

Mark Zatyryka, West Suffield, CT
HB 5212

5212
M. Zatyryka

First, I want to thank Rep. Jarmoc and Rep. Conway for allowing me to participate as a member of the Bleeding Disorder Working Group and for all you have done to support the bleeding disorders community. I also want to thank Rep. Fontana and the Insurance and Real Estate Committee for agreeing to support HB 5212, an Act Concerning Insurance Coverage For The Treatment of Bleeding Disorders and for allowing me to testify here today.

My name is Mark Zatyryka and I live in West Suffield, CT. Soon after I was born, my parents were told I had severe hemophilia A, a bleeding disorder that prevents my blood from clotting.

Hemophilia has and always will affect all aspects of my life. Hemophilia is extremely painful and I have had severe pain for as long as I can remember. I am on morphine and oxycotin and it barely helps the pain. Typically, the bleeding occurs internally and into the joints. It creates permanent damage, disfigurement and can even lead to death.

People with bleeding disorders need access to a Hemophilia Treatment Center (HTC). HTCs provide a comprehensive, team-based approach to care for people with bleeding and clotting disorders. Members of the care team at HTCs include: hematologists, pediatricians, nurses, social workers, physical therapists, orthopedists and dentists, with specialized training. The Centers for Disease Control and Prevention (CDC) reports that the care provided in HTCs significantly improves prevention of complications for persons with hemophilia. Mortality rates are 40 percent lower in people who use HTCs than in those who do not, despite the fact that the more severely affected patients are typically seen in HTCs. Receiving treatment at an HTC helps reduce costs for insurance providers. A study showed patients with bleeding disorders utilizing care provided by an HTC had a decreased hospitalization rate of >60% compared to patients receiving care outside of the HTC.

HB 5212 also addresses the need to have access to the full range of clotting factor therapies. Access to the full range of products is important because there are no generic clotting factor therapies to treat hemophilia. There are no therapeutic equivalent products. People respond differently to different products. If we were to be limited to one product, there would be a percentage of people who wouldn't respond to that one product and would end up having many more bleeding episodes. This would then require more product increasing the cost.

Safety is another concern for the bleeding disorders community. When I was 9 years old. I remember I was watching the Simpsons on TV. My dad walked in the room along with my other mother. My father picked up the remote and turned off the TV. He then looked down at me and said "Mark, you're HIV positive." I had no idea what that meant or how I could have possibly contracted the virus. All I knew was that I was going to die. The doctor told my parents I had less than a year to live. I consider it a miracle I'm still alive and every single day I am here on Earth is a blessing.

Unfortunately many people with hemophilia, including many of my friends, were not as lucky. Over 90% of severe hemophiliacs contracted HIV by taking their life saving medication to treat their hemophilia, and most died. Many wives and children also died. In addition to HIV, most severe hemophiliacs also contracted Hepatitis C. It's a period that we learned from and should never ever happen again. And if it does happen again, it won't be because we didn't think it could happen. Because we know it can.

There are 2 types of factor medication. There is plasma-derived product that is still made from human blood and there is recombinant products that are synthetic. Plasma derived products are cheaper than recombinant products. Over the years, our community has seen so many changes and limitations being created by our insurance provider, it is a very big and valid worry that we will see limited formularies that require us to use the cheapest products, plasma-derived. Nobody planned on HIV getting into our blood supply, but it did. And while plasma derived products are considered safe right now, they can not promise us safety from the unknown virus. And actually, just recently, a man in the UK, who was on a plasma derived product died from vCJD (the human strand of mad cow). Now its not known if it definitely came from the factor, but the fact is, they can't rule it out either, because there is a good chance it did.

Would any of you want your child to take that risk of using a product that doesn't guarantee safety, just so that your insurance provider can save a little cash? When there is another great option that *will* guarantee safety?

Also, in addition to that point, the decision should really be left to the treating physician anyways...not the insurance provider.

Lastly, I would like to address Section 4 of the bill that addresses entities dispensing clotting medications, or homecare pharmacies. I believe think we could add more requirements to this section.

