Hemogram

Western Pennsylvania Chapter of the National Hemophilia Foundation

Spring 2013
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DESIGNATE UNITED WAY GIFTS TO THE CHAPTER!

If your company is participating in the United Way campaign, you may designate all or a portion of your gift to the Chapter.

WPCNHF Contributor Agency Code Number is: **83**

Washington Days 2013

The trip to Washington, D.C. was a huge success! With over 350 members and chapter representatives registered from around the country, it was a record breaking advocacy effort for the National Hemophilia Foundation!

Everyone gathered in DC Wednesday afternoon for some training and pointers from lobbying and advocacy experts. There was even an Advocacy 101 for first timers to put them at ease! In addition, there was some in depth background information presented on the two main issues we would be discussing with our elected officials – maintaining HTC funding and HR 460.

Our first goal was to seek officials' support in maintaining the federal hemophilia programs at the Maternal and Child Health Bureau (MCHB) and Centers for Disease Control and Prevention (CDC) to ensure access to hemophilia treatment centers (HTCs) and other critical education, research and surveillance activities. HTCs provide multi-disciplinary care furnished by hematologists, pediatricians, nurses, social workers, physical therapists, orthopedists and dentists among others, all with specialized training. CDC studies show that mortality and hospitalization rates are 40% lower in people who use HTCs compared with those who do not, despite the fact that more severely affected patients are more likely to be seen in HTCs. This is a critical issue to our population, particularly as we were in DC the day before the sequester took effect.

The second issue was rallying support for HR 460 in the House, and asking members of the Senate to consider introducing companion legislation to HR 460, the Patients' Access to Treatment Act, which will increase access to life-saving drugs on specialty tiers by prohibiting insurers from imposing exorbitant co-insurance requirements on patients. Most commercial health insurers charge fixed co-payments for different tiers of medications: generics (Tier I), name brands (Tier II), and off formulary, name brand medications (Tier III). For example, co-pays might be set at $10/$20/$50, respectively, for medication on the three tiers. Some commercial insurers have established a fourth or specialty tier that includes expensive biologics and other drugs requiring special administration.

Continued on page 15

Letter From The President, Scott Miller

The first few months of the year have been filled with renewal and conviction. Alison and Brittani have brought some great new ideas to the chapter for both programing and fundraising. I am thankful for the work of all of our staff: Alison, Brittani, Janet, and Bud. They work tirelessly to make this Chapter a better organization and to provide superior service to our members.

This year, you will see an effort to expand programing across our entire geographic territory. Since coming on the board, I have been passionate about serving the entire 26 counties of Western Pennsylvania and the staff and Board are supportive of this vision. Therefore, as you see new programs, please take advantage of them and show your support for our efforts. You will see an expansion in topics as well as geography. To that end, please feel free to contact Alison with any ideas you have for programs – what types of educational programs do you want to see us offer? We would love to hear from you with your thoughts and ideas of what we might do to make your lives better through education, advocacy, resource and referral.

I am looking forward to a wonderful year with new, exciting events. As always, feel free to contact the office with any questions or concerns you have. We are here to be a resource for you; therefore, you should never hesitate to call.

Sincerely,
Scott E. Miller, CPA, J.D., DBA
WPCNHF Board President

Letter From The Executive Director, Alison Yazer

Dear Chapter Members and Friends,

I hope this edition of Hemogram finds you and your family well. It has been an exciting few months here at the chapter as we've all been working hard to plan a great year of informative, educational programs and fundraisers. As of last count, we were at 18 events for the year thus far – and that number will continue to grow!

Speaking of growing, our chapter staff has expanded! We are now a staff of 4 as we welcome Brittani Reed as our Fundraising & Events Manager and Bud Krapp as our new Bookkeeper & Office Assistant. Many of you already know Brittani, since she interned with the Chapter and then worked as our Development Assistant through the end of 2012. She has assumed Madonna's role and has definitively hit the ground running! Bud joined us in December, which enabled Janet Barone to move into her new role as our Member Services Manager. Janet, in her new role, will be planning and executing educational programs, doing some advocacy work and administering the Patient Assistance Program.

In addition to our staff restructuring, our office has more structure now, too! Thanks to Terry Gee of Gee Construction, we now have some walls in our office making the space much more professional. I'd like to thank Cort Furniture on McKnight Road, Re/Max Realty in Zelienople and General Rental of Cranberry for helping us furnish our "new" digs. I invite each of you to come visit us and to see our space for yourself!

I hope to see you soon at the office or one of our upcoming events!

