

PEN



inside

- 3 **As I See It:** The Sum of My Parts
- 4 **Inhibitor Insights:** No Inhibitor, No Problem?
- 5 **Richard's Review:** Disclosing Hemophilia
- 6 **YOU:** HTC vs. Home Care Company

Will Your HTC Be There When You Need It Most?

Paul Clement

For more than 40 years, hemophilia treatment centers (HTCs) have set the standard of care for treating bleeding disorders. And people with bleeding disorders have come to expect that HTCs will always be around. But the survival of HTCs isn't assured. The cost of providing care at every HTC is escalating, taking a bigger bite out of the host hospital's general budget.¹ Now, HTCs are under pressure from their hospitals to pull their own weight financially.

This isn't a new trend. From their inception, HTCs have struggled. Most HTCs face chronic underfunding. Most are battling financial pressure, given the constantly changing health insurance industry, changing federal mandates, funding fluctuations in federal and state healthcare programs, and flat

or reduced funding from federal agencies. HTCs also have to deal with shortages of physicians trained in bleeding disorders. HTCs unable to adapt to these changes may lose patients or revenue sources, and be forced to reduce services or even face closure. Will your HTC be there when you need it most?

Birth of the HTC

To understand the challenges HTCs face today, it helps to know some history. HTCs provide *comprehensive care*, an interdisciplinary team approach that cares for the whole patient and all of his or her needs, not just the medical and physical ones. The concept of comprehensive care was just emerging in the 1950s, pioneered for hemophilia treatment by a few hospitals in the UK and US in the late 1950s and early 1960s. In 1962, for example, at Orthopaedic Hospital in Los Angeles, Dr. Shelby Dietrich used seed money from a federal grant to pull together a multispecialty team that provided care for people with hemophilia.² This team included two pediatricians, an internist, a part-time orthopedic surgeon, a physical therapist, a nurse, a social worker, a vocational counselor, and a secretary. The center's reputation grew nationally, luring many Americans with hemophilia for treatments and consultations.

One of these patients was the son of Louis Friedland, then president of National Hemophilia Foundation (NHF). In the early 1970s, Friedland brought his son with hemophilia to Orthopaedic Hospital for treatment. Friedland was so impressed by the center that in 1973 he initiated a two-year NHF campaign to establish a nationwide network of hemophilia comprehensive care centers. As a result, Congress passed Public Law 94-63 on July 29, 1975, authorizing federal funding to establish a national network of comprehensive HTCs. The following year, \$3 million was appropriated to

»» page 7

1. Most HTCs are part of large teaching hospitals, not stand-alone facilities. 2. Carol Kasper, "A Brief History of Hemophilia Center at Orthopaedic Hospital, Los Angeles (2004)," www.carolkasper-md.com/Historical/historyHTC2004.pdf (accessed Dec. 29, 2015).





Here are three components of progressive healthcare in bleeding disorders: (1) safe and abundant factor, (2) HTC's, and (3) the ability to infuse at home. In 2016 we'll be looking at all three, starting this month as we examine the state of HTC's. You may not realize it, but HTC's are facing financial hurdles; comprehensive care and hema-

tologists who specialize in hemophilia are not just a given. HTC's have fought for decades for adequate budgets, expansion, and resources to provide the care we need. Read Paul Clement's excellent article to appreciate what your HTC is, and what challenges it may be undergoing.

Complementing this is my article in *YOU*. How do you choose between a home care company and an HTC to provide your factor? Will your insurance plan even allow you to

choose? Parents speak out about their fight to use a provider of their choice, and what benefits they prefer in a factor provider.

Finally, we kick off 2016 with an article about a topic that I believe may never have been discussed before in a US bleeding disorder publication, written by a member of the LGBT community. As someone with a family member who's gay, I have wondered for a long time: Do people with a bleeding disorder who are gay feel they fit into our community? We're certainly diverse: We have factor VIII, factor IX, and other factor deficiencies. We have inhibitors. We have von Willebrand disease. We are white, black, Latino/a, Asian. We are new immigrants, and citizens. In *As I See It*, Justin Levesque, a man with factor VIII deficiency and inhibitors, points out that the bleeding disorder and LGBT communities have a long, shared history, bound by tragedy. What is our future together?

I wish you all a wonderful 2016 and look forward to providing our educational services to all members of our amazing community. @

inbox

PEN, Nov. 2015

"TO PORT OR NOT?" was a great read! We had our appointment with the HTC and decided we are going to wait on a port for now. My favorite part of the article was that it was emphasized for the parents to decide and not feel pressured either way. I needed to hear that! Thank you for all your help.

Megan Spence
Tennessee



PEN's Insurance Pulse, Sept. 2015

THANK YOU TO WENDY OWENS for her amazing article "Could Cost Controls Prevent Access to Your Factor Brand?" Awesome in content and tone; truly excellent! Thank you for all the work you both do for so very, very many.

Jill Packard
Maine

THOUGH WE HAVE BEEN DEALING with hemophilia issues for many years as, keeping up with current information is so difficult! Thanks for all the information you pass along, both electronic and mailings.

Deb Parlman
Idaho

»» page 18

PARENT EMPOWERMENT NEWSLETTER | FEBRUARY 2016

EDITOR-IN-CHIEF Lauren A. Kelley | SCIENCE EDITOR Paul Clement
CONTRIBUTING WRITER Richard J. Atwood | SENIOR EDITOR Sara P. Evangelos
LAYOUT DESIGNER Tracy Brody | DIRECTOR, PROJECT SHARE Zoraida Rosado

PEN is a newsletter for families and patients affected by bleeding disorders. PEN is published by LA Kelley Communications, Inc., a worldwide provider of groundbreaking educational resources for the bleeding disorder community since 1990.

PEN respects the privacy of all subscribers and registered patients and families with bleeding disorders. Personal information (PI), including but not limited to names, addresses, phone numbers, and email addresses, is kept confidential and secure by the LA Kelley Communications editorial staff in accordance with our privacy policies, which can be viewed in entirety on our website. PEN publishes information with written consent only. Full names are used unless otherwise specified.

PEN is funded by corporate grants or advertisements. Sponsors and advertisers have no rights to production, content, or distribution, and no access to files. The views of our guest writers are their own and do not necessarily reflect the views of LA Kelley Communications, Inc., or its sponsors.

PEN is in no way a substitute for medical care or personal insurance responsibility. Parents or patients who question a particular symptom or treatment should contact a qualified medical specialist. 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.

Articles may be reprinted from PEN only with express written permission from the editor, and with proper citation. PEN and/or its articles may not be published, copied, placed on websites, or in any way distributed without express written permission.



37-39 West Main Street #8
Georgetown MA 01833 USA
978-352-7657 • fax: 978-352-6254
info@kelleycom.com
www.kelleycom.com

Credit for the photos in this issue, unless otherwise noted: Copyright © 2016 LA Kelley Communications, Inc. and its licensors. All rights reserved.

The Sum of My Parts

Justin Levesque

There's a memory I have, from when I was about eight years old, riding in the backseat of my mother's white '91 Pontiac Grand Am. When "Goodbye Yellow Brick Road" came on the radio, I repeated an offensive and overtly homophobic joke about Elton John, the punch line lying far beyond my comprehension. Perhaps I had learned that making the joke was important, an unspoken rule. Perhaps I felt it necessary to mimic the way my father spoke while creating as much distance as possible between the unconscious truth about myself and Elton's music, which blared from the radio.

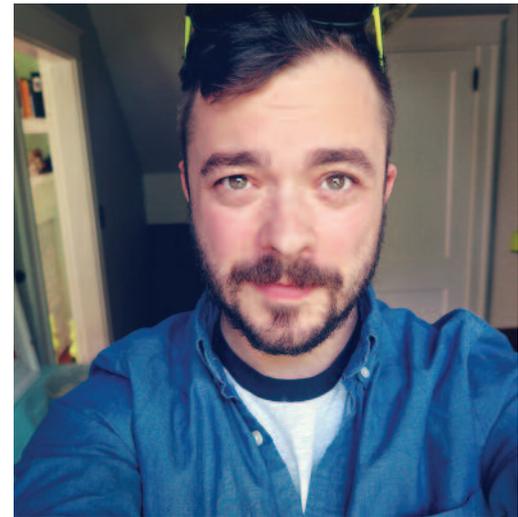
In later years, my mother would have to travel her own rocky path to reconciling the latent reality that her son was gay. But to her credit, in that moment, she steadied the wheel, disapprovingly whipped her head to look at me, and simply said, "Baby, that doesn't matter when the music is so beautiful."

While my personal identity remained mostly dormant and

unprocessed for several more years, there was never any escaping hemophilia, the medical identity that was genetically determined to be seen and heard. Born in 1986, I narrowly missed the direct impact of the HIV epidemic that swept the nation through the tainted blood supply. This was also a period in our community's history that predated prophylaxis: treatment was given only in response to a bleed, and the punctuated relationship between a bleed and pain was intimate.