The factor therapy for hemophilia is infused intravenously at home. In the past, people with hemophilia would have to go the emergency room each time they needed to treat a bleed. Now, people have learned how to infuse themselves at home. This allows patients to treat their bleeds in a much more timely matter before the bleed causes permanent damage.

Our community has benefited tremendously from these homecare companies. They are companies that specialize and provide services to people with bleeding disorders. They provide the medicine, nursing, ancillaries, social services, reimbursement support and much more. There is also a cost savings by preventing complications when promoting compliance. I didn't have access to a homecare company growing up. As a result, my joints were damaged before I was even a teenager, because no one was following up with my family to make sure I was remaining compliant. As a result I can no longer straighten either arm and my shoulders are extremely limited in their range of motion as well. I will probably need joint replacements which will be extremely expensive. Not to mention the amount of pain I have as a result of the damage from the bleeding.

They created a model that is personalized, unique, supportive, and knowledgeable. Unfortunately, many insurance providers have been bringing this service in-house and mandating people use a specific homecare or PMB or drop ship pharmacy who usually know little to nothing about hemophilia. This was a model that should have been examined and duplicated, not destroyed. You can see huge improvements in people's health and even mental well-being due to these companies.

We know that we would never get the insurance providers to let us use whatever company we want, so we are just pulling out a few very key elements of the service homecare companies provide and saying, we understand if you won't let us choose the company that best meets our needs, but if you are going to require us to use a specific homecare pharmacy, that company needs to be able to do at least these critical responsibilities.

These responsibilities are just a few items listed in the National Hemophilia Foundation's Medical and Scientific Advisory Council's, which is made up of hematologists and other professionals who treat people with bleeding disorders, recommendations (recommendation 188) for homecare pharmacies who dispense blood clotting medications.

I can't imagine what my parents had to go through and the pain they must have felt. Sadly, parents are still feeling that pain. They feel that pain when they are told their child will never live a normal life. They feel that pain when they have to put a helmet on their child, even when he is just watching TV on the couch. They feel that pain, when they have to watch a nurse stick a needle into their baby's vein in their head. And they feel that pain when they are handed the needle and told it's their turn to stick the needle into their own child.

Unfortunately these pains still exist today. But fortunately, there are many *other* things that HAVE improved over the years, but are now at risk due to changes in our insurance industry. These advancements over the past 20 to 40 years include Hemophilia Treatment Centers, Specialty Homecare Pharmacies and advancements in medicine.

This is Connecticut's opportunity to be a leader in our country. We can send the message that our health and well-being is important and a true difference can be made without high costs and politics. It is up to you. Our health is in your hands now.

Let's not go backwards. We are not asking for anything more than what we have already. Everything in this bill has been proven to be valuable, effective and beneficial to our health. This bill is just making sure we get to keep these necessities and no one will gamble with our health at stake.

Positive clinical and financial outcomes for patients with bleeding disorders receiving care at Missouri's hemophilia treatment centers

Jay Bryant-Wimp¹; D.J. Johnson¹; Roxanna Halderman Halderman¹; Sarah Cooper¹; ¹MO HealthNet, Jefferson City, MO

Objectives: There are a limited number of studies documenting hospitalization rates for patients with bleeding disorders. One study revealed 40% higher hospitalization rate for patients not seen at a Hemophilia Treatment Center (HTC)¹. Another four-year study utilizing 808 patients enrolled in HTCs provides hospitalization rates of 21.2 admissions per 100 patient years (PYs)².

A state Medicaid agency will demonstrate decreased hospitalization rates for patients with bleeding disorders receiving care administered by healthcare professionals at federally recognized HTCs versus patients receiving care elsewhere.

Methods: Data from January 1st, 2007 through December 31st, 2008 on all active bleeding disorder (n>170) patients with paid claims was utilized for this study. Utilization of patient years (PYs) allowed for inclusion of patients that were on service at the beginning of the study period and for patients that came on service after January 1st, 2007.