Alison Yazer
Executive Director

Constant Contact®

Are you receiving e-mail updates from the Chapter? Many of our members are on our e-mail distribution list and receive event invitations, reminders, and other notices through Constant Contact® e-mail. If you provided an e-mail address on your Membership Registration or Membership Survey form, you should be on this distribution list and should be receiving e-mail notifications. If your e-mail address has changed or if you did not return your registration or survey form, please send a note to info@westpennhemophilia.org. It's important that our members be fully registered with the Chapter in order receive all of the benefits of membership.

(Please know that although it's beneficial to be on our e-mail distribution list, it's not required. We realize that not all members have an e-mail address or access to a computer. Members will continue to receive our regular mailings through the U.S. Mail.)

A Friendly Reminder...

If you RSVP for an event, please make every effort to attend! We understand that occasional emergencies or unforeseen circumstances may make it difficult or impossible to attend an event, but every time you RSVP and don't come to an event, the chapter spends money that could be put toward other educational events or patient assistance. We want to be able to continue providing quality programming and events to our members, and we have to do this on a very limited budget. Let's all do everything we can to ensure that our money is spent wisely! Thanks!

Attention Campers!

Camp Hot-to-Clot is scheduled for August 4 – 10, 2013.

Registration opens: April 15, 2013
Registration deadline: July 1, 2013

Beginning April 15, you may register online at:
www.ecampFireSoftware.com

If you need a paper application, please call **412-209-7284**.

Once again, Camp Hot-to-Clot will be held at **Camp Kon-O-Kwee, in Fombell, PA**.

Calendar of Upcoming Events

Tuesday, April 9
Harrisburg Day
Harrisburg, PA

Saturday, April 13
Navigating Financial Aid and the Scholarship Process
Homestead, PA

Saturday, April 27- Sunday, April 28
Women's Retreat
Seven Springs, PA

Saturday, June 8
Run For Their Lives 5K - Erie
Findley Lake, NY

Saturday, June 15
PEN's Insurance Pulse On The Road!
Pittsburgh, PA

Sunday, July 28
WPCNHF Annual Meeting and Walk Kickoff
North Park, Allison Park, PA

Sunday, August 4 – Saturday, August 10
Camp Hot-to-Clot
Fombell, PA

Saturday, September 21
Hemophilia Walk
North Park, Allison Park, PA

Saturday, September 21
Run For Their Lives 5K
North Park, Allison Park, PA

Thursday, October 3 – Saturday, October 5
NHF Annual Meeting
Anaheim, CA

Saturday, October 26
Educational Program and Social Event
Erie, PA

Friday, November 22 – Sunday, November 24
Take A Bough
Pittsburgh, PA

CFC Combined Federal Campaign

Combined Federal Campaign

WPCNHF is an approved charitable organization for the Combined Federal Campaign (CFC). If you participate in the CFC, please consider designating all or a portion of your donation to the Chapter.

WPCNHF CFC Number is: 81343

Cash For Trash Fundraiser

Recycle your inkjet and toner cartridges

Cartridges can be brought from home, work, or local businesses. Be sure to ask your employer to sponsor us by saving their empty toner cartridges. We receive funds for the Chapter for empty laser, fax, and copier cartridges. Inkjet cartridges can earn money too! Recycling cartridges not only helps to alleviate America's landfills, it makes "cents" too! All money earned from this program will go to WPCNHF. Please drop empty cartridges at:

WPCNHF
20411 Route 19, Unit 14
Cranberry Twp., PA 16066

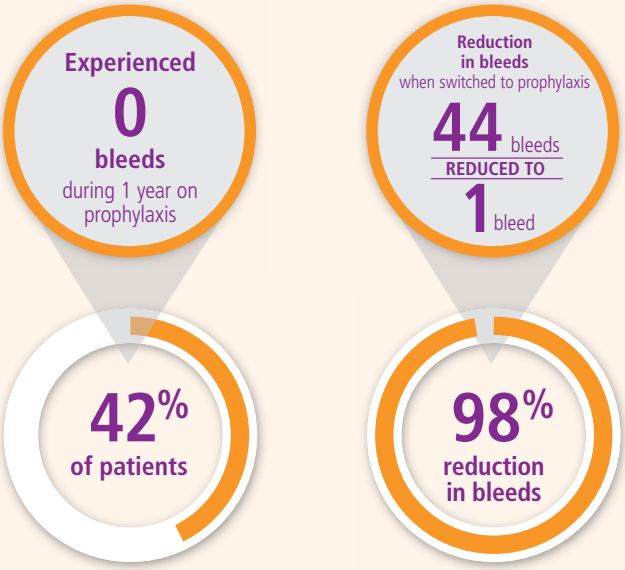
Ask us about sponsorship opportunites and how you can help!