These were formative years between a time of medical crisis and the promise of prophylaxis. It was a place where resilience became an innate part of who I am, and when I first realized an acute awareness and responsibility for my body. And it is a place where I can still find inspiration from all who came before me, and take comfort in the forged path for all who follow.

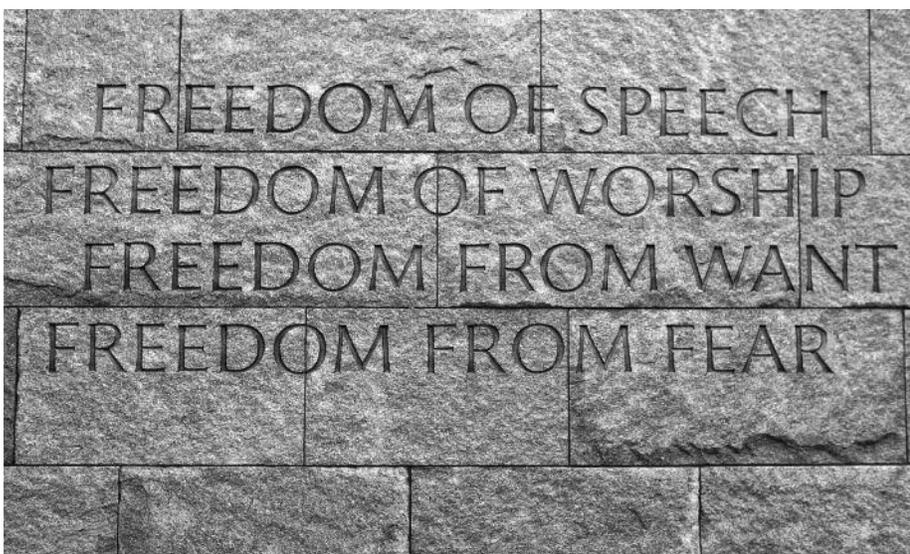
I never officially came out as gay or, as I now self-identify, as queer. Through adolescence, I embraced my medical identity as a person with a bleeding dis-



Justin Levesque

order. I accepted that there was indeed something different about me. My blood doesn't clot, and striving to project that I was "normal" seemed utterly exhausting. Each summer, my classmates would drive to an overpass with a river underneath. I'd go along, but it felt easy to choose not to jump off the bridge, down the daunting 25 feet into the dark, shallow water. What was there to prove? Allowing my medical identity to be a part of who I am was the internal scrimmage in the process of organically becoming the sum of my parts, intrinsically woven. There was no need for an "out of the closet" announcement or to explain all the missed days of school. I refused to live anything but my truth.

Of course, there have been obstacles along this journey of identity. But oddly enough, the most difficult hurdle to navigate so far has been within the hemophilia community itself. The lesbian-gay-bisexual-transgender-queer-plus (LGBTQ+, but more specifically, gay male) community and the hemophilia community have a deep and complicated historical entanglement that originated during their shared health crisis of the 1980s. At the time, both communities were just entering into what seemed like a period of liberation. LGBTQ+ folks were experiencing a newfound visibility in society while the



No Inhibitor, No Problem?

Laurie Kelley

Inhibitor Insights is typically written for families and patients with hemophilia and inhibitors. An inhibitor creates a new level of medical challenges, emotional overload, and lifestyle interference that patients with hemophilia alone don't face. That's why we have a special column just for those with inhibitors.

But not this time. Got hemophilia? Get tested!

That's the message in new guidelines from National Hemophilia Foundation's (NHF) Medical and Scientific Advisory Council (MASAC). If you have hemophilia, you should be tested for inhibitors at least annually. That goes for your child with hemophilia, too.

Inhibitors are a frightening complication of hemophilia. An inhibitor is an antibody, created by the immune system in response to what the body believes is a foreign and potentially harmful substance—factor. The inhibitor inactivates or neutralizes the injected factor so that it can't clot blood.

If factor is a normal part of the body, then why does the body reject it? When there is little to no factor in the body, and some is injected, the body does not recognize it and initiates an immune response against the factor. That's why mainly people with severe hemophilia develop inhibitors. Moderates and milds usually make enough factor for the body to recognize it when injected.

The US Centers for Disease Control (CDC) estimates that within a lifetime, up to one in five people with hemophilia will develop an antibody



(inhibitor) to the infused factor that is used to treat bleeding episodes. But some patients are more at risk than others. What are your chances? And do you *really* need to be tested?

Risk Factors

The incidence of inhibitors in severe hemophilia A patients is about 20% to 30% and about 2% to 3% for people with hemophilia B. Yet certain people with hemophilia have a higher risk of developing inhibitors: people with severe hemophilia A or B; people with certain genetic defects in the gene coding for factor VIII or IX; people with a family history of inhibitors; African Americans; and people receiving infusions while fighting an illness or infection.

The greatest risk of developing an inhibitor is within the first 50 infusions of factor, yet an inhibitor can develop at any age and even after hundreds of infusions.

Although we often worry most about babies developing inhibitors during those first 50 or so infusions, older children, teens, and adults can also be

at risk. I know a 20-year-old with mild hemophilia who got an inhibitor after a sports injury. He hadn't had factor in years, and then bombarded his system with 100% levels for five days to treat the injury. Boom—inhibitor.

Though there are certain risk factors, anyone can get an inhibitor. And so far, no one knows why they develop.

What Testing Shows

A blood test will determine whether you have (or your child has) antibodies to factor. The Bethesda inhibitor assay is a test that measures the titer (strength) of the inhibitor, described as Bethesda units (BU). An inhibitor titer can be 1 BU, 10 BU, or even as strong as 10,000 BU.

Inhibitors are classified in two ways: by titer and by the immune system's response to infused factor. An inhibitor less than or equal to 5 BU is a low-titer inhibitor; an inhibitor greater than 5 BU is a high-titer inhibitor. Low-titer inhibitors are preferable because you may be able to

» page 13

Richard J. Atwood

Disclosing Hemophilia: Mario Giannini



Linda Weaver's Studio

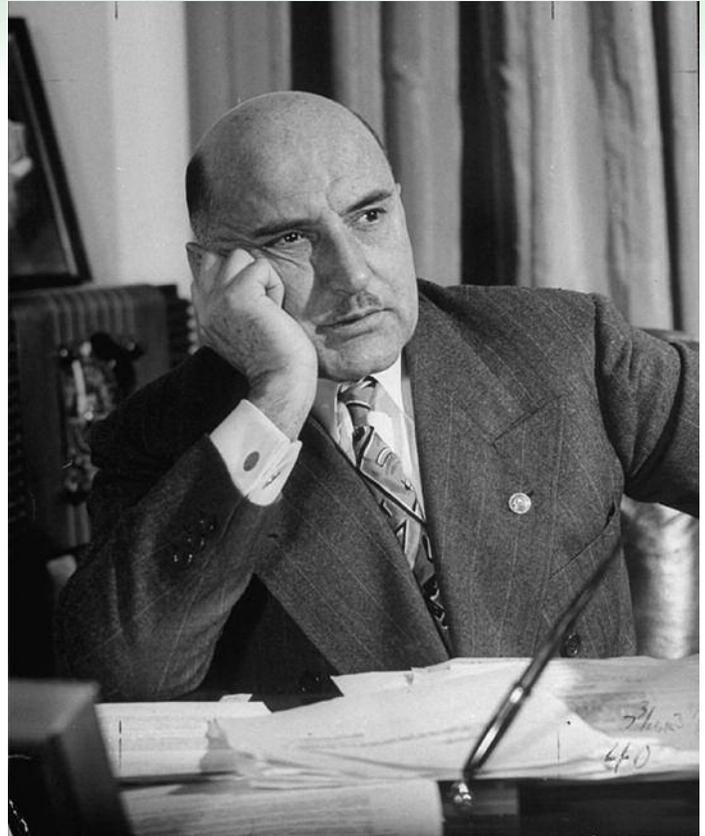
Privacy concerning your personal health information is paramount in the world of healthcare, insurance, and employment. For example, what would you disclose to your employer about your bleeding disorder? In some cases, you might need to disclose. What if you were a prominent public figure, like Bill Gates or Brad Pitt? How might your disclosure affect your business? Consider Lawrence Mario Giannini, who had hemophilia and was president of the world's largest private bank.

Mario was born in San Francisco in 1894. His father, Amadeo Peter Giannini, known as A.P., founded the Bank of Italy in 1904. A.P. began by serving the Italian immigrants in San Francisco and then expanded banking services across California by acquiring other banks. In 1930, his bank's name changed to Bank of America.

