PEN

inside

DONOR BEWARE

How Facebook Is Undermining International Hemophilia Development Efforts

Laurie Kelley

Donated factor is infused at hemophilia camp, India

The emails and Facebook notifications started arriving to my inbox in December, just before Christmas: from Maine, Virginia, Wisconsin, Texas. All referenced a young man with hemophilia in India who had asked Facebook friends to donate factor to him. No name was mentioned, but I was confident I knew who it was. Call him "Atharv."

Everyone wanted to donate factor to Atharv, but they had questions: "How do we safely donate?" "How can I get a constant supply to him all the time?" "If we give factor to Project SHARE, can you ship it over to him?"[1]

None of them knew that Atharv had also reached out to many others on Facebook; they all thought they were the only ones being asked. And none of them had ever met Atharv in person. Only on Facebook were they "friends." And though everyone asked how to get factor to him, they never asked if they should.

It seems morally right to share factor with a person with hemophilia in need, in a country where factor availability is extremely limited. But what are the legal and medical risks involved? And what impact does donating factor to one patient have on foreign hemophilia organizations that struggle to get donated factor for the thousands of patients who may have more immediate needs?

Performing a charitable deed doesn't always equal doing the *right thing*, or doing it effectively. Unfortunately, as Oscar Wilde wrote, "Charity creates a multitude of sins."

1. Project SHARE (Supplying Hemophilia Aid and Relief) is a humanitarian program of LA Kelley Communications, founded in 2002. SHARE collects unused and unwanted factor from individuals, HTCs, camps, specialty pharmacies, and manufacturers to ship to patients with bleeding disorders in developing countries. SHARE also ships to clinics, HTCs, and hemophilia organizations in developing countries, and helps to train patients and families to become better advocates.

» *page 6*

Laurie Kelley with Bilal, in Pakistan

welcome

Jimmy Carter was the first president I voted for, and I still admire him tremendously. Rather than retire, Carter has worked tirelessly on behalf of the poor, in America and overseas. His words sum up what I feel about the work I do internationally for impoverished families with hemophilia: "My faith demands that I do whatever I can, wherever I am, whenever I can, for as long as I can with whatever I have to try to make a difference."

I began personally donating factor in 1996 to developing countries, where there is little or no factor. If you've been following my humanitarian work, you'll have seen photos of the faces, the grotesquely swollen joints–and the poverty, the pain. Because of my hemophilia publications, I had an extensive database of US families with hemophilia, and contacts at drug companies and home care companies. Collecting factor was surprisingly not too hard back then. And I was good-hearted and naïve. As Carter said, I simply did whatever I could. What could be complicated about getting lifesaving drugs to someone in desperate need?

Plenty, as it turned out.

Since 1996, I've learned that offering help overseas can mean unnecessary personal risks and expense, and may even end up hurting the people in need. I've realized that there's a smart way and a naïve way to go about donating factor.

I founded Project SHARE (Supplying Hemophilia Aid and Relief) in 2002. I wanted to formalize how I was collecting and shipping factor, and our shipments grew so large and so quickly that we were even inspected by the US FDA. We now have factor donations down to a science, with many policies and follow-up protocols to ensure the medicine is correctly shipped and arrives safely. SHARE has a huge database of individual patients overseas, and we understand the clinics, physicians, and customs requirements in each country we serve.

Email and social networking have helped us reach more overseas patients, light-years faster than the snail-mail days of 1996 (when Facebook founder's Mark Zuckerberg was only 12!). Internet cafes are now available in cities and rural areas. But I have noticed an alarming trend in the past year. More and more patients overseas are reaching out online, to US citizens with hemophilia and to hemophilia organizations who are often as naïve and compassionate as I was in 1996.

Our feature article explores what you need to know about donating factor when a plea from an overseas patient arrives in your inbox or on your Facebook wall. Share this information with your colleagues and Facebook friends. Learn from my 18 years of donating factor to all corners of the world.

Fortunately, despite the legal and logistical landmines outlined in our feature, there is always a way to donate factor. In the words of Helen Keller, "Although the world is full of suffering, it is also full of the overcoming of it."

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Jeffrey Moualim

The Living Memorial

Proposed memorial to honor those who died of hemophilia and HIV

Committee of Ten Thousand (COTT) has reached out to the bleeding disorder community for over 23 years, participating in collaborative efforts to benefit those with hemophilia. In recent years, COTT has focused its efforts to build a "living memorial" to those we have lost far too early to HIV/AIDS and hepatitis C, and for the families and friends who remember them today.

The Living Memorial project is not an attempt to rekindle the fires of controversy this tragedy symbolizes, but its mission is rather to celebrate the lives of the men, women, and children we miss every day–people who, through their sacrifice, have made factor VIII and factor IX safer for all of us.

Clotting factor VIII and IX concentrates were developed in the late 1960s and became available for home use in the early 1970s. For people with hemophilia, like me, who were born in the 1950s when only fresh frozen plasma was available to stop bleeding, factor concentrate was a miracle drug: first, to halt a bleeding episode, and ultimately, through prophylaxis, to prevent bleeds from occurring. Immediate infusion of factor minimized joint damage due to hemorrhaging, and led to increased mobility and less pain, so that people with hemophilia could lead a more normal life. Factor concentrates allowed many of us to treat at home instead of visiting emergency rooms every time we had a bleed. And factor opened a world of new possibilities to attend college, travel, and–most of all–become more independent.

Jeffrey Moualim and his wife Sue

J. Moualim

Starting in the late 1970s through the mid-1980s, about half of our community became infected with–and many later succumbed to–HIV/AIDS and hepatitis C. Those who survived began a life-and-death struggle. By 1990, with the inception of COTT, a different kind of battle began: a battle for the truth, seeking to expose how factor manufacturers had allowed contaminated plasma to be used in manufacturing factor VIII and IX. The importance of this fight cannot be overstated. To this day, COTT labors to make sure an accurate history remains, always with the mindset that safety is paramount so that every generation can enjoy the benefits of factor without fear of blood-borne viruses.

The original founders of the COTT board were all infected with HIV, and most were also infected with hepatitis C. Most of them knew that inevitably they would succumb to AIDS or liver failure from hepatitis C. Nevertheless, they made great efforts to ensure that factor products became "cleaner" for future generations. Though their lives were shortened, their legacy was lengthened, as they became pioneers in blood safety. This legacy included working with government regulatory agencies such as FDA to establish more stringent regulations that protect the blood-product consumer today. As a community, we owe a debt of gratitude to all of these people, living or deceased, for their tremendous commitment and selfless efforts on our behalf to ensure safer factor products.

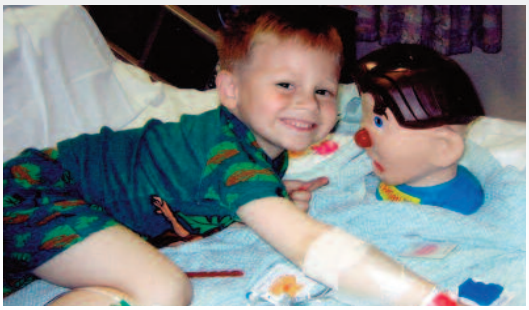
We must not forget the lessons of the past. COTT is working toward building the Living Memorial to honor those who died, and to give their families and friends a place to visit and reflect on our fallen heroes. The Living Memorial will have greater significance if the entire hemophilia community embraces the idea that all of us–past, present, and future–are in this struggle together, living with hemophilia and remaining vigilant that blood products are safe. Bridges of trust and respect must be built between the generations, because a fragmented community increases the chance of an inadequate response to any potential new crisis.

Although an official site has yet to be confirmed at the time of this writing, we do know that the Living Memorial will be located in San Francisco. Currently, COTT has raised over $10,000 (approximately 5% of what is needed to build the memorial) and we have received a donation in kind from the architectural firm RRM Design Group in San Luis Obispo, California. Under the direction of Eddie Herrera and his team at RRM, the initial renderings of the Living Memorial have been completed at no cost.

If you're interested in contributing to this valuable and significant project, please visit the COTT website at www.cott1.org to donate and to see the artists' renderings of the planned Living Memorial.

We look forward to the day when a cure is found for hemophilia, but until that time, let's honor the men and women who gave their lives so that we may have fulfilling lives today and in the future.

Jeffrey Moualim is CFO and fund development coordinator of COTT. He was cured of hemophilia through a liver transplant, but continues to help the community. He can be reached at jmoualim@aol.com.



Beating the Odds: Factor IX and Inhibitors

An Interview with Deena Lipinski

PART 1

Tyler

Tyler Lipinski has had more than his share of medical emergencies and procedures, even for a kid with hemophilia. Tyler is 14 years old, and has factor IX deficiency and an inhibitor. PEN interviewed Tyler's mother, Deena, to share his story with our readers.

Q: When was Tyler first diagnosed with hemophilia B?

When he was two days old. We had no family history, so it was a big surprise. I didn't even know he had hemophilia B until I googled it because the hospital called it Christmas disease. I guess it sounds better when you put it that way!

Before Tyler was discharged, I was told to schedule a hematology appointment and advised not to let him get cut or scratched. Unfortunately, the hematologist was going on vacation, so my appointment wouldn't be for over two weeks. I was terrified. No one gave me even a sheet of paper on Christmas disease! No cuts or scrapes? In my eyes, everything posed a risk. I made my family remove all their jewelry before holding Tyler. I cut out all those tags in his onesies. I didn't know what to expect or do.

