In this issue

THE HEMOPHILIA HEALTH CARE ACT HB 1705

Pennsylvania Chapters Move to Protect Patients with Bleeding Disorders in Pennsylvania

As patients with bleeding disorders in Pennsylvania, we are very concerned about changing insurance practices that affect our quality of life. These changes seek to undermine the very model of care that has been in effect for decades in the Commonwealth....a model of care recognized both at the state and national levels.

In response to mounting concerns from local patients with bleeding disorders and our own program staff about maintaining access to programs, factor replacement therapies and supportive services, the Delaware Valley Chapter and the Western Pennsylvania Chapter of the National Hemophilia Foundation have been working together for many, many months now. And wow, have we been busy and so have you....in supporting this joint effort!!

We have written and submitted legislation that will provide assurance to patients in Pennsylvania that their quality of life will be maintained, as well as their access to what they really need.

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The Winning Spirit

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National Hemophilia Foundation
DELAWARE VALLEY CHAPTER
222 S. Easton Road, Suite 122
Glenside, PA 19038
Phone (215) 885-6500
fax (215) 885-6074
e-mail: hemophilia@navpoint.com

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From the Executive Director

With this issue, we are enclosing a copy of The Annual Family Camp registration materials (if you are receiving your newsletter through your treatment center). If you don't receive a registration form, but would like to attend, just give us a call (215-885-6500).

This year, the Annual Family Camp Weekend will be held from Friday, September 16th to Sunday, September 18th at the Variety Club Campsite just outside of Plymouth Meeting, Pennsylvania. This is a great time to enjoy many activities with other families who share the same challenges as you!

SPECIAL HIGHLIGHTS FOR THIS YEAR'S CAMP INCLUDE.....

PLAYERS FROM THE VILLANOVA WILDCATS WILL BE WITH US ON SATURDAY AS PART OF "GETTIN' IN THE GAME!!"

AS WELL AS

MISTY BRUNNMEIER, A YOUNG WOMAN WITH VON WILLIBRAND DISEASE AND A FIRST DEGREE BLACK BELT AND NATIONAL CHAMPION IN TAE KWON DO!!

Two Saturday Morning Sessions of Interest:

- "Insurance Advocacy: What you Can Do To Become Your Own Best Advocate"
- "von Willebrand Disease: Your Questions Answered"

Please send in your registration right away. Space is limited. You will be able to just come for the Saturday programs, if that is best for your schedule....Otherwise, plan to stay for the weekend....great food, lots of fun and so much more!

We Gratefully Acknowledge...the pledge of support from the following manufacturers for 2005.

American Red Cross
Monarc-M

Baxter Bioscience
Recombinate
Hemofil M
Proplex T
Feiba VH
Bebulin VH
Albumin (Human)
Advate

Bayer Corporation
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Autoplex T
WinRho

Novo Nordisk
NovoSevenTM

ZLB Behring
Monoclate-P
Mononine
Helixate FS
Bioclate
Humate-P
Stimate
Gammar-P I.V.

Calendar

September
16 Family Camp!
19 DVC Golf Classic!

October
21 Oktoberfest!
27 NHF meeting/ San Diego!

December
10 Holiday Party!

On **April 13th**, more than 150 volunteers made legislative visits in Harrisburg as part of **"Hemophilia Awareness Day,"** regarding problems in Pennsylvania for patients with bleeding disorders! Many thanks to everyone who participated. Your dedication led to the introduction of HB 1705!! We couldn't have done it without you!!

The **Family Dinner**, held on Tuesday, **May 17th** at the Hilton Hotel, City Avenue, was just the best ever. **Representative Roy Baldwin** (sponsor of HB 1705), was joined by **Representative Sue Cornell** (Hatboro) and **Representative Chris Ross** (West Chester), both co-sponsors of HB 1705!! They heard our messages and have responded, leading the way in the House of Representatives! Dawn Rotellini, executive director of the Western PA Chapter (NHF) in Pittsburgh was our guest and Dr. Catherine Manno (medical director/CHOP Hemophilia Program) gave a great update on the status of gene therapy for hemophilia. She is always a great presenter!! Thank you Dr. Manno!!