Summary: The hospitalization rate for the combined bleeding disorder patient population was reported as 24.42 admissions per 100 PYs. Further investigation revealed the hospitalization rate for patients under the care of the comprehensive team at HTCs was 18.98 admissions per 100 PYs, while the patients cared for by clinicians not affiliated with an HTC had a hospitalization rate of 48.53 admissions per 100 PYs. The cost per admit for hemophilia related hospitalizations in this entire study group (n>170) was > \$105,000 per admission.

Conclusion: The continuity of care provided by the staff at the HTC with patients being treated by a multi-disciplinary team contributes to a remarkable decrease in hospitalization rates for patients with bleeding disorders. Patients with bleeding disorders utilizing care provided by an HTC had a decreased hospitalization rate of >60% compared to patients receiving care outside of the HTC. Extrapolating the average hospitalization cost for this study group and the difference in hospitalization rates, utilization of the HTC for care demonstrated a cost-savings of >\$3,200,000 versus utilization of non-HTCs per 100 PYs.

1. Soucie JM, Symonds J 4th, Evatt B, Brettler D, Huszti H, Linden J; "Home-based factor infusion complications among males with haemophilia." *Haemophilia* 2001 Mar; 7(2) 198-206.
2. Soucie JM, Symons J 4th, Evatt B, Brettler D, Huszti H, Linden J; Hemophilia Surveillance System Project Investigators. Home-based factor infusion therapy and hospitalization for bleeding complications among males with hemophilia. *Hemophilia* 2001; 7:198-20

Hemophilia Treatment Centers Comprehensive Care for People with Bleeding and Clotting Disorders

A national network of 140 Hemophilia Treatment Centers (HTCs) was created by Congress in 1974 to ensure access to comprehensive, specialized care for people with bleeding disorders. These primarily hospital-based programs receive federal funding from the Health Resources and Services Administration (HRSA) and the Centers for Disease Control and Prevention (CDC) to support nursing, rehabilitative and social services as well as prevention, disease management, and blood safety surveillance activities. The HTC role has expanded dramatically over the last three decades, evolving with the needs of patients with bleeding and clotting disorders. Maintaining funding for these programs is critical to the health and well being of people with bleeding and clotting disorders.

Facts about Hemophilia Treatment Centers

- HTCs provide comprehensive, team-based approach to care for people with bleeding and clotting disorders. Members of the care team at HTCs include: hematologists, pediatricians, nurses, social workers, physical therapists, orthopedists and dentists, all with specialized training.
- The CDC reports that the care provided in HTCs significantly improves prevention of complications for persons with hemophilia. Mortality rates are 40 percent lower in persons who use HTCs than in those who do not, despite the fact that the more severely affected patients are typically seen in HTCs.
- HTCs now are treating women with bleeding disorders, such as von Willebrand's Disease and people with clotting disorders such as thrombosis and thrombophilia. These patients are increasingly referred to HTCs for diagnosis and specialized treatment.

Facts about the Patients Seen in Hemophilia Treatment Centers

- Hemophilia is a rare chronic bleeding disorder affecting about 20,000 people in the United States, most of whom are male. People with hemophilia require life-long treatment with high-cost clotting factor medications and other forms of specialized care.
- In the 1980s, nearly 90% of Americans with severe hemophilia became infected with AIDS from contaminated blood and blood products. More than 50% of those individuals have since passed away. In addition, roughly 44% of all people with hemophilia were infected by the hepatitis C virus during that time.
- Women with von Willebrand's Disease (VWD) are the largest growing population of patients seen in HTCs. VWD is another genetic bleeding disorder that prevents the blood from clotting normally. Although many individuals with VWD remain undiagnosed, it may affect as many as two million people in the U.S.
- Clotting disorders, when the blood clots excessively, can result from inherited disorders or may develop as a complication of other conditions, such as prolonged immobilization or diseases. More than 600,000 Americans are affected by abnormal blood clots, and have the potential to develop dangerous clots, known as deep vein thromboses (DVTs). If left undiagnosed or untreated, these events can be life-threatening.