The Giannini family moved to San Mateo, California, in 1910. Of the eight children born to A.P. and his wife, Clorinda, only three lived to adulthood. Both surviving sons, Mario and his younger brother Virgil, had hemophilia. At that time, there was no known family history of hemophilia for these second-generation immigrants from Genoa, Italy.

A.P. worried about his two sons with hemophilia, who were not robust like their father. No records are known to exist describing how the young boys coped with their bleeding condition. By age 32, Mario had suffered frequent hemorrhages. In a nonmedical summary, he is described as having bent, stiff hips along with twisted and crippled legs. He walked with a cane. Without access to any medical records, we have to surmise that Mario might have suffered from several conditions, such as limited range of motion in his knee joints, loss of muscle mass, a shortened leg, or shortened Achilles tendons.

But nothing seemed to stop Mario from his profession. He always wanted to be a banker, and he succeeded based on his skills, not because of his father's influence. After graduating *summa cum laude* from the University of California–Berkeley and then from Hastings School of Law in 1920, Mario worked at the Bank of Italy as his father's personal assistant. Advancing over the next 10 years, Mario was elected president of Transamerica Corporation, the holding company for the bank-



Getty Images

Mario Giannini at his desk at home

ing empire. In 1932 Mario was appointed senior vice president, and two years later was elected president of Bank of America, then the nation's largest bank. He remained its president until he died in 1952. Ever expanding, Bank of America became the largest private bank in the world by 1945, and remained so for the next twenty years.

Mario devoted himself to a brutal work schedule. He could work from his apartment on California Street on Nob Hill, and later from his apartment on Green Street on Russian Hill, when he could not make it to the bank's downtown office. This may not have been bank policy then, but it resembles today's effective practice of working remotely from home.

In 1928 Mario hemorrhaged badly in the thigh muscles of both legs. He received repeated transfusions of whole blood,



HTC vs. Home Care Company: How Do You Choose?

Laurie Kelley

Wikipedia says we live in the Information Age.¹ But for families with bleeding disorders, this is the Insurance Age, one of massive cost cutting, Affordable Care Act insurance reform, and increasing cost shifting from payer² to patients. Premiums are rising and out-of-pocket costs are exploding. As payers attempt to contain skyrocketing healthcare costs, they may limit or even eliminate your choice of factor provider. In fact, when I recently asked Facebook friends which factor provider they would choose—an HTC 340B program or home care company—some laughed, “What choice?”

A response like that would have been unthinkable 10 years ago. It seems we always had choice: for many years, choice among different home care providers, and then choice between home care companies and HTC 340B programs.³

How can payers choose for us? Do they know what’s best for people with bleeding disorders? There are pros and cons of using each type of factor provider, based on your personal situation. Home care companies employ a lot of bleeding disorder patients as advocates and sales reps. Some people with hemophilia even own the companies. They give back a lot to the community by sponsoring information days, family programs, and scholarships. HTCs, as Paul Clement’s feature article shows, rely more and more on factor sales as a way to keep their doors open, to fund the medical staff our community requires. We need HTCs. And more and more, HTCs are giving back to the community. Often, both types of factor providers offer similar services.

Will your payer allow choice of provider? That’s the first thing you need to determine. If you have choice, lucky you! If your choice is restricted to one, or maybe two factor providers, find out what each offers. Who are these providers? HTC? Home care? A specialty pharmacy contracted by your insurance company? How much do they know about your bleeding disorder and your individual needs?

Factor Provider Checklist

Before choosing a factor provider, know what you *need* versus what you *want*. Sure, you might want to use a particular home care company because you really like your rep. Your payer doesn’t care about that. You may want to use your HTC because you sympathize with its financial struggle. Payers don’t care about that either. But payers do care a lot these days about saving money.

Here’s a checklist of questions to ask that will help you evaluate factor providers:

About factor:

- Which brands of factor concentrate do you provide?
- How many doses of factor do you supply at one time?
- How do I obtain factor? Do you ship the product to me?
- Do you ship during emergencies?
- Do you provide the assay sizes I need in a single dose?
- How much will I pay per unit of product?
- Do you (if an HTC) offer 340B pricing?

About services:

- Are you an in-network provider for my plan?
- What are your hours of operation?
- Are a pharmacist and nurse available 24/7?
- Do you (if an HTC) offer home care company information?
- Do you supply ancillaries: needles, syringes, bandages?
- Do you provide needle disposal containers?
- Do you contract with local home nursing services?
- Do you provide itemized lists of each shipment?
- Do you provide disease management?



»» page 14

1. https://en.wikipedia.org/wiki/List_of_time_periods (accessed Dec. 30, 2015). 2. A payer is an entity that reimburses the cost of medical services and products. A payer can be a private insurance company, or a public program such as Medicaid and Medicare, as well as certain state programs. 3. Read “Will Your HTC Be There When You Need It Most?” in this issue of PEN for an in-depth discussion of HTC 340B programs.

fund 26 HTCs, greatly expanding access to comprehensive care for patients with bleeding disorders. From the original 26, there are now 146 HTCs in the US.^{3,4}

The HTC Philosophy

What makes the care at HTCs so much better for patients than the care provided by most other hospitals? “Practice makes perfect” is part of the reason: bleeding disorders are rare, and in an entire career most physicians and nurses may see only one or two patients with a bleeding disorder. HTCs see patients with bleeding disorders every day, giving medical staff the opportunity to become skilled at treating related complications.

HTCs are centers of excellence, employing experts in the field of bleeding disorders and using best practices to achieve the best possible outcomes for their patients. These best practices include the comprehensive care approach. Indeed, HTCs have been the model for the delivery of integrated, multidisciplinary, comprehensive care. Why has this approach proved so successful?

Comprehensive care means addressing all the needs of a person with a bleeding disorder: physical, emotional, psychological, educational, financial, and vocational. The composition of HTC treatment teams and the standards of care provided are regularly reviewed and updated by bleeding disorder medical experts on NHF’s Medical and Scientific Advisory Council (MASAC). MASAC publishes a detailed outline of comprehensive care standards, including services that HTCs should provide. MASAC has identified seven “core team” members of an HTC comprehensive care team, and another seven “extended team” members.⁵ The core

team includes a program coordinator, hemophilia nurse coordinator, medical director, physical therapist, psychosocial professional, case manager, and secretary. The extended team includes a coagulation laboratory director, pharmacist, dentist, genetics counselor, orthopedist, ob/gyn, and other specialists such as HIV/infectious disease experts and nutritionists. An HTC team not only provides specialty care, but can act as a resource for family physicians and dentists who treat people with bleeding disorders. Comprehensive care is aimed at preventive care, rather than treating complications after they have occurred.

HTCs have greatly improved quality of life for people with bleeding disorders by helping them to be more independent and productive, with fewer complications. Data from the US Centers for Disease Control and Prevention (CDC) show that people with hemophilia who received some care at an HTC over a three-year period were 40% less likely to die,⁶ and a similar proportion were less likely to be hospitalized for a bleeding complication.⁷ By preventing many of the costly, debilitating complications of bleeding disorders, HTC comprehensive care has saved lives and proved highly cost-effective over the long term.

Financial Challenges

Of all the challenges that threaten HTCs today, the most serious is financial.

The \$3 million allocated by Congress in 1976 was desperately needed by fledgling HTCs. At first, funds were distrib-

3. For a complete list of HTCs, visit the CDC website at https://www2a.cdc.gov/ncbddd/htcweb/Dir_Report/Dir_Search.asp (accessed Jan. 4, 2016). 4. For more on the US HTC network and comprehensive care model, see Judith R. Baker, Sally O. Crudder, Brenda Riske et al., “A Model for a Regional System of Care to Promote the Health and Well-Being of People with Rare Chronic Genetic Disorders,” *American Journal of Public Health* 95.11 (2005): 1910; and Scott D. Grosse et al., “Models of Comprehensive Multidisciplinary Care for Individuals in the United States with Genetic Disorders,” *Pediatrics* 123.1 (2009): 407–12. 5. MASAC Recommendation 132, “Standards and Criteria for the Care of Persons with Congenital Bleeding Disorders,” March 24, 2002, <https://www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC/MASAC-Recommendations/Standards-and-Criteria-for-the-Care-of-Persons-with-Congenital-Bleeding-Disorders> (accessed Dec. 29, 2015). 6. J. Michael Soucie, R. Nuss, B. Evatt et al., “Mortality among Males with Hemophilia: Relations with Source of Medical Care,” *Blood* 96 (2000): 437–42, <http://www.bloodjournal.org/cgi/reprint/96/2/437> (accessed Dec. 29, 2015). 7. J. Michael Soucie, J. Symons, B. Evatt et al., “Home-based Factor Infusion Therapy and Hospitalization for Bleeding Complications among Males with Haemophilia,” *Haemophilia* 7.2 (2001): 198–206.



Now Available!