On **June 6, 2005**, we were out "on the links" at Edgemont Country Club for **Carlino's Golf Outing**. Kathy and John DiMichele and Laura and Pat Carlino and the wonderful employees at Carlino's Specialty Foods in Ardmore, PA made this event a real success!! It was a beautiful day in every way. We all had fun and we raised thousands of dollars in support of the DVCs efforts!

September 16, 17 and 18, 2005. Annual Family Camp. Plan to spend the weekend with us and get the opportunity to meet other families who share the same challenges! Totally fun and educational and just outside Philadelphia in Worcester, PA! Don't miss it!! **THE VILLANOVA WILDCATS WILL BE WITH US FOR THE SATURDAY PROGRAM!!!! WOW!!**

On Monday, **September 19, 2005**, plan to join all of us for the **Annual DVC Golf Classic!** Mark Marra (Golf Chairman) always does a great job of working out all of the details for this wonderful day. Again this year, it will be held at the Meadowlands Country Club in Blue Bell, Pennsylvania. This event has raised over $1 million to support the Delaware Valley Chapter! Can you believe it? Mark your calendar now and plan to be with us!!

"Oktoberfest" will be held at the Hilton Hotel on City Avenue **(a NEW LOCATION!)** on Friday, **October 21, 2005,** sponsored by Wyeth! What a great night of food, music, gaming events and a terrific live auction! For an invitation, call the office (215-885-6500)!! Help support Bob Romano, Megan McEnroe and their great committee by planning to attend this year!!

The **57th NHF Annual Meeting** will be held on **October 27, 28 and 29, 2005 in San Diego, California.** This is the only meeting in the United States completely focused on bleeding disorders. If you have never attended the NHF Annual Meeting but would like to attend this fall, call the DVC office to see if we can help with some of the expenses (215-885-6500). We sponsor more patients to this annual meeting than any other chapter in the country. Let's just say it's "part of our mission!"

DCV legislative visits in Harrisburg as part of "Hemophilia Awareness Day"

Family Dinner 2005: Shirley Serrill (standing) with Representative Sue Cornell (seated R).

Dr. Manno at the Family Dinner, with her "sidekick, Dakota!"

Ann Rogers with Dawn Rotellini (Executive Director, Pittsburgh Chapter)

Representative Chris Ross at the Family Dinner

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HB 1705 Pennsylvania Chapters Move to Protect

We want to sincerely thank Representative Roy Baldwin from central Pennsylvania for understanding what is going on and stepping forward to sponsor House Bill 1705 "The Hemophilia Health Care Act." So here are the issues addressed in the legislation. Please send us your comments by e-mail (hemophilia@navpoint.net) and we will pass them on to Representative Baldwin.

Issue #1

Access to State-Recognized Hemophilia Programs in Pennsylvania

PROBLEM

Some insurance companies, outside of Pennsylvania, have restricted access to hemophilia treatment centers by requiring individuals with hemophilia and von Willebrand Disease to be seen by "approved" hematologists, rather than paying for the services provided at a state recognized hemophilia program.

BACKGROUND

- The Hemophilia Treatment Center (HTC) network was created by Congress in 1974 to ensure access to comprehensive, specialized care for individuals with bleeding disorders.
- The Centers for Disease Control and Prevention (CDC) has been studying the outcomes for patients followed by the 141 hemophilia treatment centers (HTCs) in the United States for 30 years. Studies have demonstrated a 40% reduction in mortality (death) and morbidity (complications) associated with care provided by an HTC.
- More than 75% of the individuals with hemophilia in Pennsylvania receive their care from a state-recognized hemophilia program.
- The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the treating standard for 141 hemophilia programs in the United States.
- MASAC recommends that patients with hemophilia and von Willebrand Disease be seen regularly at a hemophilia treatment center.

OUR POSITION

Individuals with hemophilia in Pennsylvania need unrestricted access to the 8 state-recognized hemophilia programs, whether or not those physicians/programs are on an insurance company's approved provider list.

Issue #2

Access to All Blood Clotting Factor Therapies for the Treatment of Hemophilia

PROBLEM

Some insurance companies and their pharmacy benefit managers (PBMs) in Pennsylvania, including some managed care organizations servicing Medicaid beneficiaries, have tried to limit patient access to the full range of medically necessary blood clotting factor therapies. Some insurers and PBMs have attempted to require that patients utilize a single, "preferred" blood clotting factor for the treatment of hemophilia.