UNLOCKING SELF-POTENTIAL

PROPHYLAXIS WITH ADVATE REDUCED BLEEDS IN A CLINICAL STUDY[1,a]

ADVATE is the only recombinant factor VIII (eight) that is FDA approved for prophylaxis in both adults & children (0-16 years)[1]

Significant reduction in median annual bleed rate (ABR) with prophylaxis treatment compared with on-demand treatment[1,a]



- 0 bleeds experienced by 42% of patients during 1 year on prophylaxis[1,a]
- 98% reduction in median annual bleed rate (ABR) from 44 to 1 when switched from on-demand to prophylaxis[1,a]
- 97% reduction in joint bleeds from 38.7 to 1 after switching from on-demand to prophylaxis[1,a]
- No subject developed factor VIII inhibitors or withdrew due to an adverse event (AE)[2,a]

[a]In a clinical study, after switching from 6 months of on-demand treatment to 12 months of prophylaxis with ADVATE in 53 previously treated patients with severe or moderately severe hemophilia A.

Ask your healthcare provider if prophylaxis with ADVATE is right for you.

Detailed Important Risk Information for ADVATE

You should not use ADVATE if you are allergic to mice or hamsters or any ingredients in ADVATE.

You should tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines and dietary supplements, have any allergies, including allergies to mice or hamsters, are nursing, are pregnant, or have been told that you have inhibitors to factor VIII.

You can have an allergic reaction to ADVATE. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.

Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

Side effects that have been reported with ADVATE include: cough, sore throat, unusual taste, abdominal pain, diarrhea, nausea/vomiting, headache, fever, dizziness, hot flashes, chills, sweating, joint swelling/aching, itching, hematoma, swelling of legs, runny nose/congestion, and rash.

Call your healthcare provider right away about any side effects that bother you or if your bleeding does not stop after taking ADVATE.

Indication for ADVATE

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is a medicine used to replace clotting factor VIII that is missing in people with hemophilia A (also called "classic" hemophilia). ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A. Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand Disease.

Please see Brief Summary of ADVATE Prescribing Information on the next page.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

References:

1. ADVATE Prescribing Information. Westlake Village, CA: Baxter Healthcare Corporation; July 2012. **2.** Valentino LA, Mamonov V, Hellmann A, et al. A randomized comparison of two prophylaxis regimens and a paired comparison of on-demand and prophylaxis treatments in hemophilia A management. *J Thromb Haemost*. 2012;10(3):359-367.

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
There's more to life.

www.advate.com | 888.4.ADVATE

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
Brief Summary of Prescribing Information. Please see package insert for full prescribing information.

INDICATIONS AND USAGE

Control and Prevention of Bleeding Episodes

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is an Antihemophilic Factor (Recombinant) indicated for control and prevention of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

Perioperative Management

ADVATE is indicated in the perioperative management in adults and children (0-16 years) with Hemophilia A.

Routine Prophylaxis

ADVATE is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

ADVATE is not indicated for the treatment of von Willebrand disease.

CONTRAINDICATIONS

Known anaphylaxis to mouse or hamster protein or other constituents of the product.

WARNINGS AND PRECAUTIONS

Anaphylaxis and Hypersensitivity Reactions

Allergic-type hypersensitivity reactions, including anaphylaxis, are possible and have been reported with ADVATE. Symptoms have manifested as dizziness, paresthesias, rash, flushing, face swelling, urticaria, dyspnea, and pruritus. *[See Patient Counseling Information (17) in full prescribing information]*

ADVATE contains trace amounts of mouse immunoglobulin G (MuIgG): maximum of 0.1 ng/IU ADVATE and hamster proteins: maximum of 1.5 ng/IU ADVATE. Patients treated with this product may develop hypersensitivity to these non-human mammalian proteins.

Discontinue ADVATE if hypersensitivity symptoms occur and administer appropriate emergency treatment.