Want to Learn More?

Call 1-800-554-4440 for information about our Free Trial Program

NUWIQ[®]
Antihemophilic Factor
(Recombinant)

©2016. Octapharma USA Inc. All rights reserved.
Date of preparation: 1/2016. NUW-012-CAD

octapharma[®]
For the safe and optimal use of human proteins

uted directly to the HTC by what is now called the Maternal and Child Health Bureau (MCHB), a division of the US Health Resources and Services Administration (HRSA). In 1983, HRSA developed a network of 12 regional HTC “grantees,” and gave them general grant oversight responsibilities, including identifying and responding to regional needs for developing new HTCs. Your HTC is covered by one of these regional grantees.⁸

In 2012, HRSA reduced the number of grantees from 12 to 8, and through a competitive grant process, awarded the American Thrombosis and Hemostasis Network (ATHN) the first National Hemophilia Program Coordinating Center grant. ATHN works with all stakeholders and respected experts in the bleeding disorder community to facilitate, coordinate, and evaluate the implementation of activities carried out by the Regional Hemophilia Networks. ATHN is also working collaboratively with the regional grantees to develop a best practices model and facilitate uniformity and a standard of care model for HTCs.

The initial federal funding for HTCs was limited—enough to fund only one full-time nurse at a small center, or a few midlevel employees at a large one. These grants were never intended to fully finance the HTCs. Rather, they were considered seed money, intended to demonstrate the effectiveness of the comprehensive care model. It was expected that the cost of running an HTC would eventually be incorporated into the existing healthcare system.

This never happened.

Regrettably, federal funding for HTCs has not kept pace with the changing healthcare landscape. In 2015, federal HRSA funding for HTCs was only \$4.9 million. Adjusting for inflation, this \$4.9 million is about 38% less than the initial \$3 million allocated by Congress in 1976. The 2015 funding averages out to about \$35,000 per HTC—enough to fund less than half the salary of one full-time nurse in most parts of the US. And instead of being distributed to just 26 HTCs, funding is now shared among 146 HTCs. The mushrooming bleeding disorder population has also contributed to the increasing financial pressure on HTCs. Between 1990 and 2010, the number of patients seen at HTCs doubled from about 17,000 to about 35,000—mainly because of new diag-

noses of von Willebrand disease.⁹ In other words, HTCs have much more work to do with much less money.

It helps that for more than 30 years, HTCs have also received a series of small, federal grants from the CDC. These grants are primarily for public health “surveillance,” meaning the collection of medical data on bleeding disorder patients to determine trends. Three major hemophilia surveillance programs have been funded by the CDC: the HIV risk-reduction program, implemented in 1983; the Universal Data Collection (UDC) project focused on monitoring blood-borne infections and joint disease, from 1998 through 2011; and Community Counts, in 2011, focusing on inhibitors and aging in the HTC population and healthcare usage by people with bleeding disorders who are not seen at HTCs. The CDC currently funds Community Counts at about \$4.3 million per year for five years starting in 2015.

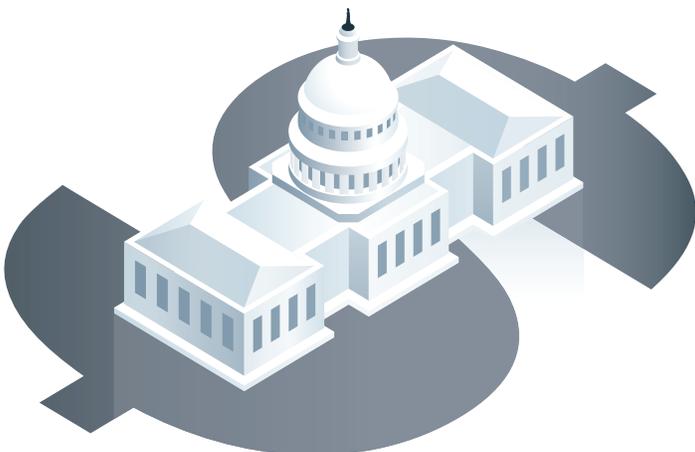
Many HTCs depend on even the modest federal funding as an integral part of their budget. But continued federal funding is not assured. Programs run by HRSA and the CDC are not immune to budget cuts, which could be fatal to some HTCs that are just getting by and depend on federal funding to balance their budgets.

As HTC budgets have eroded, their ability to provide services and education, and provide factor to poor patients, has been challenged. More costs are shifted to the host hospitals. To compound the problem, many of the preventive and educational services provided by HTCs—the hallmark of HTC comprehensive care—are considered non-billable by most payers (organizations such as health insurance companies that pay medical bills). In other words, the costs of these services can’t be recovered. Facing escalating costs, HTCs have come under increasing pressure from their hospitals to pull their own weight financially or face a reduction in services, or even closure.

Selling Factor to Survive: The 340B Program

For most HTCs, the only way to generate additional revenue is to sell factor by participating in a federal drug discount program called the 340B Drug Pricing Program.

Section 340B of the Public Health Service Act (PHS) was enacted in 1992 as part of the Veteran’s Health Care Act and is managed by HRSA, the same agency that manages the HTC hemophilia grants. The intent of the 340B program is to allow certain healthcare providers “to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services.”¹⁰ The program focuses on safety-net healthcare providers—those that care for a disproportionately high number of people with low incomes and the medically vulnerable.



8. For a listing of the 12 regions, and complete HTC directory, see https://www2a.cdc.gov/ncbddd/htcweb/dir_report/dir_search.asp (accessed Jan. 8, 2016). 9. Judith R. Baker, B. Riske, J. H. Drake et al., “US Hemophilia Treatment Center Population Trends 1990–2010: Patient Diagnoses, Demographics, Health Services Utilization,” *Haemophilia* 19.1 (2013): 21–26. 10. “Health Resources and Services Administration: 340B Drug Pricing Program,” <http://www.hrsa.gov/opa/> (accessed Dec. 29, 2015).



Contracting Specialized 340B Services

As of December 2015, 113 HTC—about 77% of all HTCs—participated in the 340B program. If HTCs are hurting for funds, why don't 100% of HTCs participate in the program?

One reason is that most physicians, nurses, and social workers aren't business people. Running a 340B program requires a pharmacy. And it means hiring additional staff who are experts in working with insurance payers; drawing up contracts; managing billing, collections, inventory control, and distribution—not to mention financial reporting and marketing. And an HTC needs startup capital of perhaps several million dollars to purchase enough factor inventory to begin a program. Some HTCs may consider all this too overwhelming. Other HTCs may be limited by facility constraints; or a host hospital may, for a variety of reasons, not give the HTC approval to operate a 340B program. HTCs that want to start a 340B program but lack experience, personnel, or resources can pay fees to contract companies that will administer the 340B program. Or HTCs may contract with outside specialty pharmacies (contract pharmacies) to distribute the factor.

340B Revenues to the Rescue

An HTC 340B program can potentially earn millions of dollars from factor sales. Profit depends on three things: (1) the total number of units of factor sold, (2) the markup price of the factor, and (3) the operating costs of running the 340B program.

How much do HTCs mark up factor above their 340B acquisition price (the price they actually pay for the factor after discounts)? It's generally a negotiating process between the payers and the HTC. For state and federal payers, pricing is mandated at a contractual rate. But to entice payers to contract with and reimburse the HTC's 340B program, HTCs must price their factor competitively. And HTCs must consider many issues when setting the sales price for factor. HTCs just starting a 340B program, and those with few patients enrolled, must spread the cost of running the program over fewer patients. Some HTCs that purchase factor through the 340B program for their Medicaid patients can bill Medicaid only at the acquisition cost (the 340B price) plus a state-mandated dispensing fee—which, in some cases, is not enough to cover the cost of providing the service. But Medicaid reimbursement varies widely among states. In some states, Medicaid reimburses at a much higher rate; and many states will not use the 340B program for Medicaid patients, preferring instead to receive the Medicaid rebate allowed by law. Some HTCs serve many uninsured poor patients, whose medical services aren't reimbursed at all and must be covered by a 340B program income or by the hosting hospital.

The 340B program requires pharmaceutical manufacturers who participate in Medicaid programs to charge discounted prices for covered outpatient drugs purchased by specified government-supported facilities, or *covered entities*. The discounted price is called PHS pricing or 340B pricing. These covered entities can then resell the drugs to their patients at a markup, keeping the revenues to help support services and programs. The potential for revenue is substantial: in fiscal year 2013, 340B entities—about 28,000 in all, 113 of which are HTCs—purchased \$7 billion in outpatient drugs, at a savings of \$3.8 billion over what they would have paid without the 340B program.