It wasn't until I had my first hematology appointment that I discovered Tyler wasn't going to bleed out with a paper cut. The household ban on paper was immediately lifted! Looking back now, I smile at some of the things I did, particularly cutting the tags, a favorite story told at my local hemophilia association. My initial baby-proofing tactics were so over-the-top, but everyone deals with the initial diagnosis in their own way.

Q: What happened when Tyler had his first bleed?

For the first six months, I lived in fear of that first bleed. How would I know? Is he crying because he's cranky, or is he bleeding internally? I read as much as I could, finding some comfort in the fact that Tyler had factor IX deficiency, focusing on the lower probability of complications. But finally it happened, and the honeymoon was over.

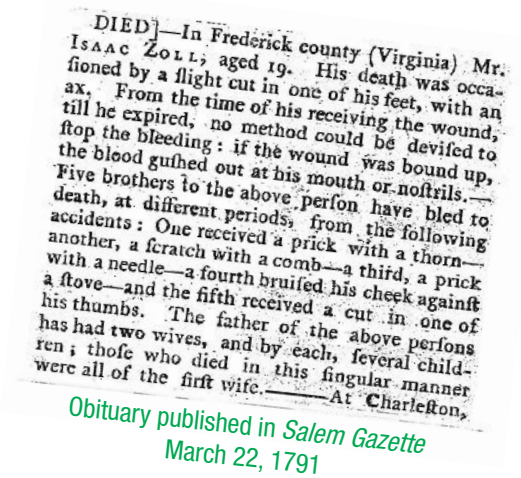
Tyler had his first knee bleed when he was six months old. In 1999, the

» page 14

richard's review

Richard J. Atwood

Going Viral about Hemophilia—in 1791!



DIED]—In Frederick county (Virginia) Mr. ISAAC ZOLL, aged 19. His death was occafioned by a flight cut in one of his feet, with an ax. From the time of his receiving the wound, till he expired, no method could be devifed to ſtop the bleeding: if the wound was bound up, the blood guſhed out at his mouth or noſtrils.—Five brothers to the above perſon have bled to death, at different periods, from the following accidents: One received a prick with a thorn—another, a ſcratch with a comb—a third, a prick with a needle—a fourth bruiſed his cheek againſt a ſtove—and the fifth received a cut in one of his thumbs. The father of the above perſons has had two wives, and by each, ſeveral children; thoſe who died in this ſingular manner were all of the firſt wife.——At Charleſton,

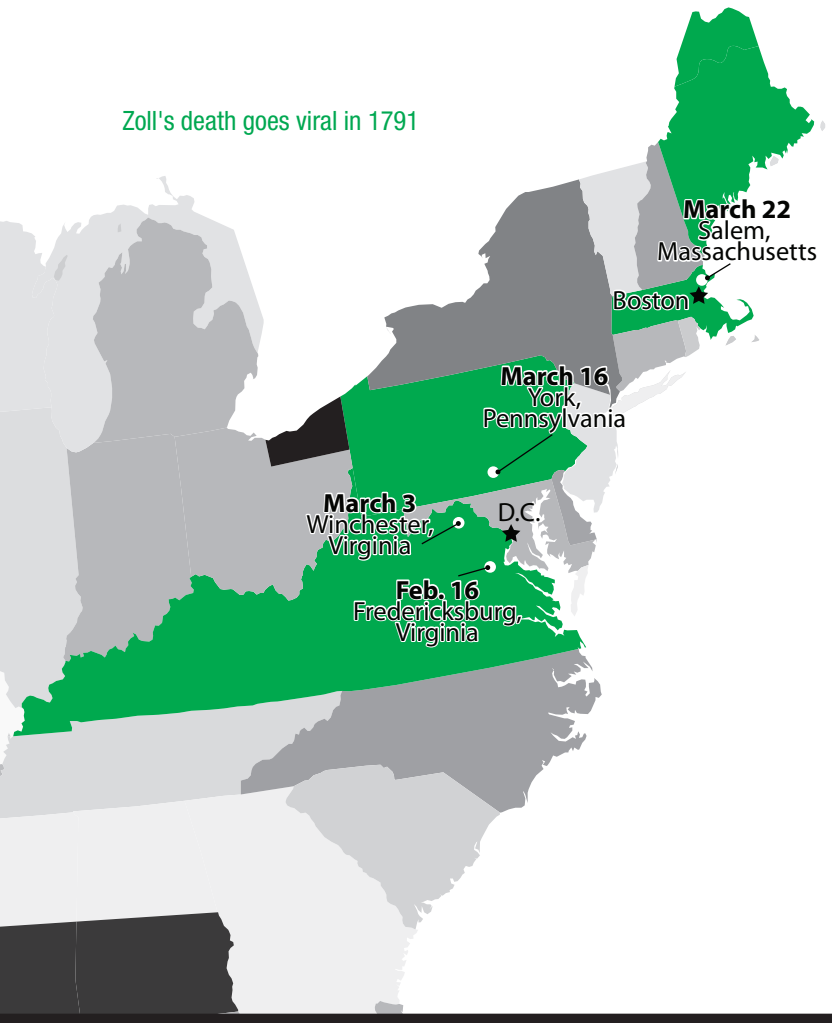
Obituary published in *Salem Gazette* March 22, 1791

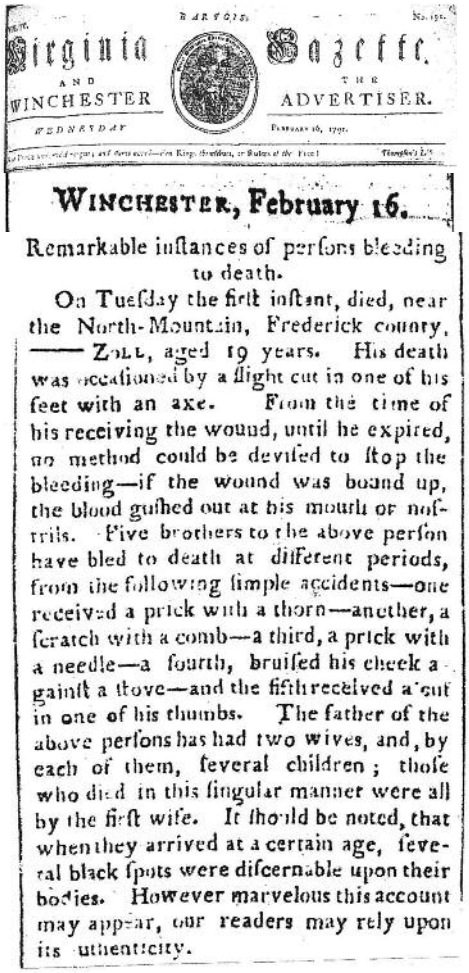
Think back to the last time you read something truly astounding about hemophilia. Wasn't one of your first reactions an urge, or even a compulsion, to share that bit of information? And maybe you did share, possibly using some form of popular social media. That's how things start going viral.

Today we have so many sources of information, but over 200 years ago, newspapers were the primary source of news. By 1790, America had almost 4 million residents living in 16 states who were served by over 250 (mostly weekly) newspapers. In the Wednesday, February 16, 1791, issue of the *Bartgis's Virginia Gazette and the Winchester Advertiser*, almost hidden on the third page, was an obituary for a 19-year-old.

This young man, whose last name was Zoll (and whose first name was never revealed), had bled to death on February 1 from an ax cut to his foot. Included with the short report was the astounding revelation that five unnamed brothers of this man had also bled to death. The Zoll family lived in the Cedar Creek community of Frederick County, Virginia, near other German settlers. The 1791 obituary of the Zoll brother is considered to be the first written report of hemophilia in America.

Winchester, the county seat of Frederick County, is located about 75 miles west of Washington, DC. In 1791, our nation's capitol was only an architect's dream because the land was still being surveyed, with no building started.





Virginia AND WINCHESTER Gazette THE ADVERTISER.

WEDNESDAY

WINCHESTER, February 16.

Remarkable inſtances of perſons bleeding to death.

On Tueſday the firſt inſtant, died, near the North-Mountain, Frederick county, —— ZOLL, aged 19 years. His death was occaſioned by a flight cut in one of his feet with an axe. From the time of his receiving the wound, until he expired, no method could be deviſed to ſtop the bleeding—if the wound was bound up, the blood guſhed out at his mouth or noſtrils. Five brothers to the above perſon have bled to death at different periods, from the following ſimple accidents—one received a prick with a thorn—another, a ſcratch with a comb—a third, a prick with a needle—a fourth, bruiſed his cheek a gainſt a ſtove—and the fifth received a cut in one of his thumbs. The father of the above perſons has had two wives, and, by each of them, ſeveral children; thoſe who died in this ſingular manner were all by the firſt wife. It ſhould be noted, that when they arrived at a certain age, ſeveral black ſpots were diſcernable upon their bodies. However marvelous this account may appear, our readers may rely upon its authenticity.

» page 12

Donar Beware... from cover

The Double-edged Sword of Social Media

Facebook has revolutionized communication, specifically social connection, providing a network of people with instant access to each other. People in the hemophilia community are now connected with thousands of other community members online–"friends" they would never have met in person or on the phone. A young man with hemophilia in America's heartland becomes friends on Facebook with a Moroccan youth who has hemophilia. How did they connect? Well, both are my Facebook friends, but I met the Moroccan fellow in person three years ago, and later friended him. On Facebook, friends can pirate other friends' friends. I have over 1,600 friends, almost all related to hemophilia, including many international friends due to my humanitarian work. Many of my overseas friends have connected with my US hemophilia colleagues and friends through *my* Facebook page. For friendship? Maybe. But increasingly, also for factor requests.