BACKGROUND

- In 2004, an insurance company in Pennsylvania solicited physicians from the hemophilia programs in Pennsylvania to promote "step therapy" using a single, "preferred" product, for the treatment of Hemophilia A.
- The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the treating standard for 141 hemophilia programs in the United States.
- MASAC opposes designating any therapy as a "preferred product" and opposes requiring that beneficiaries "fail first" and utilize "step therapy" for the treatment of hemophilia.
- There are currently only 4 recombinant factor therapies (products not derived from human plasma) available for the treatment of hemophilia A. Three of the products use human or animal albumin as a stabilizer in the product. One product contains no human or animal albumin. Some products are formulated with sucrose and may not be appropriate for individuals with sensitivities to sucrose. Different patients respond differently to the same therapy.
- Each product is different. Life-saving blood clotting factor therapies are not therapeutically equivalent. There are currently no generic products available for the treatment of hemophilia.
- The decision about which product is best for the patient is a medical decision and should be made by the

physician and the patient, not the insurance company, pharmacy benefit manager or other payer.

OUR POSITION

As patients with a chronic, life-threatening condition, we need access to the full range of medically-necessary blood clotting factor therapies without restriction from insurance companies, pharmacy benefit mangers or other fiscal agents. Insurance companies and pharmacy benefit managers should be prohibited from requiring that beneficiaries utilize a "preferred product" and that individuals "fail first" before obtaining access to the product that the patient and physician have determined is most appropriate.

Life-sustaining factor products are required to control bleeding. As a matter of public policy, it is crucial that individuals with hemophilia have access to all factor replacement therapies needed to control bleeding. Without blood clotting factor therapies, individuals with hemophilia can bleed into joints and tissues, which can cause extremely painful, and permanent, joint, ligament, and bone damage. As a community, we object to an insurance company, pharmacy benefit manager or other fiscal agent determining which medicine is best to treat our condition!

Issue #3

Access to Hospital Coagulation Laboratories

PROBLEM

Some insurance companies in Pennsylvania are directing individuals with hemophilia and von Willebrand Disease to "approved laboratories" for highly specialized tests that should be performed in a coagulation laboratory.

BACKGROUND

- Coagulation studies are highly specialized tests. Extremely accurate results are required for optimal patient care.
- Results of these laboratory tests are often medically necessary immediately or sooner than the normal turn-around-time for the carrier's participating clinical laboratory.
- Accurate test results are determined by closely supervised procedures in venipuncture and laboratory techniques in a controlled environment that are often not achieved by the carrier's participating clinical laboratory.
- Some insurance companies and other payers are directing individuals with hemophilia and von Willebrand Disease to "approved laboratories" that are unfamiliar with the specific nature of bleeding disorders and coagulation studies.
- The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the treating standard for 141 hemophilia programs in the United States.

OUR POSITION

As patients, we need unrestricted access to the coagulation laboratories connected with the state-recognized hemophilia programs in Pennsylvania, whether or not those laboratories are on an insurance company's approved provider list.

Issue #4

Access to Full-Service Pharmacy Providers and Home Supportive Services

PROBLEM

Some insurance companies in Pennsylvania are requiring that individuals with hemophilia utilize mail order pharmacies that have little, if any, experience with treating hemophilia, instead of allowing access to full service pharmacies that have the ability to provide high-quality home supportive services.

BACKGROUND

- Blood clotting factor products require careful handling and reconstitution prior to intravenous infusion.
- Optimally, blood clotting factor products are self-administered at home, preserving the patient's "quality of life" and reducing the costs associated with factor administration at the hospital.
- Home self-infusion is taught to children and adolescents with hemophilia and their families by their program treatment team and qualified home care nurses over a period of time. Some patients and families are unable to master venipuncture and factor infusion and therefore require access to trained homecare nurses who can come to their home to infuse factor.
- In 2004, insurance companies, pharmacy benefit managers and fiscal agents in Pennsylvania began to move patients from their full service home care pharmacies to mail order pharmacies that do not offer skilled, home supportive services.