NATIONAL HEMOPHILIA FOUNDATION

for all bleeding and clotting disorders

MASAC Document #188

(Replaces #181)

MASAC RECOMMENDATIONS REGARDING STANDARDS OF SERVICE FOR PHARMACY PROVIDERS OF CLOTTING FACTOR CONCENTRATES FOR HOME USE TO PATIENTS WITH BLEEDING DISORDERS

*The following recommendation was approved by the Medical and Scientific
Advisory Council (MASAC) on November 15, 2008, and adopted by the NHF
Board of Directors on November 16, 2008.*

Bleeding disorders such as hemophilia are chronic disorders characterized by bleeding episodes that may occur spontaneously or after mild to severe trauma. The timing and severity of bleeding episodes are unpredictable, even for patients on regularly scheduled treatment; thus providers of clotting factor concentrates must be able to effectively respond to varying frequency and dosing needs.

There are a number of pharmacy providers who supply clotting factor concentrates to patients with bleeding disorders treated at home. When patients do not receive optimal service from these providers, there is potential for adverse health events that lead to poor outcomes and/or increased costs.

MASAC acknowledges the necessity of cost efficiency in the provision of health care, yet cost efficiency should not occur at the expense of quality patient care. The purpose of this document is to establish minimum standards of service for pharmacy providers to meet the specific needs of individuals with bleeding disorders.

This material is provided for your general information only. NHF does not give medical advice or engage in the practice of medicine. NHF under no circumstances recommends particular treatment for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.

Copyright 2008 National Hemophilia Foundation. To facilitate the dissemination of these medical recommendations, reproduction of any material in this publication in whole or in part will be permitted provided: 1) a specific reference to the MASAC recommendation number and title is included and 2) the reproduction is not intended for use in connection with the marketing, sale or promotion of any product or service. NHF reserves the right to make the final determination of compliance with this policy. For questions or to obtain a copy of the most recent recommendations, please contact the NHF Director of Communications at 1-800-42-HANDI or visit the NHF website at www.hemophilia.org.

116 West 32nd Street • 11th Floor
New York, NY 10001

(800) 42-HANDI • (212) 328-3700 • fax (212) 328-3777
www.hemophilia.org • info@hemophilia.org

**STANDARDS OF SERVICE FOR PHARMACY PROVIDERS OF
CLOTTING FACTOR CONCENTRATES FOR HOME USE TO PATIENTS WITH
BLEEDING DISORDERS**

Patients with bleeding disorders require clotting factor concentrates for prevention and treatment of bleeding episodes. It is essential that any pharmacy provider dispensing clotting factor concentrates for home use provide services that meet the minimal standards delineated below.

A. Pharmacy Provider Staff Knowledge of Clotting Factor Concentrates and Ancillary Supplies

1. Pharmacy provider staff shall have sufficient knowledge and understanding of bleeding disorders to accurately follow the instructions of the prescribing physician and assure high quality service for the patient.
2. Pharmacy provider staff shall be experienced with filling and handling prescriptions for the full range of clotting factor concentrates.
3. Pharmacy provider staff shall be knowledgeable about necessary ancillary supplies.
4. Pharmacy provider staff shall be knowledgeable about containers for the disposal of hazardous waste.
5. Pharmacy provider staff shall direct patients to contact their established treating physicians for all medical and therapeutic questions.
6. Pharmacy provider staff shall direct staff medical questions and concerns to the treating physician.

B. Clotting Factor Concentrates and Ancillaries

1. Pharmacy providers shall be able to provide the full range of available concentrates, including all available assays and vial sizes.
2. Pharmacy providers shall be able to provide all necessary ancillary supplies for administration of clotting factor concentrates. Examples of ancillary supplies include, but are not limited to: needles; syringes; gauze; anesthetic creams; sterile field pads; sterile gloves.
3. Pharmacy providers shall provide containers for the disposal of hazardous waste, and the collection of such containers shall be arranged pursuant to state and federal law.
4. Some consumers of clotting factor concentrates require additional services, such as nursing services. If the pharmacy providers do not offer these services directly, they shall coordinate with the nursing agencies to ensure that all of the patient's needs are adequately met.