The good news is that with Internet, social media, email, and texting, requests for factor from developing countries to international hemophilia development nonprofits like World Federation of Hemophilia (WFH, headquartered in Montreal, Canada) and programs like Project SHARE can be processed the same day, increasing the likelihood that a child's life or limb can be saved with an emergency shipment of donated factor.

The bad news is that requests are also increasing to citizens not affiliated with international development organizations–and who are inadequately informed on what it takes to safely and legally donate factor.

Donating factor as one individual to another in a foreign country can help someone in need in the short term, but may undermine humanitarian efforts in the long term. It's a double-edged sword. Facebook and social media can lead us to make mistakes, because we may react emotionally rather than strategically and effectively, as part of a humanitarian program. Let's look at some caveats about getting involved in a factor request from overseas. First, the person making the request–the person in a developing country.

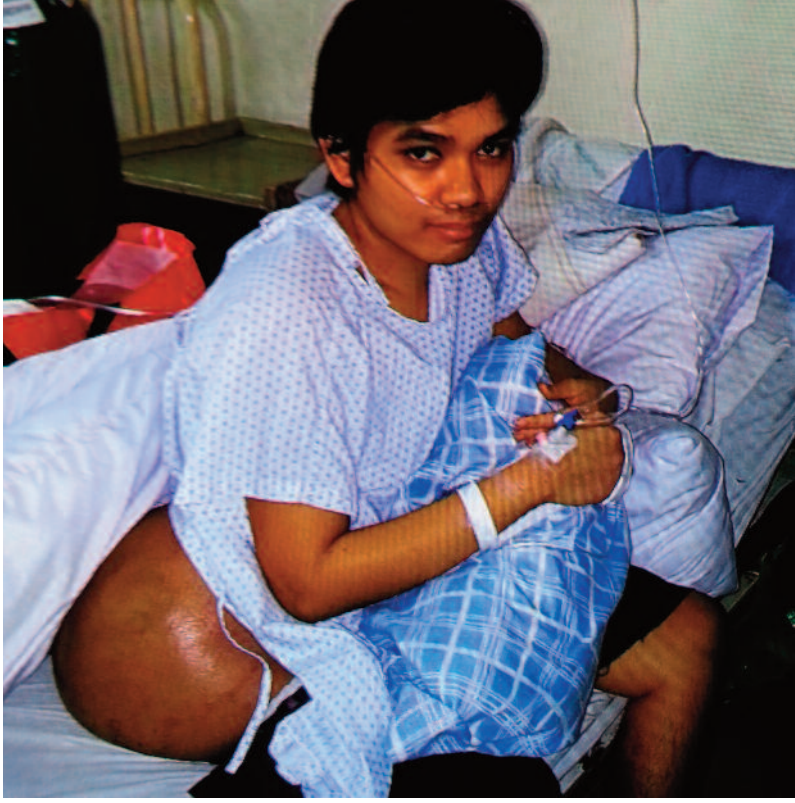
Is the Request Authentic?

Americans are known for being altruistic. We're often the first to toss a coin to a panhandler, write a check to a food bank, adopt a stray kitten, or try to save a country in turmoil.

We act charitably because we recognize a need, and we often respond emotionally to that need. But what if we *first* get more background information on the person we're helping?

How much do you really know about the person requesting factor? How can you verify the information he provides? Does he really have hemophilia? What type? Facilities to diagnose severity and type are lacking in many countries. What do you know about the country where he lives, and the available treatments?

"Bob" of New England was recently approached via Facebook to donate his factor to "Charlie" in the Bahamas. He wasn't sure what to do. Charlie said he had hemophilia, but Bob didn't know him outside of Facebook. Bob wanted to send Charlie some factor, but he called Project SHARE first. When we asked how Bob could be sure that Charlie had hemophilia, Bob knew he was in over his head and decided not to assist.



Surgery helped Jhon, but only because donated factor was available

Even if you're sure a Facebook contact has hemophilia, ask yourself: Is the request urgent? Is it valid? In developing countries with little or no factor, people with hemophilia may get desperate. A request may sound to you like an urgent plea. Or you may interpret the request as an emergency, propelling you to act swiftly.

Here's the problem: requests made via Facebook or email are *unilateral*–one person contacts another person. There is no way to verify the request or the person making the request. At SHARE, we require that each request be accompanied by a completed Factor Donation Request Form, downloadable from our website (www.kelleycom.com). To ensure the request is authentic, we ask for three signatures on each form: (1) the person making the request, (2) the patient's doctor, and (3) the national or local hemophilia organization, if one exists. We need proof given by someone who knows the patient, preferably a medical professional. Once the form is completed, SHARE creates a file for that individual, who we now know has hemophilia. But we still require a new form for each request, to verify the situation and bleed.

One advantage of this procedure is that it tells the doctor that the patient is in touch with foreigners. Imagine how upset your child's hematologist would be if you were getting blood-clotting medicine from a foreign source without the doctor's knowledge! Doctors need to know what's happening in the medical lives of their patients.

Second, submitting the form also requires the local or national hemophilia organization to get involved. The organization is responsible for tracking its patients with hemophilia.

Ideally, we want the organization to tell us, in each case, whether a donation is a good thing to do: "Please don't ship the medicine; we already gave this patient 20,000 IU." Or, "We know this patient, and the request is authentic."

Third, having a form signed by a hematologist clarifies the diagnosis. Some patients aren't sure if they are factor VIII or IX deficient. They may never have been tested. It's important to get a hematologist to verify the diagnosis. Unfortunately, you can't always accept what the patient tells you.

The more people in the country who know about the requested donation, the better. Project SHARE avoids unilateral donations unless we know the person well.

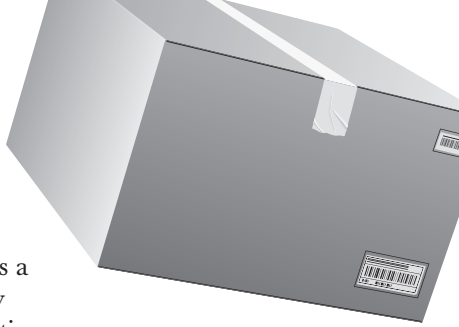
Factor donations can help if processed correctly

Shipping Up, Slipping Up

Even if you have personally authenticated a request, can you ship factor legally? Do you know how? Factor is a biological drug and requires temperature control to maintain its potency. It may take several days for a shipment to get to a country, and the factor can then end up in customs offices (which may not be air-conditioned) for days (or months without proper paperwork), waiting to be cleared. To efficiently and safely ship factor internationally, here's what's required:

- Understand the customs policies of the country to which you ship.
- Estimate the number of days the shipment will take to arrive.
- Know how to package the factor so it is not damaged.
- Provide the correct paperwork for customs.
- Pay for the shipment, which may cost several hundred dollars.

Shipping may seem less complicated if you ship within the US to the relatives or friends of your Facebook friend, who are flying back to their home countries and can courier the donated factor. But there are still concerns: technically, shipping drugs without a license across state lines–even to yourself, and even for charitable purposes–is a federal offense, although many people do it. And there are questions you must answer if you choose to ship:



- How much factor is acceptable to carry back to the home country?
- Should the factor be declared in customs?
- Is the US patient's name on the medicine?
- Will the factor be confiscated by customs?

If you choose to ship internationally, you should know that it is considered illegal to ship prescription drugs across borders, unless you have a commercial license or operate under a humanitarian program.

Shotgun Approach

Think about it. If one person with hemophilia contacts you via Facebook or email, might he not already have asked hundreds of others online too? This is the shotgun approach: many scattered requests might yield one successful hit. The shotgun approach may be successful for the patient making the request, but it could leave him with the lion's share of donations, bypassing others in more need.

About a year ago, SHARE received a query from a US hemophilia organization: a young man with hemophilia in Pakistan had emailed the organization, asking for a donation of factor. Could we advise? When I saw the forwarded email, I recognized the person requesting, because I had met him in 1999 while I was in Pakistan, and we had stayed in touch. An hour later, another chapter called us. Then another, and another. Clearly, this well-meaning man had used the shotgun approach and contacted all US chapters at once–without their knowledge–to solicit donated factor. We immediately sent an email to all chapters, informing them about the situation.

If we hadn't done this, and each chapter had acted on the request, one man might have ended up with a lot of factor. Not a bad thing, because this young man is trustworthy and helps many other people. But this isn't an effective use of resources. Twenty chapters all sending out various amounts of factor–and perhaps not knowing how to package, how to ship. There are more effective ways to handle such requests.

Beware the Bottomless Pit

It's a well-known phenomenon in charity: once you start giving to a cause or person, you'll be asked to keep on giving. People don't just appreciate your single charitable act and call it a day. Hemophilia is a lifelong chronic disorder, and many people in developing countries have little or no access to factor. Once you start giving, you are encouraging the recipient to expect charity, and that person will ask again. And again. Are you prepared for that?

About 75% of the world's population with hemophilia receives little or no factor

At SHARE, we have policies to "triage" overseas requests, much like an emergency room handles patients in order of need. We have no problem telling a patient to wait in line, no matter how well we know him. In our triage protocol, cranial bleeds come first, followed by gastrointestinal tract bleeds or neck and throat bleeds. Muscles and joints next, unless it's a complication like compartment syndrome. We never donate factor for use in prophylaxis: there simply isn't enough factor to go around. And forget about circumcisions and other elective surgeries.

