compete in football, even though I was quick enough; or understanding why basketball was out due to 'what if...' It really hurt to know that although I was capable of playing during recess, I'd never get the chance to wear a jersey during a game.

"Depressed after hearing doctors tell me about weak joints, I decided to fight back. I began exercising my body to keep my joint areas strong. That year in high school, I ran track.

Although I couldn't always place high, I always finished the race and, more important, I was there—participating.

"Even with this blood disorder, I remained *me*. I became social and gained more friends. Almost at once, after I stopped trying to be 'regular,' my life got better, and at the same time my worries decreased. Hemophilia has made me a courageous person, unafraid to do something different from the norm. At age 16, I thought, face it: *I'm not normal, I'm me*. That's been my outlook ever since.

"Today makes 20 years, five months and three weeks since my birth, and it's been a rough ride. My thoughts stay positive, and I remain strong-willed. I live my life to the fullest, with health a priority. I live a healthy hemophilic lifestyle—not too rough, not too easy!"

## Hemophilia Statistics and *PEN* Readership

**Readers:** Find out who is reading *PEN* in your state. Help us inform and support the hemophilia community by spreading the word about *PEN*.

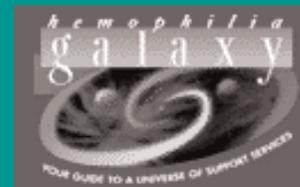
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We have highlighted our “star” states in the chart. In star states, more than 10% of the hemophilia population is reading *PEN*.

| State                | Population* | Estimated # of people with Hemophilia | Individual <i>PEN</i> Readership | Readership as % of Estimated Hemophilia Population |
|----------------------|-------------|---------------------------------------|----------------------------------|--|
| California           | 34,501,130  | 4929                                  | 161                              | 3%   |
| Texas                | 21,325,018  | 3046                                  | 107                              | 4%   |
| New York             | 19,011,378  | 2716                                  | 124                              | 5%   |
| Florida              | 16,396,515  | 2342                                  | 86                               | 4%   |
| Illinois             | 12,482,301  | 1783                                  | 74                               | 4%   |
| Pennsylvania         | 12,287,150  | 1755                                  | 101                              | 6%   |
| Ohio                 | 11,373,541  | 1625                                  | 136                              | 8%   |
| Michigan             | 9,990,817   | 1427                                  | 85                               | 6%   |
| New Jersey           | 8,484,431   | 1212                                  | 43                               | 4%   |
| Georgia              | 8,383,915   | 1198                                  | 33                               | 3%   |
| North Carolina       | 8,186,268   | 1169                                  | 57                               | 5%   |
| Virginia             | 7,187,734   | 1027                                  | 54                               | 5%   |
| Massachusetts        | 6,379,304   | 911                                   | 85                               | 9%   |
| Indiana              | 6,114,745   | 874                                   | 31                               | 4%   |
| Washington           | 5,987,973   | 855                                   | 30                               | 4%   |
| Tennessee            | 5,740,021   | 820                                   | 36                               | 4%   |
| Missouri             | 5,629,707   | 804                                   | 71                               | 9%   |
| Wisconsin            | 5,401,906   | 772                                   | 46                               | 6%   |
| Maryland             | 5,375,156   | 768                                   | 42                               | 5%   |
| Arizona              | 5,307,331   | 758                                   | 43                               | 6%   |
| Minnesota            | 4,972,294   | 710                                   | 60                               | 8%   |
| Louisiana            | 4,465,430   | 638                                   | 61                               | 10%  |
| Alabama              | 4,464,356   | 638                                   | 24                               | 4%   |
| Colorado             | 4,417,714   | 631                                   | 11                               | 2%   |
| Kentucky             | 4,065,556   | 581                                   | 38                               | 7%   |
| South Carolina       | 4,063,011   | 580                                   | 28                               | 5%   |
| Oregon               | 3,472,867   | 496                                   | 24                               | 5%   |
| Oklahoma             | 3,460,097   | 494                                   | 21                               | 4%   |
| Connecticut          | 3,425,074   | 489                                   | 32                               | 7%   |
| Iowa                 | 2,923,179   | 418                                   | 41                               | 10%  |
| Mississippi          | 2,858,029   | 408                                   | 16                               | 4%   |
| Kansas               | 2,694,611   | 385                                   | 50                               | 13%  |
| Arkansas             | 2,692,090   | 385                                   | 31                               | 8%   |
| Utah                 | 2,269,789   | 324                                   | 25                               | 8%   |
| New Mexico           | 1,829,146   | 261                                   | 11                               | 4%   |
| West Virginia        | 1,801,916   | 257                                   | 12                               | 5%   |
| Nebraska             | 1,713,235   | 245                                   | 18                               | 7%   |
| Nevada               | 2,106,074   | 301                                   | 11                               | 4%   |
| Idaho                | 1,321,006   | 189                                   | 6                                | 3%   |
| Maine                | 1,286,670   | 184                                   | 16                               | 9%   |
| New Hampshire        | 1,259,181   | 180                                   | 14                               | 8%   |
| Hawaii               | 1,224,398   | 175                                   | 4                                | 2%   |
| Rhode Island         | 1,058,920   | 151                                   | 6                                | 4%   |
| Montana              | 904,433     | 129                                   | 4                                | 3%   |
| Delaware             | 796,165     | 114                                   | 5                                | 4%   |
| South Dakota         | 756,600     | 108                                   | 5                                | 5%   |
| Alaska               | 634,892     | 91                                    | 2                                | 2%   |
| North Dakota         | 634,448     | 91                                    | 9                                | 10%  |
| Vermont              | 613,090     | 88                                    | 6                                | 7%   |
| District of Columbia | 571,822     | 82                                    | 2                                | 2%   |
| Wyoming              | 494,423     | 71                                    | 3                                | 4%   |

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- Yes!  No

*PEN* maintains a special network of patients and parents to provide us with information for upcoming articles and projects. We want to get your ideas, opinions and experiences periodically through telephone surveys, interviews, or written questionnaires. If you'd like to be on our elite team, check "Yes" in the box to the left, and send or email this form to us.

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