The 340B program places some limits on 340B operations. Participating HTCs can sell outpatient drugs at 340B prices only to people who fall under the federal definition of a "patient." They can't sell outpatient drugs purchased at 340B prices to other parties, such as home care companies (an illegal practice called *diversion*). And HTCs can't force their patients to buy factor or other outpatient drugs from them—patient participation in the 340B program is voluntary. Not only that, but HTCs are required to notify patients that they can choose between the HTC's 340B pharmacy and a home care company, disease management company, or specialty pharmacy, if their payer allows it.¹¹

The HRSA hemophilia grant also restricts the way HTCs use 340B program revenues, which must stay in the HTC to benefit its patients and cannot be used by the host hospital to boost its general fund. HTCs use program revenue to support their clinical programs, and to provide essential services such as nursing, physical therapy, social services, training programs for new hematologists, outreach and education, and medical care and factor for poor patients. HTCs also use the revenue to fund HTC-supported summer camps, telephone triage, outreach clinics (especially in rural areas), transportation assistance to get to the HTC, as well as psychosocial and vocational counseling—and these services are non-billable. The operation of the 340B program itself may also consume a lot of program revenue, with expenses that include purchasing factor, personnel costs for filling prescriptions, packaging, delivery to clients, billing, and managing inventory.

11. Specialty pharmacies are distinguished from other pharmacies mainly by the kinds of drugs they offer. Most drugs handled by specialty pharmacies are injectable or infusion therapies; and many are biological drugs or *biologics*—drugs produced by living cells that have been modified by recombinant DNA technology to secrete a specific drug or protein, such as recombinant clotting factor concentrate. The use of specialty pharmacies has skyrocketed over the past 20 years, chiefly because the new biologics require expensive and complex inventory control procedures that are beyond the capabilities of most retail pharmacies.

Most HTC's share control of the disposition of 340B revenues with their hosting hospital, and make joint decisions on how to spend the funds. Some HTC's have formed committees with consumer representatives to advise the HTC and hospital on the best ways to spend the revenue and to ensure grant objectives are met. For most HTC's with 340B programs, revenue from factor sales is the primary source of income. Some HTC's, like the Charleston Area Medical Center (CAMC) Hemophilia Treatment Center in Charleston, West Virginia, are completely self-sustaining: 100% of the CAMC budget is funded through 340B revenues and HRSA and CDC federal grants.

Educating Payers

To stay financially solvent, it sounds like all an HTC has to do is offer a 340B program, right?

For the near future, the answer is a conditional yes. But market forces may eventually limit the ability of HTC's to recruit bleeding disorder patients for their 340B programs or even retain all of their current patients. The patient's health insurance plan dictates whether a 340B or another specialty pharmacy will provide factor. Payers, under constant pressure to contain costs, may "narrow" their network, meaning they restrict the number of healthcare providers in the plan's network. You are covered only by using the providers in your plan. With a narrow network, you may have no access to an HTC, or no access to a specific hematologist. Patients who visit an out-of-network HTC may have to pay substantially more, or their insurance company may not reimburse the cost of the visit at all. When out-of-network, the HTC loses patients, and bleeding disorder patients lose access to quality healthcare.

Skyrocketing drug costs have also impacted the number of patients who can buy factor through HTC 340B programs, as payers turn to pharmacy benefit managers (PBM's) and specialty pharmacies as a way of reducing costs. PBM's are private companies that are hired to manage drug benefits for large groups of people, such as enrollees in an insurance plan or employees of a self-insured company. By negotiating discounts from participating pharmacies and rebates from preferred manufacturers, PBM's can often bring down the price of drugs for health insurance plans. To keep drug prices low, PBM's contract with specialty pharmacies, and sometimes own their own specialty pharmacy (a practice widely seen as a conflict of interest). PBM's offer attractive one-stop shopping for insurance companies, and their cost-saving strategies are believed to save money for both the payer and the consumer.

But this may come at a cost. For people with bleeding disorders, one unpopular cost-cutting strategy used by PBM's is to restrict drug choices, a practice called *formulary management*. A formulary is a list of drugs that will be reimbursed through a health plan. Unfortunately, formulary decisions are often based on price alone: the drugs that make it onto the

formulary are more likely to include those that have the best rebate from the manufacturer. And some PBM's treat all factor products as equal in effectiveness. But there are no generic forms of expensive biologic drugs such as clotting factor, and every patient responds differently to different factor brands. A health plan with a restricted formulary may require you to switch to a single factor brand the PBM believes to be equivalent.

If HTC's with 340B programs can sell factor at lower prices, wouldn't all health plans and PBM's want to have HTC's in-network?

Not always. Including another provider will often cost the health plan *more* money. And the PBM may have concerns about losing access to patient data or losing the ability to track inventory and factor usage. Sometimes, the PBM offers the payer its rock-bottom price if the payer agrees to use the PBM's specialty pharmacy *exclusively*. In this situation, without an exclusive pharmacy contract, the payer would be paying higher prices on *all* drugs for *all* patients in its plan. Compared with this, the savings on factor purchases from a 340B program for a few patients with hemophilia don't amount to much.



COMING SOON
KOVALTRY™
Antihemophilic factor (recombinant)

Register for updates at
www.KOVALTRY.com

Bayer and the Bayer Cross are registered trademarks of Bayer. KOVALTRY is a trademark of Bayer. © 2015 Bayer HealthCare Pharmaceuticals Inc. All rights reserved. Printed in USA 08/15 PP-675-US-0008

Further working against HTC is the fact that payers know there is wide variation in care at HTCs.¹² For example, according to CDC UDC data, at some HTCs no children are on primary prophylaxis, while at other HTCs, 100% of the children are on primary prophylaxis.¹³ Similarly, at some HTCs, 100% of patients are screened for inhibitors, while at other HTCs, no one is screened for inhibitors.¹⁴ Payers may ask, Why should we include HTCs in-network as providing the standard of care, when HTCs themselves may not agree on what the standard of care should be?¹⁵

HTCs can't rest on their laurels as being centers of excellence for hemophilia care. They must educate payers on the benefits of HTC care for people with bleeding disorders. To promote HTC care, HTCs are now working with advocates such as the Hemophilia Alliance, which counts 95 of the 113 HTCs with 340B programs as members; the 340B Coalition; and ATHN. NHF is also acutely aware of coming changes in the healthcare industry and their potential effect on HTCs, and is taking steps to ensure that payers understand the value of the HTC comprehensive care model.¹⁶

Surprisingly, HTCs must also educate their own patients about the benefits of using HTCs! HTCs have been victims of their own success. Why? Gone are the days when people with severe hemophilia spent extended time each year at the HTC, receiving treatment for bleeds and follow-up physical therapy. Today, many teens on prophylaxis have never spent the night in a hospital! And many hemophilia patients go to the HTC only for an annual checkup.¹⁷ Many people with hemophilia today often feel that they don't really need the HTC. Yet research proves otherwise. When a person with hemophilia suffers from a severe bleed, traumatic injury, or other complication, the care he receives at an HTC can reduce the chance of hospitalization—and even death.^{18,19}

Will Your HTC Survive?

What's the future for your HTC in this rapidly changing healthcare industry?

If it operates a 340B program, your HTC will probably be better off financially than those that don't. But revenues from 340B factor sales may slowly decrease, as HTCs lose out to PBMs, specialty pharmacies, and movement of Medicaid patients to a "managed" Medicaid program—which may have a narrow network that limits a patient's access to his or her HTC. With essentially flat federal funding, increasing



federal mandates, and a growing patient population, HTCs that don't offer 340B programs may be forced to start them to remain financially solvent. An HTC that cannot or will not adapt will close.

What can you do to help your HTC? First, when selecting an insurance plan, make sure your HTC and your doctor are in-network. Make sure you're seen at least yearly at the HTC for a comprehensive checkup. Consider buying your factor through your HTC's 340B program, if it meets your specific needs. Finally, get involved: join your local hemophilia foundation, NHF, and Hemophilia Federation of America (HFA). Sign up for email alerts and mailing lists. If a call goes out to mobilize the forces in response to pending legislation that could harm HTCs, do your part by writing letters to your legislator. Participate in your local chapter's legislation day and NHF's Washington Days. Make your voice heard so we continue to enjoy the standard of care that has transformed hemophilia—once a dreaded, painful disorder with a short life expectancy—into a condition that is highly manageable with a normal life expectancy. @

I wish to thank Judith R. Baker, DPH (public health director, Western States Regional Hemophilia Network at the Center for Inherited Blood Disorders, and adjunct assistant professor, UCLA Division of Pediatric Hematology/Oncology) for reviewing this article. I also thank Derek Robertson, MBA, JD, CHC (Apogenics 340B Program Services), Michelle Rice (NHF), and Donna Arden, RN, MS (Charleston Area Medical Center HTC), as well as others, for their input.

Paul Clement is a retired high school science teacher and contributing editor of PEN. He has a BS in biology and MA in science education from California State Polytechnic University. Paul lives in Southern California with his wife Linda and children Erika (30) and Brett (28), who has severe hemophilia A.