On my recent visit to India, in November 2013, a doctor commented, "If we use the words 'free factor' with patients, then we are finished. The families will come to expect free factor all the time. They get angry when you don't have factor. They don't understand the time and effort it takes, the scarcity of the product."

Charity often involves an emotional connection–at least in the mind of the giver–but distributing scarce resources requires a tough, rational, strategic policy. At SHARE, we try to help every person who comes to us for factor, but we must triage. If our policies, such as providing the Factor Donation Request Form and requiring usage logs, are not followed, then we will not donate to that person or organization again. Patients and organizations who follow our policies assist us in doing our job to help people effectively and to prevent the bottomless pit of expectations that might occur.

Now let's look at what happens at your end, when you check out everything, the request is authentic, and you want to donate factor. What do you need to know in order to make the decision to donate? And what do you risk?

Can You Say "Insurance Fraud"?

There's no other way to say it: giving away your child's factor, or your own, is insurance fraud. You don't buy that factor out of pocket, so it isn't yours. Yes, you do pay monthly premiums for insurance coverage–for medicine needed by you or your loved one. If you use Medicaid or are a member of a state high-risk insurance pool, the state is paying these benefits for you, from taxpayer revenue. The factor is only for your use, not for anyone else's. Any way you slice it, *this is not your factor to donate.*

In almost all cases, donating your factor obtained through insurance is fraud. Donating or selling your prescription drugs to others is known as *drug diversion.* Drug diversion is a major problem: it drains health insurers of up to $72 billion a year and increases healthcare costs for us all. Drug diversion and insurance fraud are illegal, and the penalties can be severe. It doesn't matter how good-hearted and compassionate you are, or whether a child may die in Africa if you don't ship factor. It doesn't even matter if you plead ignorance–that you didn't know you couldn't donate factor.

When can you donate factor? You may be able to legally defend a donation if you or your loved one's factor is not needed (even though it is in-date); if your hematologist switches brands for medical reasons; or if the current brand is no longer working, as when when an inhibitor develops.

Can an HTC Donate Factor?

Yes. This is a different scenario. No insurance is involved. An HTC purchases factor and pays for it with revenues. So, in some cases, an HTC can legally donate factor. Of course, this may not be financially advisable because most HTCs have slim profit margins, but all institutions have a charitable arm and may consider a donation. The only caveat is when the HTC stocks factor for federal or state programs such as Medicaid. That factor, usually kept in a different inventory, cannot be donated.

HTCs must also be careful of liabilities: it's risky to dispense factor to a patient not registered under the HTC's direct care. "Margie," a kind-hearted HTC nurse, greeted an African woman who had entered the HTC asking for a donation of factor for a relative in Africa. Margie gave two vials, and only afterwards realized she hadn't asked any questions: Who was this woman? Who had the hemophilia? Was the patient under a hematologist's care at home? Margie phoned SHARE, nervous about what she had done, and we agreed that she should have done more research. When emotions run high, we may make poor decisions if we're in unfamiliar territory. And not many people have experience donating factor overseas.

Spreading the Wealth

US families with hemophilia who are originally from a developing country may be tempted to donate their factor back to their home country, to relatives with hemophilia still living there. At SHARE, it's a red flag when a US citizen offers factor to us to ship to a relative overseas. "José," originally from Ecuador but living in the US, once asked if we would ship 20,000 IU of factor IX, which he possessed, to his nephew with hemophilia in Ecuador. He told us he'd raised money at an office party to buy the factor. Must have been quite a party! Exactly how much money was raised? José hesitated, and said he'd forgotten. How had he bought the factor? From the manufacturer, José replied.

Impossible. And illegal in the US. Finally, José told the

truth: he was offering his own son's factor, and wanted us to transport it for him to his nephew in Ecuador. Insurance fraud was written all over this one.

SHARE offers humanitarian assistance, and we love to receive factor to donate, but we must be careful: factor prescribed for a US patient is not legally available to donate overseas (unless for documented medical purposes, as explained earlier). It happens, it's illegal, but there is little way to police this, short of requesting the empties back for a cross-check.

Undermining International Efforts

We've explored how a single shipment can cause trouble when you're not careful. But what's the bigger picture? How does aiding hemophilia patients overseas one-to-one undermine efforts by international agencies such as WFH that are engaged in global international hemophilia development?

Up to 75% of the world's 400,000 people with hemophilia have little or no factor. International agencies such as WFH are helping people with hemophilia in developing countries. These agencies work with the countries to improve hemophilia treatment over the long term. WFH specifically encourages each country's self-sufficiency: the ability to identify and diagnose patients, provide treatment, establish HTCs, educate patients, and raise public awareness. There are some established strategies to improve care:

- Meet with the Ministry of Health (MoH) of the developing country to encourage a *tender*, an international bid to purchase factor.[2]
- Work with local physicians and healthcare providers to train them properly in treating hemophilia.
- Establish diagnostic labs to provide accurate tests of type of factor deficiency and factor levels.
- Train hemophilia foundations or organizations–National Member Organizations (NMO)–to register and educate patients and have them seen by an HTC.

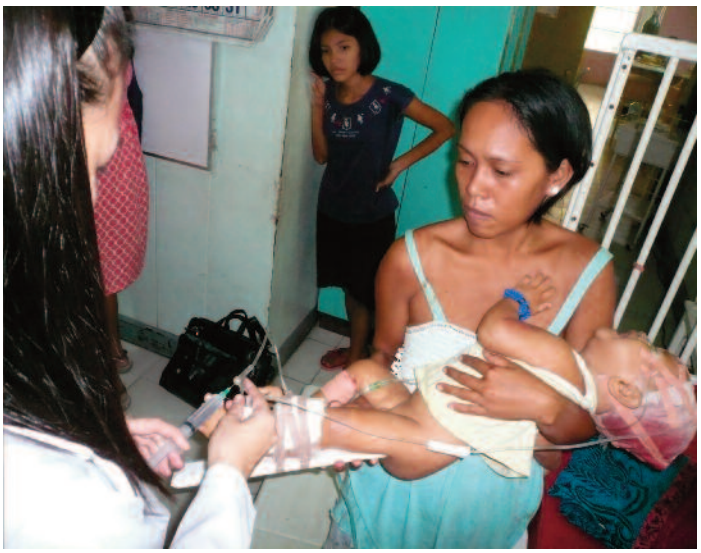
A high priority is securing the tender. But what's the incentive for a country to participate in a tender, when there are limited financial resources and the MoH believes it cannot afford to buy factor?

Factor donations.

WFH provides factor donations strategically to encourage governments to improve care. SHARE donates about $6 million per year in factor, while WFH can provide up to $33 million or more annually, as part of its Global Access Program (GAP). The factor manufacturers themselves typically donate large quantities of factor to WFH for GAP. WFH enters into a contractual agreement with the MoH of a carefully vetted country to provide free factor–if the government will start buying factor after three years.

The success of GAP depends in part on having patients come to treatment centers for clinic visits (not always easy in a developing country) and also participate in their hemophilia organization. This shows the HTC, government, and WFH that patients are registered, accounted for, monitored, and involved.

But if patients can get their donated factor from the Internet via their friends on Facebook, they may lose the incentive to visit the clinic, come to meetings, or volunteer at a local level. When people offer free factor to individual patients without first checking with the physician, HTC, or local organization, the strategies of hemophilia treatment in developing countries are circumvented and eventually weakened. The global plan of WFH and SHARE is to improve care for *all* patients with bleeding disorders in a developing country, not just the lucky few. And the lucky few on Facebook become more dependent on Americans, rather than building ways to be more independent in obtaining factor through their government–which takes hard work and advocacy.



Factor from Project SHARE is used for baby Christian Ramos, Philippines

Gathering information firsthand by visiting patients ensures donations are used properly

2. A national tender is a bid to purchase goods or services from international entities by a government. For factor, typically the government will announce a tender in the news media, and will request sealed bids on prices per unit by brand and by class of product (plasma-derived or recombinant). The idea is to encourage competitive prices among different manufacturers to meet the limited financial resources of a developing country.

Factor donations by individuals are not sustainable or desirable in the long run.

No one understands this better than Dana Kuhn, person with hemophilia and president of Patient Services, Inc. Dana has been a major US advocate for blood safety. When Dana was approached to donate factor to Atharv, he truly wanted to help. "When I see the pain and damage caused by inadequate or inaccessible treatment, it really tears me apart," says Dana. But he acknowledges that the big picture is crucial. "It's a good policy, to make sure patients are involved locally, and have an incentive to stay involved. You don't want them to become even more dependent on us."

Dana was one of several key hemophilia advocates in the 1980s and 1990s who researched incriminating documents that brought factor manufacturers to the legal bargaining table to secure settlements for hemophilia patients infected with HIV. He also was instrumental in pushing through the Ricky Ray Act, which resulted in government monetary settlements for the same group. Dana explains, "The passing of the Ricky Ray Act wasn't about me getting a settlement from the government as an HIV-positive person; it was how to help others [get a settlement] as well. To me, that's the highest form of charity. How do you help one person while helping the many?"