C. Processing of Prescription Orders

1. Pharmacy provider staff shall work with prescribing physicians to ensure that prescription orders are filled within 48 hours.
2. Prescriptions of clotting factor concentrates shall be dispensed as written by the prescribing physician. No changes or substitutions shall be made unless approved by the physician.
3. If the prescription does not indicate a specific brand name of product, the pharmacist shall ask the prescribing physician which product should be dispensed.
4. Filling of all prescription orders shall be within plus or minus 5-10 % of prescribed assays, barring extenuating circumstances. This standard shall not be violated by dispensing a number of vials so excessive that it would compromise compliance or so low a dose that it would compromise medical outcome.
5. Clotting factor concentrates shall have acceptable outdates based on diagnosis and frequency of treatment. Short-dated product (outdate within 6 months) shall only be dispensed after consultation with the prescribing physician.

6. Pharmacy provider staff shall supply any ancillary supplies required by the patients and prescribed by their physicians.

D. Hours of Operation / Access to Staff

1. Pharmacy providers shall be open, at a minimum, Monday through Friday, excluding holidays, during regular business hours (9:00 am to 5:00 pm) in their service area time zones. If a pharmacy serves all 48 contiguous states, it will need to be open from 9:00 am until 8:00 pm Eastern Time, Monday through Friday, not including holidays.
2. Pharmacy staff shall provide 24-hour emergency access including multilingual interpreters in case of emergency.
3. If the pharmacy receives a call about an emergency situation, the treating physician shall be notified immediately. Pharmacy provider in consultation with the treating physician shall have plan in place to ensure that, in case of emergent need, patient shall have access to factor concentrate within 12 hours of expressed need, with a goal of 3 hours where logistically possible.

E. Delivery

1. Routine orders from established patients shall be correctly filled and delivered within 48 hours from the time the order is placed.
2. If the pharmacy receives a call about an emergency situation, the treating physician shall be notified immediately. Pharmacy provider in consultation with the treating physician shall have plan in place to ensure that, in case of emergent need, patient shall have access to factor concentrate within 12 hours of expressed need, with a goal of 3 hours where logistically possible.
3. Pharmacy providers shall have a plan in place to meet delivery requirements in the event of a natural disaster.
4. Product shall be delivered to the location requested by the patient that has been determined by the pharmacy provider to be appropriate and safe.
5. Shipping of all clotting factor concentrates shall meet all federally mandated standards, including those for temperature control.
6. Pharmacy providers shall adhere to all HIPAA confidentiality guidelines.
7. Pharmacy providers shall have an emergency contact number for customers to report problems with deliveries.

F. Recordkeeping, Billing and Product Recall

1. Pharmacy providers shall have an accurate record-keeping system that meets state and federal requirements. In addition, pharmacy providers shall have treatment prescription information available for patients and prescribing physicians.
2. Pharmacy providers shall explain patient copay, deductible and coinsurance payment responsibilities, and lifetime cap limits clearly at the time the first order is placed and annually when updating insurance information, or sooner if there has been a change in insurance.
3. Pharmacy providers shall provide a statement of factor cost per unit dispensed to the consumer.
4. Pharmacy providers must be able to trace the path any bottle of clotting factor concentrate has taken and the way it has been handled from the time it left the manufacturer until the time it is delivered to the consumer.
5. Pharmacy providers shall participate in the National Patient Notification System for clotting factor concentrate recalls.

GLOSSARY

EMERGENCY: a situation in which the patient's condition requires immediate medical attention and/or treatment.

HOME USE: use of clotting factor concentrate in the home or another outpatient setting.

PHARMACY PROVIDER: an entity that dispenses clotting factor concentrates to patients for home use.