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HB 1705

- The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the treating standard for 141 hemophilia programs in the United States.
- MASAC opposes "sole provider contracts" that limit the pharmacy options for an individual with hemophilia. MASAC also recognizes the value of home, supportive services for patients, particularly in the early years.
- Without full service providers for those patients who need skilled home care, individuals with hemophilia must miss work or school, and travel to the hemophilia treatment program at a hospital for administration of their factor products. This type of medical management is below the current standard of care.
- Loss of full service providers is also costly. Lack of adequate support will result in time lost from work or school, the ability to maintain employment and an increase in development of hemophilia related complications for many patients.

OUR POSITION

Individuals with bleeding disorders and their treatment program staff should have the ability to choose the pharmacy provider that best meets their needs and be assured that the provider is knowledgeable and experienced in the treatment of their condition. The type of home supportive services that a patient needs is a medical decision and is best made by the physician and patient, not an insurance company. Individuals with bleeding disorders need options in pharmacy and home supportive services. Retaining competition between providers helps to ensure that patients are receiving the absolute highest level of care and service possible.

Issue #5

30,000 Women with von Willebrand Disease Have Unnecessary Hysterectomies Each Year in the U.S.

PROBLEM

Current medical studies suggest that hysterectomies and other invasive uterine procedures may not be necessary for some women with menorrhagia (excessive menstrual bleeding), particularly women with bleeding disorders such as von Willebrand Disease. The National Hemophilia Foundation estimates that approximately 30,000 women annually have hysterectomies due to undiagnosed von Willebrand Disease. A hysterectomy for someone with von Willebrand Disease may not be necessary and it does not address the cause of the excessive bleeding.

BACKGROUND

- von Willebrand Disease (VWD) is a bleeding disorder that results from a missing factor protein. It is prevalent in both men and women, although the symptoms are more apparent in women. The CDC estimates 1-2% of the U.S. population may have a form of Von Willebrand Disease.
- In December 2001, The American College of Obstetricians and Gynecologists issued recommendation 263, "von Willebrand Disease in Gynecologic Practice." This recommendation calls upon OB/GYN's to consider bleeding disorders such as Von Willebrand Disease prior to a hysterectomy.
- A hysterectomy only addresses the symptom of menorrhagia and not the underlying cause of bleeding in patients with von Willebrand Disease. The condition can be treated with intravenously infused blood clotting factor, in severe cases, and with desmopressin acetate intravenously or as a nasal spray in mild to moderate cases.
- The Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation (NHF) sets the standard for treating von Willebrand Disease.
- The United States Congress approved congressional appropriations report language in 2003 calling upon the National Heart Lung and Blood Institute to create a pilot screening program for von Willebrand Disease among OB/GYN's treating women.
- Project Red Flag, an initiative of the National Hemophilia Foundation, supported by funding from the Centers for Disease Control and Prevention, is educating OB/GYN's about von Willebrand Disease. At present, the condition is still under-recognized and under-diagnosed.

OUR POSITION

Women should not be subjected to hysterectomies or other invasive uterine procedures in cases where it is not needed. Menorrhagia, resulting from von Willebrand Disease, does not require a hysterectomy, as the condition can be treated with medical therapies. A screening requirement for von Willebrand Disease, in cases where a hysterectomy is suggested due to menorrhagia, will prevent needless invasive surgeries and spare women from the trauma associated with such a procedure. Women with von Willebrand Disease or other bleeding disorders should participate in comprehensive care at the eight state-recognized hemophilia programs. Coordination of care to meet the special needs of these patients can be achieved more completely. Women who are diagnosed with von Willebrand Disease can be monitored and treated by the eight state-recognized hemophilia programs in Pennsylvania.

Point. . . Counter Point

A Dad's Perspective

By John Stephen Nicolosi

Following is a letter sent to the DVC from "another dad"… We invite all comments about current issues for our community. Keep writing to us!!

Being the father of a son with hemophilia, I can understand the anxiety of "Just a Dad" in the winter issue of The Winning Spirit. It is easy to feel all alone when everyone outside of your circle of influence doesn't seem to understand or care. I view things a little different.

I thank the manufacturers of the factor my son takes for concentrating on hemophilia and developing these great new medicines to help my son.

I thank my hemophilia treatment center (HTC) for being there when I had all those medical emergencies at all hours. I appreciate their patience in training my wife, son and myself to infuse. I thank the social worker at the HTC for helping me look for alternative insurance options and opening the door for my son to meet other children with hemophilia.