Neutralizing Antibodies

Carefully monitor patients treated with AHF products for the development of Factor VIII inhibitors by appropriate clinical observations and laboratory tests. Inhibitors have been reported following administration of ADVATE predominantly in previously untreated patients (PUPs) and previously minimally treated patients (MTPs). If expected plasma Factor VIII activity levels are not attained, or if bleeding is not controlled with an expected dose, perform an assay that measures Factor VIII inhibitor concentration. *[See Warnings and Precautions (5.3) in full prescribing information]*

Monitoring Laboratory Tests

The clinical response to ADVATE may vary. If bleeding is not controlled with the recommended dose, determine the plasma level of Factor VIII and administer a sufficient dose of ADVATE to achieve a satisfactory clinical response. If the patient's plasma Factor VIII level fails to increase as expected or if bleeding is not controlled after the expected dose, suspect the presence of an inhibitor (neutralizing antibodies) and perform appropriate tests as follows:

- Monitor plasma Factor VIII activity levels by the one-stage clotting assay to confirm the adequate Factor VIII levels have been achieved and maintained when clinically indicated. *[See Dosage and Administration (2) in full prescribing information]*
- Perform the Bethesda assay to determine if Factor VIII inhibitor is present. If expected Factor VIII activity plasma levels are not attained, or if bleeding is not controlled with the expected dose of ADVATE, use Bethesda Units (BU) to titer inhibitors.
  - If the inhibitor titer is less than 10 BU per mL, the administration of additional Antihemophilic Factor concentrate may neutralize the inhibitor and may permit an appropriate hemostatic response.
  - If the inhibitor titer is above 10 BU per mL, adequate hemostasis may not be achieved. The inhibitor titer may rise following ADVATE infusion as a result of an anamnestic response to Factor VIII. The treatment or prevention of bleeding in such patients requires the use of alternative therapeutic approaches and agents.

ADVERSE REACTIONS

The serious adverse drug reactions (ADRs) seen with ADVATE are hypersensitivity reactions and the development of high-titer inhibitors necessitating alternative treatments to Factor VIII.

The most common ADRs observed in clinical trials (frequency ≥ 10% of subjects) were pyrexia, headache, cough, nasopharyngitis, vomiting, arthralgia, and limb injury.

Clinical Trial Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in clinical trials of another drug and may not reflect the rates observed in clinical practice.

ADVATE has been evaluated in five completed studies in previously treated patients (PTPs) and one ongoing study in previously untreated patients (PUPs) with severe to moderately severe Hemophilia A (Factor VIII ≤ 2% of normal). A total of 234 subjects have been treated with ADVATE as of March 2006. Total exposure to ADVATE was 44,926 infusions. The median duration of participation per subject was 370.5 (range: 1 to 1,256) days and the median number of exposure days to ADVATE per subject was 128.0 (range: 1 to 598).[1]

The summary of adverse reactions (ADRs) with a frequency ≥ 5% (defined as adverse events occurring within 24 hours of infusion or any event causally related occurring within study period) is shown in Table 1.

No subject was withdrawn from a study due to an ADR. There were no deaths in any of the clinical studies.

IMMUNOGENICITY

The development of Factor VIII inhibitors with the use of ADVATE was evaluated in clinical studies with pediatric PTPs (< 6 years of age with > 50 Factor VIII exposures) and PTPs (≥ 10 years of age with > 150 Factor VIII exposures). Of 198 subjects who were treated for at least 10 exposure days or on study for a minimum of 120 days, 1 adult developed a low-titer inhibitor (2.0 [BU] in the Bethesda assay) after 26 exposure days. Eight weeks later, the inhibitor was no longer detectable, and *in vivo* recovery was normal at 1 and 3 hours after infusion of another marketed recombinant Factor VIII concentrate. This single event results in a Factor VIII inhibitor frequency in PTPs of 0.51% (95% CI of 0.03 and 2.91% for the risk of any Factor VIII inhibitor development).[1,2] No Factor VIII inhibitors were detected in the 53 treated pediatric PTPs.

In clinical studies that enrolled previously untreated subjects (defined as having had up to 3 exposures to a Factor VIII product at the time of enrollment), 5 (20%) of 25 subjects who received ADVATE developed inhibitors to Factor VIII.[1] Four patients developed high titer (> 5 BU) and one patient developed low-titer inhibitors. Inhibitors were detected at a median of 11 exposure days (range 7 to 13 exposure days) to investigational product.

Immunogenicity also was evaluated by measuring the development of antibodies to heterologous proteins. 182 treated subjects were assessed for anti-Chinese hamster ovary (CHO) cell protein antibodies. Of these patients, 3 showed an upward trend in antibody titer over time and 4 showed repeated but transient elevations of antibodies. 182 treated subjects were assessed for mulgG protein antibodies. Of these, 10 showed an upward trend in anti-mulgG antibody titer over time and 2 showed repeated but transient elevations of antibodies. Four subjects who demonstrated antibody elevations reported isolated events of urticaria, pruritus, rash, and slightly elevated eosinophil counts. All of these subjects had numerous repeat exposures to the study product without recurrence of the events and a causal relationship between the antibody findings and these clinical events has not been established.

Of the 181 subjects who were treated and assessed for the presence of anti-human von Willebrand Factor (VWF) antibodies, none displayed laboratory evidence indicative of a positive serologic response.