White Powder, Black Market

Does donating factor contribute to or cause a black market for factor in a country, so that factor doesn't go where it's most needed? This was the first question Jeff Moualim, development officer for Committee of Ten Thousand (COTT), asked when he was also approached to donate factor to Atharv. "Factor is expensive and in short supply in these countries," he notes. "I can see how it might set patients up for undesirable behavior."

A black market might mean that a factor donation gets diverted and sold–to a doctor or a patient willing to pay. Generally, the concern isn't so much a black market for factor as the threat of factor not being distributed fairly, to those most in need. "Mostly it's not the poor or needy who get the donated factor when it's solicited from Facebook," says Usha Parthasarathy, who served on the executive board of Hemophilia Federation (India) for 18 years. "The poor have no access to Facebook. Only to their own chapter. It's more experienced patients [who receive the donated factor]; this is unethical or self-serving. But in developing countries, it's survival of the fittest."

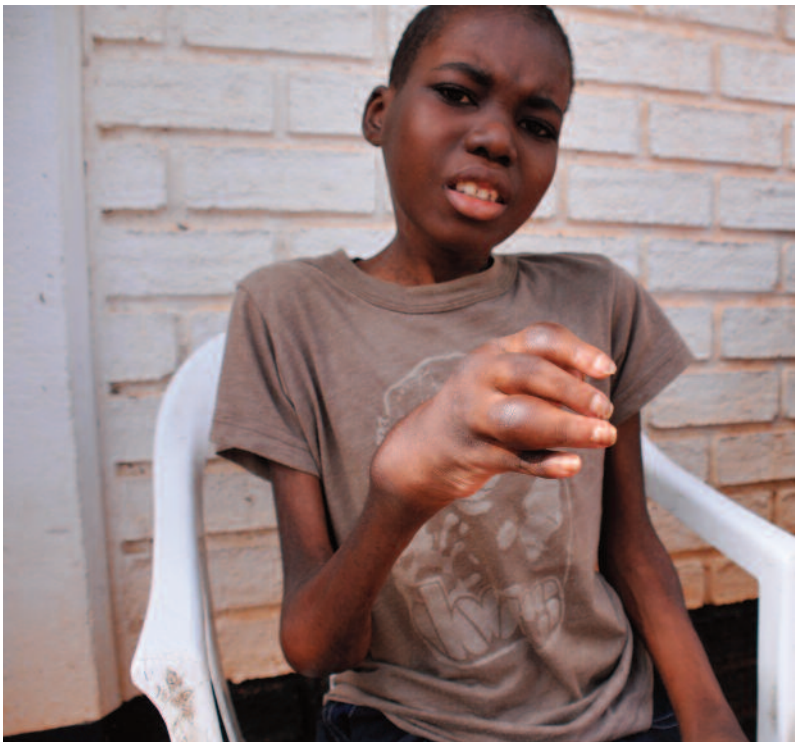
PROJECT SHARE

It's time to give back

Dana expands on this. "In the US, we feel a responsibility to help others." But, he warns, "Charity can be abused; if you are savvy, you can scam. A patient can say he will help others, but he could horde it and use it as a power instrument, instead of using it for the right reasons, to help those in most need."

At SHARE, we avoid the problems of black market and unfair distribution. How? By using our Factor Donation Request Form, which requires triple signatures. This informs everyone of the request, but also allows the national organization and HTC to strengthen their ability to solve problems. All

Most hemophilia patients live in substandard housing, villages, or slums



Factor donations to Zimbabwe keep Brian alive

How much do you really know about someone who contacts you online to request factor?

requests are vetted through the local or national hemophilia organization in a country. India has more than 65 chapters, so a local chapter is best. By contrast, the Dominican Republic has only one national organization, so a patient request will be handled through it.

Our philosophy is to never primarily "rescue" a patient, but to work with the country's infrastructure–its HTCs and hemophilia organizations–to solve what is really the country's problem: the lack of factor locally.

Facebook Friends Don't Let Friends Commit Fraud

Kristin Marema, mother of a child with hemophilia, volunteers with Midwest Hemophilia Foundation (Kansas City, Missouri) managing the foundation's Facebook page. She was also contacted to donate factor recently, and has noticed increasing requests for factor via Facebook from patients in developing countries over the past three years.

"This is first time I've ever been personally asked," notes Kristin. "I'm picky [about] who I am friends with on Facebook. A patient from India said [his] chapter had people in need. I was able to get a bit to donate. I was supposed to hear back from our contact when it was received, but I haven't heard anything. Who knows where it is?"

Kristin now wonders if she did the right thing. "I received the request right after my son Trenton had a rough time with his tonsillectomy, and I was emotional, thinking if we didn't have factor, Trenton would have died, so I was operating from an emotional decision. And after I shipped the donation, I thought, I shouldn't have done that. I worried about its potency. What if they used it to save a child's life, and it didn't work?"

Next time? Says Kristin, "I would not personally donate factor again. Legal ramifications did not cross my mind; after the fact, I thought, would it get tracked to me?"

Kristin believes that chapters, and maybe even National Hemophilia Foundation (NHF), need to focus on educating the community about the increasing use of Facebook for factor solicitations from overseas, the legal consequences, and safer ways to donate. This has never been more important, as NHF is now forging closer ties with WFH, and with Nigeria in particular as part of WFH's Twinning program, which links hemophilia organizations in developed countries with those in developing countries.

"I think chapters need to have a policy about donating factor," stresses Kristin. "In our state, stay-at-home moms are on Facebook a lot, and it's easy to 'friend' people, though you don't know who they are. They could just be putting up a front, and yet you are shipping them factor. Some families don't have money to donate to help patients overseas, so they think that donating factor is a way to help."

A Simple Solution

There's an easy solution to handling a factor donation request that arrives via Facebook, email, or any social media.

Alert Project SHARE. There's a good chance that we know the patient already; it's a small world when it comes to hemophilia. After 18 years of donating factor, SHARE has a robust and efficient program that has worked in more than 70 countries. We almost never lose a shipment to customs, and we have saved many lives. We use donations as a tool to build a stronger hemophilia organization in the country–by involving the organization, requiring it to check on its own patients, find its own solutions first, and return log sheets of factor usage to SHARE.

We also notify WFH, so it knows exactly where each shipment goes. Knowledge, freely accessible, with several parties involved, is the key to honesty and efficiency. And factor–that liquid gold–is the key to building stronger hemophilia programs in developing countries, with the involvement of the government, healthcare workers, and patient organizations.

Remember: in your own efforts to help, don't rely on your emotions. Think strategically. Don't weaken the system by trying to act on your own.

And what became of Atharv, the young man from India who contacted so many people in December? He was legit. He was acting to help the people in his chapter. Score one for honesty and charity. But let's win this game going forward: tell Project SHARE about any request for factor you receive from a developing country. In turn, we will ensure that the people who need factor the most are helped. And we'll coordinate with WFH and national or local hemophilia organizations while we work to improve care for all in the developing world.

Visit www.kelleycom.com and click on the SHARE logo to read about our policies, download forms, access our annual reports, and read personal stories of people we have helped. Visit Gallery to see photos of our trips and the many families whose lives we have touched.

Richard's Review... from page 5

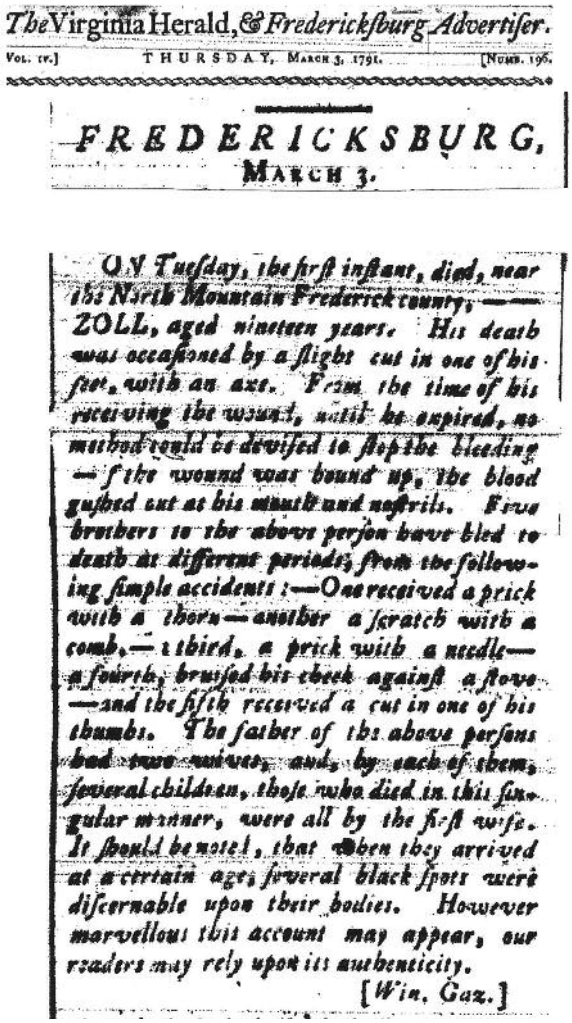
Bartgis's Virginia Gazette, the newspaper that first published the Zoll obituary, had its office in Winchester. Similar to today's practices, editors at that time culled interesting articles from other newspapers to spread the news or possibly to fill space in their papers. So the editor of the *Virginia Herald and Fredericksburg Advertiser* in Fredericksburg, Virginia, about 45 miles south of Washington, DC, reprinted the Zoll obituary in the Thursday, March 3, issue with a few revisions: the header was dropped, and the entire text was italicized–perhaps to contrast it with surrounding notices, or to provide more emphasis. The Fredericksburg newspaper acknowledged the original source with the slightly inaccurate abbreviation "Win. Gaz."