Yes, I also thank my insurance company for paying for the factor my son takes that costs eight times my annual salary every year and I understand how that affects their bottom line. I won't accept anything less than their full support for my son.

Most of all I thank the early generations of people with hemophilia that sacrificed their lives and quality of life to inspire the pharmaceutical companies to create those medicines.

I thank the members of the National Hemophilia Foundation (NHF) who support efforts to change insurance companies and effect laws on our behalf. The role the NHF plays cannot be overstated.

I thank the home healthcare companies and I count them as part of my team because they are my drug store and are proficient and friendly. I appreciate the moneys they contribute to the community, but know it is from the profits made on us.

Things are changing and change is not easy. I understand in most, if not all cases, insurance companies tell the HTCs what they will pay as they do with homecare companies and local drug stores. This is one area where the NHF focuses their energy and I commend and admire the dedication, knowledge and drive of these professional people of the NHF.

We have come to expect this level of dedication from the NHF as we have come to expect the best care possible from our treatment center and their staff. Neither has let me down.

I would not expect the staff at the treatment center to propose a bill for my son's rights in Washington and I wouldn't expect those people who fight for his rights in Washington to infuse my son. I do expect them to support each other and I feel this is the case. Letters like the one from "Just a Dad" are not helpful in this regard.

There are many players on my son's team, each with a purpose and a job to do. Understanding the players and their area of expertise is the job of the team manager, ME! There is much work ahead and I refuse to drive my team apart. I will be the catalyst to make them stronger. Call me with your comments (570-954-2590) or send your comments by email: jnicolosi3@epix.net

Wyeth Offers Refacto Trial Prescription Program

Wyeth is offering a Refacto Trial Prescription Program to all Hemophilia Treatment Centers. A patient can receive a one-time shipment of up to 20,000 IU of Refacto at no charge. Ask your physician about this free trial.

Bayer Announces Clinical Trials for Longer-Acting Kogenate

Bayer HealthCare, LLC Biological Products Division, has announced it will begin Phase I clinical trials of its longer-acting Kogenate product. This product is intended to have a longer time of activity in the body, which could result in weekly, or even less frequent, infusion in prophylaxis.

The Philadelphia and Bleeding Disorders Story

By Richard Atwood, MPH, Region IV South Coordinator

Philadelphia is where the debate on whether women could have hemophilia began.

In 1803, John Conrad Otto, MD, of Philadelphia reported that only males were affected with a "hemorrhagic disposition" while females were exempt from it, but were capable of transmitting this disposition to their male children.

This American medical article prompted the reporting of additional cases. The opinion that only males suffered from hemorrhagic tendency was substantiated by nine other American articles, plus additional European articles, until Thomas Smethurst, surgeon of Ramsgate, England, reported in 1841 on two female cases subject to hemorrhagic diathesis, including a Mrs. C. who was born in 1790. This English article prompted the reporting of additional female cases.

One characteristic of medical journal articles during the 1800s was that new cases of hemophilia were added to existing cases, so that an international prevalence was known. Thus Lange reported in 1845 on 260 cases of hemophilia, of whom 31 were female. Grandidier reported in 1872 on 631 cases of hemophilia, of whom 48 were female. In 1883, Dunn reported on 780 cases of hemophilia, of whom 63 were female. This summary comprised the total medical literature on hemophilia, yet there were still some questions on the validity of the diagnosis of hemophilia in the female.

The first description of true hemophilia in the female was reported by Sir Frederick Treves of London, England, in 1886. Florence Parker was the first reported female case in an extended family that laid claim to having many members with unusual bleeding episodes. The diagnosis of hemophilia A was confirmed in 1973 when factor VIII clotting activity was assayed at <1% in Ada B., one of Florence's sisters and in Frank J., one of their nephews. The medical community has been aware of documented cases of females with bleeding disorders for over 150 years.

Reference: Dunn TD 1883. Hemophilia. American Journal Medical Science. 85:68-83.

QUESTION: Could these women of the 1800s have had von Willebrand Disease?

Just a Thought

**We are what we repeatedly do.
Excellence, then, is not an act,
but a habit.**

Aristotle