Post-Marketing Experience

The following adverse reactions have been identified during post-approval use of ADVATE. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to drug exposure.

Among patients treated with ADVATE, cases of serious allergic/hypersensitivity reactions including anaphylaxis have been reported and Factor VIII inhibitor formation (observed predominantly in PUPs). Table 2 represents the most frequently reported post-marketing adverse reactions as MedDRA Preferred Terms.

Table 1
Summary of Adverse Reactions (ADRs)[a] with a Frequency ≥ 5% in 234 Treated Subjects[b]

MedDRA[c] System Organ Class	MedDRA Preferred Term	Number of ADRs	Number of Subjects	Percent of Subjects
General disorders and administration site conditions	Pyrexia	78	50	21
Nervous system disorders	Headache	104	49	21
Respiratory, thoracic and mediastinal disorders	Cough	75	44	19
Infections and infestations	Nasopharyngitis	61	40	17
Gastrointestinal disorders	Vomiting	35	27	12
Musculoskeletal and connective tissue disorders	Arthralgia	44	27	12
Injury, poisoning and procedural complications	Limb injury	55	24	10
Infections and infestations	Upper respiratory tract infection	24	20	9
Respiratory, thoracic and mediastinal disorders	Pharyngolaryngeal pain	23	20	9
Respiratory, thoracic and mediastinal disorders	Nasal congestion	24	19	8
Gastrointestinal disorders	Diarrhea	24	18	8
Gastrointestinal disorders	Nausea	21	17	8
General disorders and administration site conditions	Pain	19	17	8
Skin and subcutaneous tissue disorders	Rash	16	13	6
Infections and infestations	Ear infection	16	12	5
Injury, poisoning and procedural complications	Procedural pain	16	12	5
Respiratory, thoracic and mediastinal disorders	Rhinorrhea	15	12	5

[a] ADRs are defined as all Adverse Events that occurred (a) within 24 hours after being infused with investigational product or (b) all Adverse Events assessed related or possibly related to investigational product or (c) Adverse Events for which the investigator's or sponsor's opinion of causality was missing or indeterminate.

[b] The ADVATE clinical program included 234 treated subjects from 5 completed studies in PTPs and 1 ongoing study in PUPs as of 27 March 2006.

[c] MedDRA version 8.1 was used.

Table 2
Post-Marketing Experience

Organ System [MedDRA Primary SOC]	Preferred Term
Immune system disorders	Anaphylactic reaction[a] Hypersensitivity[a]
Blood and lymphatic system disorders	Factor VIII inhibition
General disorders and administration site conditions	Injection site reaction Chills Fatigue/Malaise Chest discomfort/pain Less-than-expected therapeutic effect

[a] These reactions have been manifested by dizziness, paresthesias, rash, flushing, face swelling, urticaria, and/or pruritus.

References: 1. Shapiro A, Gruppo R, Pabinger I et al. Integrated analysis of safety and efficacy of a plasma- and albumin-free recombinant factor VIII (rAHF-PFM) from six clinical studies in patients with hemophilia A. Expert Opin Biol Ther 2009 9:273-283. **2.** Tarantino MD, Collins PW, Hay PW et al. Clinical evaluation of an advanced category antihaemophilic factor prepared using a plasma/albumin-free method: pharmacokinetics, efficacy, and safety in previously treated patients with haemophilia A. Haemophilia 2004 10:428-437.

To enroll in the confidential, industry-wide Patient Notification System, call 1-888-873-2838.

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Basic Overview of Gene Therapy in Hemophilia and Studies Offered at HCWP

By Kim Goldby-Reffner, RN, BS, CCRC. Reviewed & edited by Margaret Ragni, MD, MPH

One of the many current trending research topics in hemophilia is gene therapy. However, many are unsure of what "gene therapy" actually entails and if it is something they might want to participate in. Others wonder where they can go for more information. It is my hope that this article can help de-mystify gene therapy by touching on the basics, the history, where we are now, and where to get additional information.