12. Mark W. Skinner, J. Michael Soucie, and Kathryn McLaughlin, "The National Haemophilia Program Standards, Evaluation and Oversight Systems in the United States of America," *Blood Transfusion* 12, Suppl. 3 (2014): e542–48. 13. J. Michael Soucie, S. McAlister, A. McClellan et al., "The Universal Data Collection Surveillance System for Rare Bleeding Disorders," *American Journal of Preventive Medicine* 38:4, Suppl. (2010): S475–81. 14. Skinner, Soucie, and McLaughlin, "The National Haemophilia Program Standards." 15. Ibid. 16. To address the issue of variation in the standard of care between HTCs, NHF is sponsoring, in conjunction with MASAC and McMaster University, the production and maintenance of evidence-based clinical practice guidelines on models of care delivery in hemophilia. Basically, the guidelines discuss the types of care and services provided by HTCs that are key to improving patient outcomes. In 2014, NHF also kicked off the Comprehensive Care Sustainability Collaborative (CCSC), an initiative to develop a "framework for pilot programs that could be replicated across the United States between payers and hemophilia treatment centers (HTCs) to facilitate cost effective hemophilia management integrating the HTC comprehensive care model." <https://www.hemophilia.org/Newsroom/Advocacy-Legislative-News/NHF-Announces-the-Comprehensive-Care-Sustainability-Collaborative-CCSC> (accessed Jan. 8, 2016). 17. According to the CDC, approximately 70% of the estimated 20,000 people with hemophilia in the US are seen at an HTC. Baker et al., "US Hemophilia Treatment Center Population Trends 1990–2010." 18. Soucie, Nuss, Evatt et al., "Mortality among Males." 19. Soucie, Symons, Evatt et al., "Home-based Factor Infusion Therapy and Hospitalization."

advent of factor concentrate gave people with hemophilia fewer trips to the emergency room and the “invisibility” of home infusion. That golden age was short-lived, and these two groups of people, essentially strangers, became embroiled in the fight of their lives. Fueled by fear and biased media coverage, the only choice was for both groups to acknowledge the other in what would become a relationship that was publicly antagonistic yet quietly supportive.

Some of the public antagonism cut deeply: people with hemophilia were portrayed as innocent victims of the epidemic. In response, LGBTQ+ activists rejected their presumed guilt, and proclaimed “All People with AIDS Are Innocent.” National Hemophilia Foundation established a “gay blood ban” in 1983. Meanwhile, hemophilia families looked beyond their HTC to seek information and resources from LGBTQ+ HIV/AIDS support groups, and hemophiliacs themselves became activists.

Michael Davidson writes, “Hemophiliacs, by association with a ‘gay-related’ disease, were subject to homophobia on the one hand and what one commentator has called ‘hemophobia’ on the other.”¹ Ryan White, threatened and targeted himself, in many ways encapsulates this sentiment. Specifically, in the mainstream media, his image was in part responsible for desensitizing the stigma around HIV/AIDS, but it also—despite Ryan’s consistent rejection of the criticism of homosexuality—created

distance between hemophilia and the LGBTQ+ community.

When it comes to being gay/queer and having hemophilia, it can sometimes feel like these two key parts of my identity are in conflict. This, in turn, never fails to take me by surprise, given how important each was to the other in my coming-of-age tale. And, as I would learn during community events, that conflict inside me was not unwarranted. In 2014, after moderating a rap session for LGBTQ+ people with bleeding disorders, I was questioned on the validity of holding such a session. Then I was asked, “Why are you trying to bring AIDS back into our community?”

Even with the amazing progress toward equality we’ve seen in 2015, it’s clear there is still a wound to be healed among us. ☺

Justin Levesque holds a BFA in photography from the University of Southern Maine, with a specialization in the critical analysis of images and their impact on social norms and community expectations. He lives in Portland, Maine, and runs his own design studio, Shop Geometry. Justin serves on the board of directors for Hemophilia Alliance of Maine and runs a group of programs called FOLX (folxfolx.org) that celebrate arts and creativity in the bleeding disorder community. For more information on the LGBTQ+ community, please visit <http://www.glaad.org/reference/lgb> or thesafezoneproject.com.

1. Michael Davidson, *Concerto for the Left-hand Disability and the Defamiliar Body* (Ann Arbor: University of Michigan Press, 2008), 41.



novoeight®

Antihemophilic Factor (Recombinant)

Visit Novoeight.com to find your local Novo Nordisk Representative and learn more.

Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536 U.S.A.

Novoeight® is a registered trademark of Novo Nordisk Health Care AG.
© 2015 Novo Nordisk All rights reserved. 0915-00028414-1 September 2015



effectively treat bleeds by infusing larger-than-normal doses of standard factor concentrate.

If the inhibitor titer is less than or equal to 5 BU and remains at that level even after a factor infusion, then you are (or your child is) a *low responder*. If the inhibitor titer rises above 5 BU within a few days after an infusion, then you're a *high responder*. Treating a bleed in the presence of high-responding inhibitors requires special factor concentrates because standard factor concentrates are not effective at stopping a bleed.

Not all inhibitors are troublesome. Low-responding inhibitors that disappear spontaneously after weeks or months are called *transient* inhibitors. Of course the ones that stick around are the most worrisome, but *all* inhibitors should be considered serious, and need expert treatment by your HTC. In people with hemophilia and inhibitors, bleeds can be treated with special factor concentrates known generically as *bypassing agents*. Inhibitors can often be eliminated through a process called *immune tolerance therapy* (ITT) or *immune tolerance induction therapy*.

Be Prepared

Don't wait until you notice that factor isn't working as it should. Don't wait for a bleed to worsen, while you wonder what's happening. Don't assume you need to give just one more infusion, or a higher dose, to stop that bleed. And even if you have mild hemophilia, don't believe that inhibitors could never happen to you—or to your child.

Get tested annually, even when you don't show any signs of an inhibitor. And always get tested before any surgery.

NHF recommends you take these steps:

- Ask your doctor about your risk for an inhibitor, how often you should be tested for inhibitors, and what you can do to help avoid developing one.
- Participate in the Community Counts program and take advantage of the free inhibitor testing provided as part of this CDC project.
- Participate in NHF's My Life, Our Future genotyping program; your genotype (the specific genetic change that causes you to have hemophilia) is a key indicator of your inhibitor risk.
- Participate in research studies because it takes data from lots of patients to identify the major risk factors for inhibitors.

MASAC's guidelines are based on results from the CDC's Hemophilia Inhibitor Research Study (HIRS). HIRS investigators and CDC researchers found that (1) people with hemophilia of all ages are at risk for developing an inhibitor and (2) unless people are regularly tested for an inhibitor, they may not know they have one until it causes a severe bleeding problem.

Don't wait until inhibitors are active and doing their work. Ask yourself: When was the last time—if ever—that I was tested, or my child was tested, for an inhibitor?

Make an appointment today! ☺

Adapted from eNews@hemophilia.org, November 20, 2015. Sign up to receive this free e-publication from NHF at www.hemophilia.org!

Learn more about inhibitors from the world's only book on inhibitors, *Managing Your Child's Inhibitor*, by Lauren A. Kelley and Paul Clement. Available free at www.kelleycom.com.



Sponsor a child with hemophilia

It's rewarding and teaches unforgettable lessons

Facing another morning infusion, 10-year-old Andrew* looks at the picture of his beneficiary, 12-year-old Abil from the Dominican Republic, and sees Abil's swollen knees from repeated untreated bleeds. Each time this reminds Andrew just how fortunate he is to live in a country with factor.

Become part of our world family. A sponsorship is only \$22 a month!

A child is waiting for you at: www.saveonelife.net
Or email: contact@saveonelife.net

* name has been changed

Strong-Arming It: Switched!

Some families have learned the hard way that they lack choice of factor provider: their payer forced them to use a particular provider, which they later learned didn't meet their needs.

Tara Banks confides, "I've been so unhappy with the specialty pharmacy we are made to use, mainly because I don't have nursing available. My husband's employer is locked into using them. I have been trying to get it changed for almost six years."

Joy Stein Fitzgerald was switched by her insurance company from her HTC 340B program to a large mail-order specialty pharmacy. Mail-order companies can seem very impersonal. Joy had a great relationship with her HTC, and loved ordering from it. "I hate this new company," she laments. "My HTC was not able to overturn the insurance company's decision."

Change Is Good

Some families are able to choose, and can find a factor provider that meets their unique needs. Steph Reitberger chose her HTC's 340B program. "We were at first given an information packet on 340B pharmacies but were told it was our choice. The packet strongly suggested using their 340B program. However, we did price compare, and the cost difference was astronomical if we *didn't* use the HTC."