Then on Wednesday, March 16, the *Pennsylvania Herald and York General Advertiser* in York, Pennsylvania, reprinted the Zoll obituary with only minor alterations from the original. A noticeable error was the incorrect date of death: "the first instant" (or Feb. 1) was printed as "the 11th instant" (or Feb. 11). The York newspaper listed two sources for the obituary: first, "Georgetown, Feb. 26," most likely that issue of the *George-town Weekly Ledger* in Georgetown, Maryland, or a correspondent there; and second, the inaccurate abbreviation "Wm. Gaz." York is located about 90 miles north of Georgetown, now Washington, DC. The area of Pennsylvania surrounding York is significant because the Zoll family is thought to have lived in Earl Township of Lancaster County before moving to Virginia in 1777, so any remaining friends or relatives would have had an interest in the obituary.

Next, on Tuesday, March 22, the *Salem Gazette* in Salem, Massachusetts, published an abbreviated version of the original Zoll obituary. The medical history, with a sentence mentioning several black spots that were found on the brothers' bodies, was omitted. But more surprisingly, the 19-year-old Zoll brother who bled to death was given the first name Isaac. How the editor of the Salem newspaper knew that information remains a mystery. But it was the Salem newspaper's version of the Zoll obituary that later provided the connection to hemophilia when the obit was mentioned along with another family of "bleeders" (as people with hemophilia were known at the time) in an 1834 history text on the nearby Massachusetts town of Ipswich. That obit was again mentioned in a 1962 medical journal article by Victor McKusick of Johns Hopkins University. The existence of the original Winchester newspaper obituary, plus the newspaper reprints in Fredericksburg and York, are recent discoveries–and possibly more newspaper reprints wait to be discovered.

The newspaper obituary for a 19-year-old man named Zoll who died on February 1, 1791, offers several lessons on sharing information about hemophilia. Printed matter, an important source of information in 1791, is still crucial today, though now we may also rely on many other sources. Yet we always need to ensure the accuracy of what we read, because mistakes can be passed on when something is shared, telephone-game style. And we need to understand what we are reading. Newspaper readers in 1791 didn't know that they were reading about hemophilia in early America; they only knew that the human story was interesting, and they wanted to share it. Of course, we can now go viral instantly on today's electronic devices, but have we really changed that much over the past 200 years?

For more information on the 1791 newspaper obituaries that are recognized as the earliest written record of hemophilia in America, or for the list of references used in this article, please contact Richard at info@kelleycom.com or visit www.kelleycom/newletters.html.



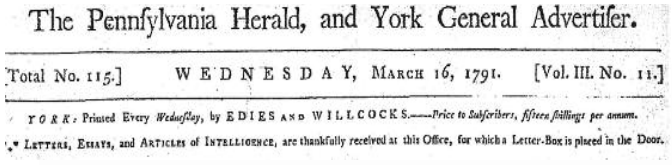
The Virginia Herald, & Fredericksburg Advertiser.

Vol. IV.] THURSDAY, March 3, 1791. [Numb. 196.

FREDERICKSBURG, March 3.

ON Tuesday, the first instant, died, near the North Mountain Frederick county, —— ZOLL, aged nineteen years. His death was occasioned by a slight cut in one of his feet, with an axe. From the time of his receiving the wound, until he expired, no method could be devised to stop the bleeding—if the wound was bound up, the blood gushed out at his mouth and nostrils. Five brothers to the above person have bled to death at different periods, from the following simple accidents:—One received a prick with a thorn—another a scratch with a comb.—a third, a prick with a needle—a fourth, bruised his cheek against a stove—and the fifth received a cut in one of his thumbs. The father of the above persons had two wives, and, by each of them, several children, those who died in this singular manner, were all by the first wife. It should be noted, that when they arrived at a certain age, several black spots were discernable upon their bodies. However marvellous this account may appear, our readers may rely upon its authenticity.

[Win. Gaz.]

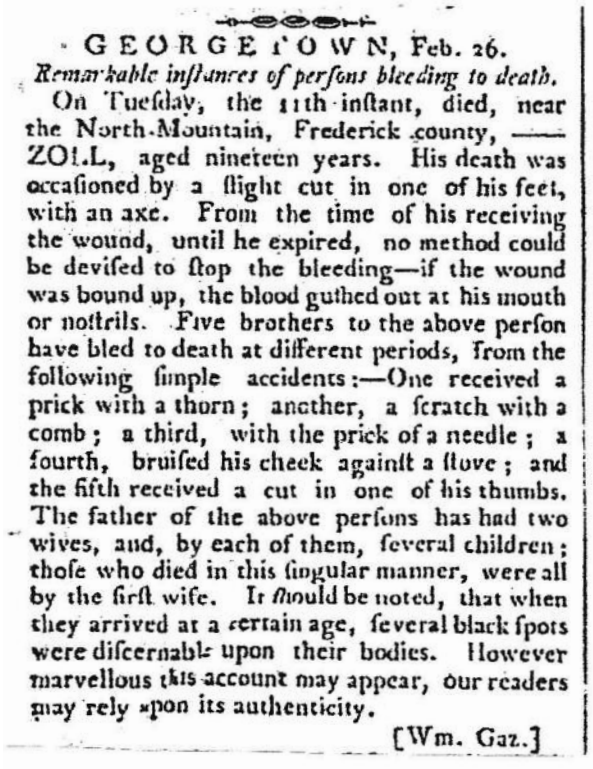


The Pennsylvania Herald, and York General Advertiser.

[Total No. 115.] WEDNESDAY, March 16, 1791. [Vol. III. No. 11.]

YORK: Printed Every Wednesday, by EDIES AND WILLCOCKS.—Price to Subscribers, fifteen shillings per annum.

** LETTERS, ESSAYS, and ARTICLES of INTELLIGENCE, are thankfully received at this Office, for which a Letter-Box is placed in the Door.



GEORGE TOWN, Feb. 26.

Remarkable instances of persons bleeding to death.

On Tuesday, the 11th inſtant, died, near the North-Mountain, Frederick county, —— ZOLL, aged nineteen years. His death was occaſioned by a ſlight cut in one of his feet, with an axe. From the time of his receiving the wound, until he expired, no method could be deviſed to ſtop the bleeding—if the wound was bound up, the blood guſhed out at his mouth or noſtrils. Five brothers to the above perſon have bled to death at different periods, from the following ſimple accidents:—One received a prick with a thorn; another, a ſcratch with a comb; a third, with the prick of a needle; a fourth, bruiſed his cheek againſt a ſtove; and the fifth received a cut in one of his thumbs. The father of the above perſons has had two wives, and, by each of them, ſeveral children; thoſe who died in this ſingular manner, were all by the firſt wife. It ſhould be noted, that when they arrived at a certain age, ſeveral black ſpots were diſcernable upon their bodies. However marvellous this account may appear, our readers may rely upon its authenticity.

[Wm. Gaz.]

Emphasizing Education:

Spotlight on Scholarship

Evan Poole

Travis Albright

Mike O'Connor

Patients and families in the hemophilia community face challenges that extend beyond treatment. For some, the cost of attending college or technical/vocational schools can be significant. Costs including tuition, books and supplies, room and board, health insurance, and transportation can add up quickly.

The hemophilia community is fortunate to have numerous scholarship programs available for current and future college students with bleeding disorders. The Soozie Courter Hemophilia Scholarship is a Pfizer-sponsored tuition assistance program, named in honor of Soozie Courter, a valued and respected friend of the hemophilia community who passed away in 1999.

For the 2013–2014 academic year, $50,000 in scholarships was awarded to 17 graduate and undergraduate students with hemophilia who presented the best combination of creative and persuasive essays, excellent recommendations, and superior academic standing. Through their involvement in local hemophilia chapters and mentorships, as well as their commitment to future plans, these students personify what it means to overcome challenges to make a difference in their communities.

Evan Poole never let hemophilia B get in the way of his schoolwork or athletic pursuits. When a bad bleed meant missing school, he always stayed on top of his assignments. And he found athletic passions, such as golf, that he was able to pursue. Evan is now in his first year studying engineering at Trine University in Indiana. He was also selected to take part in the National Hemophilia Foundation (NHF) National Youth Leadership Institute (NYLI), based in part on his significant involvement in the hemophilia community. Although Evan was initially anxious about managing his condition away from home, without his parents, he is now excited about the challenge of college!

Travis Albright, a University of Michigan senior with hemophilia A, first became involved in the hemophilia community when he was 10 years old and attended Camp Bold Eagle in Muskegon, Michigan, run by Hemophilia Foundation of Michigan (HFM). His commitment to the community steadily evolved year after year, as he too became involved with NYLI, and also with HFM's MYLIFE youth leadership group. Travis became a mentor to youth with hemophilia, and encouraged peers to educate themselves about ways to successfully live with their disorder. Following his passions to educate policymakers about hemophilia and advocate for access to treatment, Travis landed an internship in Washington, DC, where he assisted Rep. Gary Peters and was invited to speak at NHF's annual Washington Days event. He is now working to complete his degree in public policy.