Gene therapy was first introduced as a concept in the early 1970's. Scientists began to contemplate if "good DNA" could replace "defective DNA". Throughout the next few decades, research was done in animals, and then advanced to human trials for diseases like thalassemia, cystic fibrosis, SCID (severe combined immunodeficiency) and some forms of cancer. DNA (which stands for deoxyribonucleic acid) along with another genetic material called RNA (which stands for ribonucleic acid) can be thought of as blueprints or instructions for development and functioning for all living things (including people). Both DNA and RNA are located inside cells and are code for proteins that do things in the body. I like to think of DNA and RNA as the blueprints to a new house. I equate contractors to proteins. The house blueprints tell the contractors where, when and how to build the house and its contents. The contractor's job is to gather all the equipment (like lumber, nails, drywall, pipes and even appliances) to build and make the house so it functions properly. It's the same way with DNA, it directs the proteins where, when and how to build the human body so it functions properly. If something in the house doesn't work, for example, the pipes are leaking, then the contractors need to come back and fix the pipes. If there are no contractor's available, the pipes keep leaking! If a person is injured and is bleeding, then they need proteins to come back and fix the bleeding. A person with hemophilia (especially severe hemophilia) doesn't have enough proteins to stop the bleeding. They need additional proteins to stop the bleeding. The basic goal of gene therapy in patients with hemophilia is to "deliver" a new package of blueprints (DNA or RNA) to produce lasting and functional proteins (contractors) that the body can use when it needs to stop bleeding. Scientists started working on gene therapy in the 1990's to see if the code to make Factor VIII and Factor IX (called a gene) could be "delivered" into cells of men with hemophilia. The goal of course is for these men to begin producing their own functioning FVIII or FIX.

Success was varied, with promising results best shown for hemophilia B (or FIX deficiency). Within the last few years physicians and researchers like Dr Katherine High, Drs Nathwani, Nienhuis and Davidoff have collaborated with a gene therapy research product (called "scAAV2/8-LP1-hFIXco") or AAV8 for short. To condense hours of scholarly papers and articles, recently published results show that AAV8 gene therapy might hold promise for converting a severe hemophilia B patient (with FIX levels less than 1%) into a mild hemophilia B patient. Publications report that FIX levels achieved are around 3-7% which helps by reducing the number of patient bleeds. This means that the new blueprints are being followed (to some degree) and the contracted factor IX proteins are showing up for the job! It's a start. Of course this is a very unscientific way to explain what is occurring and it is for this reason I urge you to speak more about gene therapy with the expert staff at your Hemophilia Treatment Center and your hematologist. Gene therapy is after all, research and not without risks like liver enzyme elevations and immune system responses (just to name two). Additionally, gene therapy research also involves a time commitment. It is however showing some promise and researchers are moving forward.

Two current gene therapy studies offered at our center are called St Jude's Gene IX prescreening study and CHOP's (Children's Hospital of Philadelphia) AAV8-hFIX-101 study. Both are approved by an ethics committee for adult males with severe hemophilia B. In addition to speaking with your HTC and doctor, you can find information about gene therapy at http://www.nlm.nih.gov and locating gene therapy studies near you at http://www.clinicaltrials.gov.

Bowling Marathon to Benefit WPCNHF

The Western Pennsylvania Chapter of the National Hemophilia Foundation Strikes Out Bleeding Disorders

Starting on February 25, 2013 20 participants were given their own team fundraising page and asked to raise $1,000. The 30 day fundraising campaign ended on Sunday, March 24, 2013 with WPCNHF's First Annual Bowling for Bleeding Disorders bowling marathon.

Bowling for Bleeding Disorders was held at Paradise Island Bowl in Neville Township, Pennsylvania where 11 teams collectively bowled 100 frames in three hours. The event was open to the general public on a first come, first serve basis. The registration fee was $25 per participant.

The highest fundraising team award along with a basket donated by The Sweet Shoppe and Nut House went to Awesome John's Crusaders who raised $1,755. The highest individual fundraising award went to Susan Eyrolles. Trophies were also given out during the event to the highest scoring and lowest scoring bowler and team.

Around 50 people attended the event. Industry sponsors included the Hemophilia Center of Western Pennsylvania, Pfizer, Grifols, CSL Behring, Accredo, and Octapharma. After expenses, $6,758 was raised to benefit the Western Pennsylvania Chapter of the National Hemophilia Foundation.

WPCNHF would like to thank all who attended and participated in making the 1st Annual Bowling for Bleeding Disorders a success!

Hemophilia Not Protective Against Heart Disease

By Sarah Aldridge

In the past decade, research has shown that people with hemophilia are just as prone to heart conditions as the general public.

In a six-state study of more than 3,400 men with hemophilia, investigators working with the Centers for Disease Control and Prevention in Atlanta uncovered some hints about heart health and adults with hemophilia. "After HIV and intracranial hemorrhage, the third most common cause of death was heart disease," says Roshni Kulkarni, MD, director of pediatric and adolescent hematology/oncology and professor in the Department of Pediatrics at Michigan State University in East Lansing. She was lead author of the study, published in the American Journal of Hematology in 2005. Using data from hospital records, Kulkarni and associates found the incidence of ischemic heart disease (reduced blood supply to the heart usually from coronary artery disease) was not significantly different when compared to nonhemophilic men. "They were at risk for heart disease just like the rest of the population, so hemophilia was not protective."