Catelyn* also chose an HTC 340B program. "Our pharmacist is awesome! She is in the same building as our hematologist. If I need a prescription change, it happens immediately with no waiting on phone calls. Plus, a small portion of money made off meds goes directly back to the HTC. They don't offer home nursing, but I received port training and vein training at my HTC."

Julie Riley Willams prefers a home care company. "The home care company we started with when my son was diagnosed was good, and what we liked most about it was the regional care coordinator assigned to us. But as the years passed, the company changed for the worse, and we began searching for a new factor provider. We found our old regional care coordinator at a new company, and we switched to it immediately. Our HTC has been wonderful about all this."

Provider Doesn't Matter: Needs Do!

Hathairat Desnoyers describes her special need: as a military family using TRICARE insurance, they needed flexibility whenever they moved. "Our hematologist in Virginia was the best ever. He was knowledgeable, experienced, and kind, so we asked him to help us choose a factor provider that would fit with our military lifestyle. He suggested we go



with one of the big companies that will be able to service us throughout the country wherever we move. Now we are with a small home care company that is taking care of us like family. I love that they not only provide us factor, but also information and help for our family."

Joy just wanted to keep a provider close by. "Not one located across the country that can't get things to us in an emergency or on time." When her son had surgery, her factor provider at the time, a mail-order provider, took five days to ship supplies. "Some things were out of stock," says Joy. "Then the flight was delayed. I had to call the HTC, and they were able to get emergency supplies to me in four hours." Because her insurance company dictates which provider to use, Joy chose to go out-of-network to get a factor provider that worked best for her. It happened to be a small home care company, located only four hours from her house.

Ethical Matters

One element of choice should matter to all families: ethics. The pressure on factor providers to bring in revenues, and to survive grinding cost cutting by payers, can make even good people do questionable things. You should be aware of what factor providers can and cannot do.

For example, home care companies legally can't offer cash or in-kind payments if you help bring new patients to them. They can't buy you gifts, send you to bleeding disorder meetings, or hire you so they can get sales dollars from your factor usage, if they are your provider. "It seems to me that more than a few home infusion companies need to clean house and to appropriately train their sales team," suggests Ogden Forbes, who has five children with hemophilia.

Similarly, 340B programs must adhere to specific guidelines. Above all, if they recommend that you use their program, they must also offer choice of factor provider, including competitors such as home care companies. And they must never withhold services if you decide to use a home care company instead of the 340B program. Most HTC's adhere to these rules, but some slip.

Mary* notes, "Our HTC implied that they could lose funding if we didn't use the 340B program. They said that they depend on their patients getting factor from them. And if we switched [to a home care company], they wouldn't be able to keep enough of my son's product on hand to treat an emergency. We chose to use a home care company. Ever since we switched, I feel as if the HTC treats us differently."

But Kari Franklin reports that although her HTC staff told her they rely on factor sales, "They never pushed the issue. They just told us what our options were." And she ended up using the HTC.

Advocating for Progress

One thing the bleeding disorder community does well is advocating. And that's something you may need to do to get what

you need. Your payer may take away choice, telling you no, you can't use a preferred factor provider. But will that stop you?

This is why it's so important to know what you need (not just what you want). Document your communications and interactions with your factor provider. Is your provider ethical? Does it meet your unique needs? Availability, service, convenience, correct assay, and price are all crucial to your decision. But remember, if you want to change providers, you may need to fight for it.

Emily Boyer jokes, "Choosing? How do you do *that*?" Then seriously, "Our private insurance allows one factor provider, and we have thought of sending a letter to our insurance asking for a choice, but other patients have been unsuccessful."

Though it may be difficult to get a change in provider once your payer has contracted with one or two, small victories are possible. A few families have been able to switch providers if there's a good reason, and through advocating. Our community has shown that we can make progress in healthcare, for ourselves personally, and for the greater good. Let's turn the Insurance Age into the Progressive Age. ☺

* Some names have been changed for anonymity.

Richard's Review... from page 5

which stabilized the bleeding but left the joints in both legs swollen and painful. A month later, Mario was admitted to St. Francis Hospital to undergo orthopedic treatment. His specific treatment is not recorded, but he probably underwent corrections for fixed flexion contracture of the knees (inability to straighten), and possibly tendon and ligament lengthening using casts, splints, and slings.

Though immobilized in bed for several months, Mario continued to conduct bank business from his hospital room. During his recovery, as he started to walk again with a cane, Mario fell in love with a nurse named Mercedes Anne Collins. Mario married Mercedes in 1929, and they had two daughters and lived in Atherton, California.

Mario received recognition for his accomplishments. He earned Italy's highest civilian medal. President Harry Truman offered Mario an appointment as secretary of defense, which he refused, and an appointment to the Foreign Trade Financing Commission, which he accepted. Mario took his father's position as a University of California regent, though he later resigned during a controversy over academic freedom.

Mario's health challenges continued, especially affected by legal battles as he fought accusations by the Federal Reserve Board that Transamerica was a banking monopoly. This stressful legal dispute included frequent cross-country trips to testify in Washington, DC. The bank, however, did not publicize Mario's medical condition. Mario anticipated retiring at age 60 in 1954.

While in Palm Springs in 1952 with a family friend, Mario had another medical crisis. On March 10 he flew back to San Francisco to be admitted at Franklin Hospital. His physician reported that the hospital admission was for treatment of arthritis. (Another report listed the reason for admission as an operation for hemorrhoids, which may have been confused with hemorrhage or hemophilic arthropathy.) For this admission, the press reported Mario's medical condition, but the information wasn't always accurate. Mario never left the hospital. He contracted influenza that developed into viral pneumonia. While in critical care, Mario died of a heart attack on August 19, 1952, at age 57.

Not everyone grows up to head the world's largest bank. Mario Giannini lived in an era with less scrutiny of personal health issues than is possible today, with our 24/7 invasive news cycle. Mario's hemophilia was known, yet it was not publicized by him, by his family, or by the bank, so details about his medical condition are sparse. For a current comparison, recall how the eagerly awaited press release reports of Steve Jobs's cancer condition affected speculation about buying and selling Apple stock. If you're a public figure today, the fact that everyone knows your health status can affect your personal life, your privacy, and your career. For most of us, what personal health information we reveal is our decision. Prepare ahead of time what you might disclose about your bleeding disorder, depending on your type of employment and where you work. ☺

headlines

nonprofit

Direct Aid to Poor with Hemophilia



SAVE ONE LIFE

Save One Life has a variety of programs that offer direct assistance to the world's poor with bleeding disorders. Operating in 11 countries, since its inception Save One Life has provided 1,500 individual annual sponsorships, 15 microgrants, 130 college scholarships, and 18 summer camp grants.

Why this matters: This is the first and only program to offer direct aid to the poor with bleeding disorders in developing countries.

For info: www.saveonelife.net

Lucky Number

Retreat
June 17-19
Denver, Colorado



For families living with factor VII deficiency, the retreat offers medical and treatment updates, psychosocial and stress management sessions, and childcare. Supported by an educational grant from Novo Nordisk. **Why this matters:** This is the only psychosocial program in the US for factor VII families.

For info: www.comphealthed.com



HFA Annual Symposium

March 31-April 2
Las Vegas, Nevada

Hemophilia Federation of America's annual meeting is a community-centered educational event that draws hundreds of bleeding disorder community members to share information, learn new advancements, and build a network of support.

Why this matters: This is a friendly, grassroots way of connecting and learning in person about advocacy, bleeding disorders, and research.

For info: www.hemophiliafed.org

Inhibitor Family Camp!

Painted Turtle Camp
April 15-18
Lake Hughes, California



For families with a child age 6-18 who has an active inhibitor or had one in the past year. Separate, simultaneous programs for children with inhibitors, siblings, and teens. Supported by an educational grant from Novo Nordisk.

Why this matters: Over 52% of parents say this is the only hemophilia camp their child attends.

For info: www.comphealthed.com

payer



Hemophilia's Lion's Share

In 2014, the majority of Washington State Medicaid drug spending paid a small number of claims (633) for 106 clients requiring hemophilia medications. Washington State spent more than \$20 million for antihemophilic factor, according to a report by the US Senate Finance Committee. **Why this matters:** State Medicaid budgets are stretched to the max, causing states to implement cost-cutting methods that could eventually affect hemophilia patients' choice of treatment and product.

For info: stateofreform.com

Guilty!

An Iowa appeals court ruled that the state's largest health insurer, Wellmark Blue Cross and Blue Shield, improperly refused to pay claims for drugs to treat hemophilia patients. Iowa City pharmacist Michael Stein, a provider in Wellmark's network, sued Wellmark after the company refused to reimburse him for \$7 million in claims for factor sales. **Why this matters:** Cost cutting by payers can interrupt the choice, delivery, and reimbursement of factor.