» *page 15*

Insights... from page 4

standard procedure was to wait and see what kind of "bleeder" each patient would be before surgically implanting a port-a-cath and starting prophylaxis. Later that month, Tyler had his second bleed, and I started to fight for prophylaxis. I think the fact that Tyler had an unfamiliar mutation,[1] and was already having bleeds, made the doctors nervous about what was to come. They took an aggressive approach, and prophylaxis was started immediately.

Q: Did prophy go smoothly?

Yes. When Tyler was seven months old, he had a port surgically implanted so he could be infused prophylactically at home. Surgery went fine, using BeneFIX [factor IX] to control his bleeding. All was going as planned, and within the next week I'd be infusing Tyler at home. No more late-night runs to the hospital! Life on prophy would be much better for Tyler, and would give me the peace of mind I desperately needed. "This is the first day of the rest of our lives!" I told my family and friends the morning of our first scheduled prophy treatment. That turned out to be the biggest understatement.

The first time Tyler's port was accessed, he had an anaphylactic reaction to the BeneFIX. He had another the following day in the hospital, and a low-titer inhibitor was soon detected. I hadn't known this could happen. I remember saying, "What are these bumps on his back?" because I had never seen a hive before. The next thing I remember is the nurse saying, "I don't like his coloring" and running out of the room. Within seconds, a full staff ran in, and the paramedics were called. It all happened so fast–I had no idea what was going on. It was the scariest day of my life. The next day I was told prophylaxis would never be an option; factor IX could not be used again, or so we thought.

Q: How did you manage the stress you felt?

There were no other families I could relate to. All the other young boys in our community were on prophy. I tried to find information online about factor IX and anaphylaxis, but there wasn't much in 1999. Even my bible, *Raising a Child with Hemophilia*, had only two sentences on the subject. Apparently, inhibitors in factor IX patients are uncommon–only 1% to 3% of patients get them. I was desperate to find someone like Tyler so I wouldn't feel alone.

Q: How did the doctors treat Tyler at this point?

For the next five years, we used factor VII on demand for Tyler's bleeds. During this time, he suffered countless bleeds and developed a target joint in his left ankle that later required an arthroscopic synovectomy. Life consisted of excruciating bleeds that could not be prevented–just treated. One of Tyler's worst bleeds was an uncontrollable ankle bleed that required a dermal drip, as well as morphine pushed every two hours to relieve his pain. He was four years old, and things seemed out of control.

Not long after, I learned firsthand about other serious bleeds and complications such as compartment syndrome. We basically were running to the hospital every week. Phoenix Children's Hospital became our second home. Tyler called it "my hospital." It seemed like every time I put on my PJs for bed, a crisis would hit. A fever that required a blood culture at Phoenix Children's ER, a bleed causing uncontrollable pain–the list goes on and on. I started going to the ER in my PJs; I was too tired to even care. I don't think I realized how bad things had become. We had progressively reached this point, and it became our reality.

During this time, I was able to treat Tyler at home with NovoSeven, but constantly doing infusions every two hours for days on end, all on my own, was unrealistic and terrifying. I had kept up this schedule many times before, until one day my hematologist said, "Just let me know when you feel you've had enough, and we can admit him." Apparently, I had missed this option. I said, "What? You'll admit him to treat a bleed?" *Note to self:* Pay better attention! Nevertheless, admitting Tyler for serious bleeds made life easier and brought me some peace of mind.

Q: What about immune tolerance therapy (ITT)?

After several years of this crazy lifestyle, Tyler's hematologist stated that his "quality of life" was very poor, and the decision to try to desensitize him to factor IX was made. By 2005, when Tyler had just turned six, there were reports of boys in Japan having success with desensitization to factor IX, so we were encouraged. [Attempting to desensitize hemophilia B patients using ITT is less successful than in hemophilia A: ITT is successful in only about 36% of patients with hemophilia B, compared to the almost 80% success rate

1. This was a substitution in a location on the gene that hadn't been seen before.

in hemophilia A. About a third of hemophilia B patients on ITT[2] also develop kidney problems–nephrotic syndrome.]

Our first two attempts at ITT were done in Phoenix Children's Hospital. Tyler was in ICU for three weeks the first time, and on the hematology/oncology floor for the second attempt. After three weeks in ICU, we were sent home feeling optimistic, because his allergic reactions had stopped. Within three weeks, the reactions started again, and we returned to the hospital for round 2.

The second attempt lasted another three weeks and really took its toll on Tyler. He had ballooned up due to the steroids, gaining seven pounds in three weeks. He looked miserable and was depressed–not smiling, laughing, or really talking. This was our lowest point. I had my first major breakdown–well, that I can remember, though the nurses might say otherwise.

The second attempt at ITT failed weeks after returning home, as Tyler's reactions slowly returned. With every daily infusion, I feared anaphylaxis. Would his reaction be just hives, coughing, itchiness, shortness of breath, or would it proceed to a life-threatening anaphylactic reaction? I didn't know which, if any, symptom Tyler would have on any given day, but he seemed to tend toward severe reactions. It was just the two of us home alone with an EpiPen and cell phone. Eventually, the reactions got serious enough that infusing at home was no longer an option. Once again, no more factor IX.

2. This figure is higher for patients with severe allergic reactions, such as Tyler.
3. CADD stands for computerized ambulatory drug delivery.

Q: What happened after two attempts at ITT failed?

For the next eight months, I felt defeated. Our options were running out, but I wasn't ready to quit, and thank goodness neither were our hematologist and HTC nurse.

A new plan was hatched. Tyler would be desensitized by receiving factor IX continuously. He would get a Broviac port, attached to a CADD[3] pump to allow this. When presented with the idea of connecting a pump to infuse factor 24/7, I imagined a little diabetic pump. I admit I was upset when this big CADD pump showed up. Compared to a diabetic pump, it was huge! My sister took the biggest CD player carrying pouch we could find and converted it into a CADD carrying pouch. Tyler wore this pump around his waist for 18 months. At his post-ITT prophylactic peak, he was receiving 20,000 IU per day of factor IX, in an attempt to overwhelm his immune system and keep the inhibitor from returning.

Then, after Tyler ran through reclaimed irrigation water with the neighborhood kids one day–resulting in a port infection–the hematologist decided it was time to get rid of the pump, and while we're at it, the infected port. If Tyler was trying to get rid of his pump, he couldn't have done a better job! At this point, one large (3,000 IU) daily bolus of factor was enough to prevent the allergic reactions. This was a victory for Tyler. He was now seven years old and able to self-infuse!

To be continued…Read more of Tyler's story in the May/June issue of PEN.

Emphasizing Education... from page 13

Michael O'Connor, a graduate student with hemophilia B, says he thinks of his life with hemophilia in two phases: before and after he started swimming. Swimming became a passion as well as a way to help Michael get in tune with his body and better manage his condition. He swam competitively for many years and also started coaching. Michael believes that if you are smart about yourself and your body, work hard, and do what you love, your plans for life will work out in the end. And things are working out for Michael. He is back in the water–but in a different way. He is currently pursuing a master's degree in geosciences at the University of Texas–Austin, where he's taking part in research that focuses on quantifying the role of coastal delta islands in filtering out nitrogen in the Mississippi River before it reaches the Gulf of Mexico and causes ecological damage.

We wish these scholarship winners–and all students–much luck and success in the coming school year and beyond.

For more info on the Soozie Courter Hemophilia Scholarship program, and to see video clips of Evan, Travis, and Michael, visit hemophiliavillage.com and click on "Scholarship assistance" in the lower right, and visit www.facebook.com/OurHemophiliaCommunity.

EDUCATIONAL ANNOUNCEMENT SPONSORED BY PFIZER INC.

headlines

PHARMA

NovoEight: One Step Closer

The US FDA has approved Novo Nordisk's Biologics License Application (BLA) for NovoEight, the company's recombinant factor VIII (rFVIII) product, for use in adults and children with hemophilia A. Novo Nordisk plans to launch the product soon after April 2015, after expiration of existing patents held by competitors. Why this matters: An alternative rFVIII product allows more choice, and may affect prices.

For info: www.novonordisk.com (search turoctocog alfa)

Long-Lasting Factor VIII Competition

Baxter International has completed enrollment in its phase III clinical trial of BAX 855, an investigational, extended half-life, recombinant factor VIII product to treat hemophilia A. Called Prolong-ate, this study is evaluating BAX 855 in 146 adult patients with previously treated severe hemophilia A. The trial is assessing safety, immunogenicity, and efficacy of BAX 855 in reducing annual bleed rates in both prophylaxis and on-demand treatment schedules. Why this matters: BAX 855 will be a direct competitor to Biogen Idec's Eloctate.

For info: www.baxter.com

New FEIBA Indication

Baxter received FDA approval of FEIBA for prophylactic treatment of hemophilia A and B patients with inhibitors. Approval is based on data from a phase III study in which treatment with a FEIBA prophylactic regimen showed a 72% reduction in median annual bleed rate (ABR) compared to treatment with an on-demand regimen. Why this matters: The presence of an inhibitor makes treatment more challenging, and patients with inhibitors have an increased risk of developing complications. Fewer bleeds means less joint damage.

For info: www.baxter.com

Factor X Product on Horizon

Bio Products Laboratory (BPL) announced that it has submitted a BLA to the FDA for the world's first purified factor X concentrate. Why this matters: Factor X deficiency is an ultra-orphan bleeding disorder that affects an estimated 1 in 500,000 to 1,000,000 people; only about 50 cases of congenital factor X deficiency are documented worldwide.