In a 2009 research study published in Haemophilia, Barbara Konkle, MD, and colleagues identified cardiovascular disease as a co-morbidity of older men with hemophilia. A 2010 study in the Journal of Thrombosis and Haemostasis then showed that men with hemophilia had equivalent incidences of atherosclerosis (narrowing of the arteries) as men in the general population. A study of 185 men with hemophilia at the Indiana Hemophilia & Thrombosis Center in Indianapolis, published in Haemophilia in 2011, further showed they were twice as likely to develop coronary artery disease, stroke or heart attack as non-Hispanic white males. High blood pressure and smoking were contributing risk factors.

Further, exposure to high factor levels can trigger increased clot formation. This is a greater concern for patients with hemophilia B or inhibitors who use activated prothrombin complex concentrates (APCCs). "Taking an APCC is clearly a risk factor for heart disease," Kulkarni says. "If you have bad blood vessels in your heart, they can form a clot there."

Routine Heart Screenings
Primary care providers (PCPs) who perform routine screenings for cholesterol and triglycerides, can help identify and treat conditions that lead to heart disease.

So if you're anxious about the angina that runs in your family, schedule an appointment with your PCP today. You'll do your heart a favor.

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Antihemophilic Factor (Recombinant), Plasma/Albumin-Free

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talk to your health care provider to see if XYNTHA® SOLOFUSE® is right for you. One-time offer.*

Terms and Conditions can be found at FreeTrialXyntha.com

* You must be currently covered by a private (commercial) insurance plan. If you are not eligible for the XYNTHA Trial Prescription Program, you may find help accessing Pfizer medicines by contacting Pfizer's RSVP program at 1-888-327-RSVP (7787).

What is XYNTHA?

Xyntha® Antihemophilic Factor (Recombinant), Plasma/Albumin-Free is indicated for the control and prevention of bleeding episodes in patients with hemophilia A (congenital factor VIII deficiency or classic hemophilia) and for surgical prophylaxis in patients with hemophilia A.

XYNTHA does not contain von Willebrand factor and, therefore, is not indicated in von Willebrand's disease.

Important Safety Information for XYNTHA

- Call your healthcare provider or go to the emergency department right away if you have any of the following symptoms because these may be signs of a serious allergic reaction: wheezing, trouble breathing, chest tightness, turning blue (look at lips and gums), fast heartbeat, swelling of the face, faintness, rash, or hives. XYNTHA contains trace amounts of hamster protein. You may develop an allergic reaction to these proteins. Tell your healthcare provider if you have had an allergic reaction to hamster protein.
- Call your healthcare provider right away if bleeding is not controlled after using XYNTHA: this may be a sign of an inhibitor, an antibody that may stop XYNTHA from working properly. Your healthcare provider may need to take blood tests to monitor for inhibitors.
- The most common adverse reaction in the safety and efficacy study is headache (24% of subjects) and in the surgery study is fever (43% of subjects). Other common side effects of XYNTHA include nausea, vomiting, diarrhea, or weakness.
- XYNTHA is an injectable medicine administered by intravenous (IV) infusion. You may experience local irritation when infusing XYNTHA after reconstitution in XYNTHA® SOLOFUSE®.

Please see brief summary of full Prescribing Information.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Wyeth® Manufactured by Wyeth Pharmaceuticals Inc.

Marketed by Pfizer Inc.

Printed in USA/September 2012

Pfizer

xyntha

Antihemophilic Factor (Recombinant), Plasma/Albumin-Free

xyntha solofuse

Antihemophilic Factor (Recombinant), Plasma/Albumin-Free

R_x only

Brief Summary

See package insert for full Prescribing Information, including patient labeling. For further product information and current patient labeling, please visit XYNTHA.com or call Wyeth Pharmaceuticals toll-free at 1-800-934-5556.

Please read this Patient Information carefully before using XYNTHA and each time you get a refill. There may be new information. This leaflet does not take the place of talking with your healthcare provider about your medical problems or your treatment.

What is XYNTHA?

XYNTHA is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia A. Hemophilia A is also called classic hemophilia.

XYNTHA is not used to treat von Willebrand's disease.

What should I tell my healthcare provider before using XYNTHA?

Tell your healthcare provider about all your medical conditions, including if you:

- are pregnant or planning to become pregnant. It is not known if XYNTHA may harm your unborn baby.
- are breastfeeding. It is not known if XYNTHA passes into your milk and if it can harm your baby.