For info: <http://www.kcrg.com/content/news/Court-Wellmark-Must-Pay-Claims-for-Hemophilia-Patients-363466361.html>



Iran Helps Refugees



In an unprecedented move, the government of the Islamic Republic of Iran is now covering the healthcare costs of nearly 1 million refugee immigrants, some with hemophilia. The Universal Public Health Insurance (UPHI), also known as Salamat Health Insurance, is based on an agreement between the UN High Commission for Refugees (UNHCR), the Bureau for Aliens and Foreign Immigrants Affairs of the Ministry of Interior, the Ministry of Health, and the Iran Health Insurance Organization. **Why this matters:** UNHCR hopes that other countries will adopt Iran's approach to refugees.

For info: www.unhcr.org



soundbites

- Biogen is collaborating with Arsia Therapeutics to develop **subcutaneous injections** to treat hemophilia B. Biogen will bring to the partnership its expertise in researching formulations; Arsia will contribute its patented formulation technology.
- Market forecasts by the research and consulting firm **GlobalData** predict that the hemophilia treatment market value will grow slowly, from \$5.4 billion in 2014 to \$6.3 billion by 2024.
- But the company Research and Markets forecasts the **global recombinant factor market** to reach around \$9.9 billion in 2020, growing 7.6% annually during the forecast period of 2015 to 2020.
- At the Federation of American Societies for Experimental Biology's congressional briefing, Dr. Timothy Nichols, director of the Francis Owen Blood Research Laboratory at UNC-Chapel Hill, reported that **dogs treated with gene therapy** have continued to produce 1% to 3% factor IX for more than seven years, enough to substantially reduce bleeding events.
- Of **Nigeria's** population of approximately 170 million, more than 16,700 are estimated to have hemophilia but are not yet identified.
- The US supplies 61% of **plasma collected globally** for fractionation, according to a presentation this month by the Marketing Research Bureau at the 4th Annual Bioplasma World Asia 2015 conference in Shanghai.
- Octapharma's Nuwiq® (NU-veek) is the first and only **fourth-generation recombinant factor VIII** produced in a human cell line without chemical modification or protein fusion.

manufacturer

World's First Recombinant VWD Product

Baxalta's Vonvendi, the first recombinant factor product for adults with von Willebrand disease, is now available.

Why this matters: Though all factor products on the market today are considered safe, Vonvendi offers adults with VWD an extra layer of safety because it's derived from human genes in a lab, not from human blood.

For info: baxaltahematology.com

patient resources

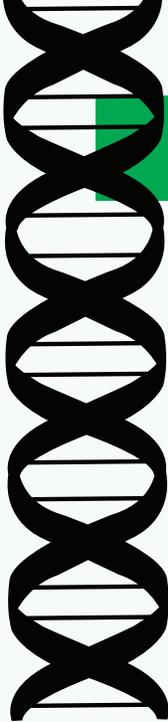
NAVA No More

Baxalta has discontinued the NAVA program as of December 31, and is developing a new program that will offer assistance and resources. For now, Baxalta is still offering the CARE program and options for copay assistance and insurance support; Education Advantage, Baxalta's scholarship program; Hello Talk educational program; and Beat Bleeds.

Why this matters: Manufacturers are constantly seeking ways to improve programs to meet hemophilia's evolving needs.

For info: baxaltahematology.com





A First in Gene Therapy

BioMarin has enrolled the first patient in a phase I/II clinical trial of BMN 270, an investigational gene therapy for patients with hemophilia A. BMN 270 is an adeno-associated virus (AAV)-factor VIII vector. BioMarin licensed this hemophilia gene therapy program from University College London and St. Jude Children's Research Hospital in February 2013. The study will evaluate the safety and efficacy of BMN 270 in up to 12 patients with severe hemophilia A. **Why this matters:** BMN 270 is the first company-sponsored gene therapy trial to enroll hemophilia A patients.

For info: www.bmrn.com



Honors for Research

John S. (Pete) Lollar III, MD, has been named a fellow of the National Academy of Inventors (NAI). Lollar is director of hemostasis research in the Aflac Cancer and Blood Disorders Center at Children's Healthcare of Atlanta and Emory University. He is also professor of pediatrics at Emory School of Medicine, and a holder or co-holder of 10 US patents. He has directed a research team for more than 25 years with the goal of improving treatment for hemophilia. Lollar helped develop a porcine (pig) recombinant factor VIII. The result was the drug Obizur, a treatment for acquired hemophilia A, a condition in which the immune system mistakenly attacks factor VIII. Baxalta brought Obizur to the market in October 2014. **Why this matters:** Praise and awards are well deserved by researchers because research for new drugs often starts at the university level.



For info: www.choa.org

SIPPET Good?



An abstract of the interim data analysis from the Survey on Inhibitors in Plasma Product-Exposed Toddlers (SIPPET) was presented at the annual meeting of the American Society of Hemophilia in December. Initial findings show that the use of a single recombinant factor VIII (rFVIII) product to treat previously untreated patients (PUPs) with severe hemophilia A was associated with a 1.87-fold higher incidence of inhibitors compared to use of a single plasma-derived factor VIII. This study aims to test the hypothesis that plasma-derived factor VIII products with von Willebrand factor are less immunogenic (less likely to cause inhibitors) than rFVIII products. The study is not yet complete and has not been published in a peer-reviewed medical journal. **Why this matters:** Though many questions are unanswered, if valid, the study results could affect choice of product for managing PUPs.

For info: www.sippet.org

Inbox... from p. 2

THANK YOU SO MUCH for the fantastic book *Raising a Child with Hemophilia* and newsletters! I found myself relaxing after reading the first few pages—seriously. I may be an experienced adoptive mom, but I have no idea what I'm doing with hemophilia (being a nurse and being Kai's mother are two different things). I feel like I can do this! I've said it to my social worker and friends, but now I'm believing it.

My husband has your book on his agenda for next week when he has a few moments to himself. Thank you for this wonderful resource. I so appreciate your taking the time to reach out and help us.

Tamara Parker
New York

THANK YOU SO MUCH for your quick turnaround and assistance. The books, especially *Raising a Child with Hemophilia*, couldn't have come at a better time. I'm hooked on it. As a first-time mother who received the unexpected diagnosis of hemophilia B days after my son was born, the first few chapters have been a godsend as I struggle to accept his diagnosis and wait for more answers. I truly appreciate these resources. Thanks for your kindness and for being a wealth of knowledge and support.

Kristina Klein
Illinois

inbox

YOU SAVED ME FROM GOING OFF THE DEEP END so many times. Every time I felt scared or didn't understand what I was going through, I would open one of your books and then, behold! I felt strong and not alone. Thank you, and I love, love your work.

Sarah Guierrez
Ohio



Project SHARE

THANKS SO MUCH for the 36 vials of factor VIII you sent. My son is now discharged from the hospital and continues infusion at home. The day he was discharged, his younger brother was also in the ER awaiting admission. Because of the availability of the factor, we stayed only overnight in the hospital. Thank you for saving my sons' lives.

Anna Karenina Villavert Marzona
The Philippines

YOU ARE ALWAYS IN MY HEART for your kind support to Nepal Hemophilia Society and helping people with hemophilia in Nepal. You always are with us in every up-and-down condition in our lives, and you best know how we are suffering in Nepal.

Thank you for providing the money for my brother's new artificial leg. Indeed, your small help will be a huge thing for my brother and for us. It will help my brother to be comfortable in his daily life.

Thank you from the bottom of my heart for your kind support and your blessed work. God bless you.

Jagat Monsoon
Nepal



I AM THANKFUL for the help you have given me. It made me feel better with my chronic right knee. Thank you for helping hemophilia patients like me who do not have access to factor VIII.

Abraham D. Charvet
The Philippines

YOUR PROGRAM'S DONATIONS are saving lives of unfortunate patients who need the medicines. Thanks for saving lives!

Dr. Nandamuru
India

I RECEIVED THE FACTOR. We really appreciate it. Thank you very much!

Lordana Reid
Jamaica

OUR DEEPEST THANKS TO PEN'S CORPORATE SPONSORS

Baxalta

888-BAX-8379
www.baxaltahematology.com



800-727-6500
www.novonordisk-us.com/biopharm

Parenting Moment

If you can't explain it to a six-year-old, you don't understand it yourself. — *Albert Einstein*



37-39 West Main Street #8
Georgetown, MA 01833 USA
www.kelleycom.com

Visit Your HTC Annually!

AT CSL BEHRING, WE'RE WORKING TO UNVEIL
INNOVATIVE SOLUTIONS IN **HEMOPHILIA A & B**

A century of experience... a future of discovery

Continuing the legacy of Emil von Behring,
recipient of the first Nobel Prize in medicine,

CSL Behring specializes in developing
biotherapeutics for serious and rare conditions,
including hemophilia A and B.