For info: www.bpl.co.uk

Eloctate Expected Mid-2014

Biogen Idec will postpone marketing of Eloctate, its long-lasting recombinant factor VIII product, in the US until mid-2014. The delay will give Biogen time to comply with an FDA request asking for more information about the way the manufacturing data are collected and reported. Why this matters: Eloctate will be the first significant advancement in hemophilia treatment in 10 years.

For info: www.bloomberg.com

Factor XIII Patients Get New Treatment

The FDA has approved Novo Nordisk's Tretten® (coagulation factor XIII A-subunit [recombinant]) for routine prophylaxis of bleeding in people with congenital factor XIII (FXIII) A-subunit deficiency. This is one of the rarest inherited bleeding disorders, with an incidence of 1 in 1 million to 1 in 5 million. FXIII is composed of two subunits, A and B, with genes on two different chromosomes. FXIII deficiency is usually caused by a deficiency of the A-subunit. In the absence of FXIII, loosely formed clots are developed, leading to bleeding complications similar to those in severe hemophilia A. Tretten is the only recombinant treatment for the disorder. Why this matters: Patients with congenital FXIII A-subunit deficiency have a lifelong susceptibility to bleeding, including intracranial hemorrhage (spontaneous bleeding into the brain), which could be life-threatening if untreated.

For info: TRETTEN-US.com

WORLD

Scotland to Produce Factor

ProFactor Pharma is developing a novel manufacturing process for a recombinant factor VIII to treat hemophilia A. ProFactor Pharma secured funding from Scottish venture capitalists Kelvin Capital earlier this year, enabling the company to progress in developing its production process. Why this matters: This new process may help Scotland become more self-sufficient in factor production.

For info: www.profactorpharma.com

Project Recovery

Canadian Blood Services (CBS), Biotest, Grifols, and the World Federation of Hemophilia (WFH) have launched an innovative program to salvage unused factor VIII left over from the manufacture of plasma products from Canadian blood donors. The WFH Humanitarian Aid Program will then supply the resulting factor VIII concentrate to hemophilia A patients in developing countries. Why this matters: It's projected that at least 5 million international units (IUs) of factor VIII will be donated each year, treating approximately 5,000 joint hemorrhages annually.

For info: www.wfh.org

NONPROFIT

HFA's 20th Anniversary Symposium

March 27–29, 2014

Tampa, Florida

Hemophilia Federation of America's (HFA) annual symposium, "Honoring Our Past, Building Our Future," is a community-centered educational event that draws over 400 patients and their families, 100 exhibitors, and 20 speakers. Over 100 travel scholarships for first-time attendees are awarded each year to families needing financial assistance. Why this matters: This national meeting is a place where community members come together to share information, learn new advancements, and build a network of support.

For info: www.hemophiliafed.org

Plan Now for NHF's 66th Annual Meeting!

September 18–20, 2014

Washington, DC

At last year's annual meeting, nearly 3,000 community members gathered in Anaheim, California, including families with bleeding disorders, physicians and researchers, HTC providers, chapter staff and volunteers, and industry partners. Why this matters: This is the largest national gathering of hemophilia community members in the world.

For info: www.hemophilia.org

New Website for Rare Disorders

NHF has announced a new website for people with rare bleeding disorders and their healthcare providers. Why this matters: This is the first dedicated site for rare disorders including factor V and XI deficiencies.

For info: www.rarecoagulationdisorders.org

ADVOCACY

HIV Patients' Organs May No Longer Be Turned Away

The US House of Representatives passed the HIV Organ Policy Equity (HOPE) Act on November 12. Once signed into law, this would allow "positive-to-positive" organ donations, permitting organs from deceased, HIV-positive donors to be transplanted to HIV-positive recipients. Why this matters: The HOPE Act has the potential to save 1,000 HIV-infected patients with liver and kidney failure each year.

Source: medcitynews.com

SCIENTIFIC

Inhibitor Study

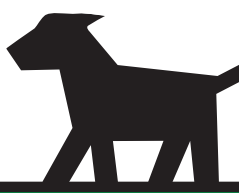
A large multicenter retrospective study of 1,112 mild to moderate hemophilia A patients in Europe and Australia found that people with certain mutations to the factor VIII gene have a significantly higher risk of developing inhibitors, as compared to other mild and moderate patients. Mild and moderate hemophilia A are usually caused by "missense" mutations, in which a single nucleotide in the factor VIII gene is different. Among a total of 214 missense mutations in the factor VIII gene able to cause mild or moderate hemophilia, 19 were identified as being associated with inhibitor development. The study also found that the risk of developing inhibitors in people with mild to moderate hemophilia A continued to increase up to 100 exposure days (exposure to factor VIII). This means that some people with mild or moderate hemophilia A, because of infrequent factor use, may still be at risk of developing inhibitors as they get older. This is in contrast to severe hemophilia A, in which the risk of developing inhibitors after more than 50 exposure days decreases to less than 1%, and most people with severe hemophilia A exceed 50 exposure days within the first few years of life. Why this matters: This study highlights the potential value of factor VIII *genotyping* (determining the sequence of nucleotides in DNA and the type of mutation) to estimate individualized risks of inhibitor formation.

Source: *Blood*, Sept. 12, 2013

Gene Therapy for Dogs Raises Hopes for Hemophilia

A research team led by David Wilcox at Medical College of Wisconsin, Milwaukee, used a virus as a microscopic Trojan horse in gene therapy tests on dogs. The team reported treating hemophilia in dogs by fixing a flawed gene. A functioning version of a gene called ITGA2B was tucked into a harmless virus. The virus was then used to infect three dogs with hemophilia A, delivering the good gene into stem cells that make platelets to clot the blood. Two of the dogs producing the highest levels of factor VIII after the therapy had no episodes of severe bleeding throughout the two-and-a-half-year duration of the study. Why this matters: Successful gene therapy in dogs marks a step forward in treating hemophilia in humans.

For info: www.mcw.edu



Parenting Moment

You cannot help someone get up a hill without getting closer to the top yourself.

— *General H. Norman Schwarzkopf*

Service to others is the rent you pay for your room here on earth.

— *Muhammad Ali*

inbox

I'M INTERESTED IN LEARNING MORE ABOUT THE recombinant factor product for von Willebrand disease. Your publication had some info on this ["New Factor Concentrates," PEN, Aug. 2013], but stated that a release date would be in 2016. Do you know the source for that info? The clinical trials are currently scheduled in be completed in 2014.

I wonder why the long product delay compared to other products and trials listed.

ANONYMOUS

Ed.: Baxter has not issued a press release on its BAX 111 (vonicog alpha) recombinant VWD treatment since October 2011. The current status on the development of this drug is unknown. The projected 2016 release date comes from a marketing research report, and is based on the expected completion date of the phase III clinical trial (March 2014), after which Baxter would have to apply to the FDA for a Biologics License Approval, which may take a year or more. If approved, production facilities would have to be built and also approved by the FDA.

Project SHARE

I AM GLAD TO INFORM YOU THAT RAJIB, A SAVE One Life and Project SHARE beneficiary, purchased a refrigerator with your financial assistance. Ms. Kelley and Mrs. Usha Parthasarathy visited his home and gave him 12,000 rupees to purchase a refrigerator for storing factor. Rajib's entire family is grateful for your visit to their small home. As a secretary of Durgapur Chapter, I also express my sincere gratitude for your continuous support.

AJOY ROY
Durgapur Chapter
Hemophilia Federation (India)

On behalf of my family, I want to thank you for the donation of factor IX for my son, Mikhail. Your assistance has been invaluable. We sincerely appreciate the help and generosity you have provided.

Mikhail had multiple teeth extracted on Dec. 16 under the supervision of Dr. Flery Hernandez. May the good Lord continue to bless you and your company that you may continue to be a blessing to the many people in need.

Maria Thelma L. Palacio
Philippines

Thank you for this valuable support [of factor]. During this summer we had a boy with severe intracranial bleeding, in a coma, in a life-threatening situation, who needed an emergency surgical intervention. Now he is fine, but it was so important to have had at the right time, in the right amount, the right medication.

We are so grateful for your support. We had our society's general assembly on Nov. 30, and distributed factor to more than 25 people, assuring at-home prompt treatment. It has been a wonderful gift for the winter season.

Dr. Margit Serban
Romania

On behalf of Hemophilia Society Mumbai Chapter (HSMC), we are so grateful to you for making yourselves available for our camp in India for three days in spite of your hectic schedule. We also thank you for supporting the camp through factor and funding.

This humane effort truly speaks volumes about your care and concern for the underprivileged, and your whole-hearted commitment to making a difference in the lives of those affected with hemophilia.

We look forward to your continued support in future too.

Indira Nair
Hemophilia Society Mumbai Chapter

Pulse on the Road

We really enjoyed the Pulse on the Road presentation. We are lucky to have good insurance, and we pay attention to the costs pretty carefully, but we still learned a lot about the details and how the marketplace as a whole works. It's also great to hear about all the positive changes happening as part of the Affordable Care Act that have such an impact on the opportunities my boys will have someday.

Thanks for everything you do for the hemophilia community in the US and elsewhere.

Briana Reinking
Colorado

Laurie Kelley attends hemophilia camp in Mumbai, India, November 2013

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HEMOPHILIA

Here's to your defining moments.

Living with hemophilia isn't just about hemophilia, it's about life … and the incredible moments that define it. This understanding is the driving force in everything we do, whether developing new therapies or creating meaningful programs.

So here's to you and to the defining moments that inspire all of us.

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