Tell your healthcare provider and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal remedies.

XYNTHA contains trace amounts of hamster proteins. You should not use XYNTHA if you are allergic to hamster protein.

How should I infuse XYNTHA?

Step-by-step instructions for infusing with XYNTHA are provided at the end of the complete Patient Information leaflet. The steps listed below are general guidelines for using XYNTHA. Always follow any specific instructions from your healthcare provider. If you are unsure of the procedures, please call your healthcare provider before using.

Call your healthcare provider right away if bleeding is not controlled after using XYNTHA. Your body can also make antibodies against XYNTHA (called "inhibitors") that may stop XYNTHA from working properly. Your healthcare provider may need to take blood tests from time to time to monitor for inhibitors.

Call your healthcare provider right away if you take more than the dose you should take.

Talk to your healthcare provider before traveling. Plan to bring enough XYNTHA for your treatment during this time.

What are the possible or reasonably likely side effects of XYNTHA?

Common side effects of XYNTHA are:

- headache
- fever
- nausea
- vomiting
- diarrhea
- weakness

Call your healthcare provider or go to the emergency department right away if you have any of the following symptoms because these may be signs of a serious allergic reaction:

- **wheezing**
- **difficulty breathing**
- **chest tightness**
- **turning blue (look at lips and gums)**
- **fast heartbeat**
- **swelling of the face**
- **faintness**
- **rash**
- **hives**

Talk to your healthcare provider about any side effect that bothers you or that does not go away. You may report side effects to FDA at 1-800-FDA-1088.

How should I store XYNTHA?

Do not freeze.

Protect from light.

XYNTHA Vials

Store XYNTHA in the refrigerator at 36° to 46°F (2° to 8°C). Store the diluent syringe at 36° to 77°F (2° to 25°C).

XYNTHA can last at room temperature (below 77°F) for up to 3 months. If you store XYNTHA at room temperature, carefully write down the date you put XYNTHA at room temperature, so you will know when to either put it back in the refrigerator, use it immediately, or throw it away. There is a space on the carton for you to write the date.

If stored at room temperature, XYNTHA can be returned one time to the refrigerator until the expiration date. Do not store at room temperature and return it to the refrigerator more than once. Throw away any unused XYNTHA after the expiration date.

Infuse XYNTHA within 3 hours of reconstitution. You can keep the reconstituted solution at room temperature before infusion, but if you have not used it in 3 hours, throw it away.

Do not use reconstituted XYNTHA if it is not clear to slightly opalescent and colorless.

Dispose of all materials, whether reconstituted or not, in an appropriate medical waste container.

XYNTHA SOLOFUSE

Store in the refrigerator at 36° to 46°F (2° to 8°C).

XYNTHA SOLOFUSE can last at room temperature (below 77°F) for up to 3 months. If you store XYNTHA SOLOFUSE at room temperature, carefully write down the date you put XYNTHA SOLOFUSE at room temperature, so you will know when to throw it away. There is a space on the carton for you to write the date.

Throw away any unused XYNTHA SOLOFUSE after the expiration date.

Infuse within 3 hours after reconstitution or after removal of the grey rubber tip cap from the prefilled dual-chamber syringe. You can keep the reconstituted solution at room temperature before infusion, but if it is not used in 3 hours, throw it away.

Do not use reconstituted XYNTHA if it is not clear to slightly opalescent and colorless.

Dispose of all materials, whether reconstituted or not, in an appropriate medical waste container.

What else should I know about XYNTHA?

Medicines are sometimes prescribed for purposes other than those listed here. Talk to your healthcare provider if you have any concerns. You can ask your healthcare provider for information about XYNTHA that was written for healthcare professionals.

Do not share XYNTHA with other people, even if they have the same symptoms that you have.

This brief summary is based on the Xyntha® [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free] Prescribing Information LAB-0516-3.0, revised 06/12, and LAB-0500-7.0, revised 06/12.

Wyeth®

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Infusion Day

WPCNHF held our second annual Infusion Day in conjunction with the Hemophilia Center of Western Pennsylvania (HCWP) on March 2nd. Kristen Jaworski, RN, BSN, CCRC of HCWP committed to the event with her colleagues in order to further empower members of the bleeding disorders community. There were three stations setup throughout the event- an infusion station supported by Anne Graham, RN and Donna Flemm, Medical Assistant, a BayCuff™ (an adjustable cuff worn on the hand or arm that allows patients to practice the technique of self-infusion without actually infusing into their own vein) practice station supported by Anna Dracar, RN and Sarah Simpson, RN, and a Good Veins/Bad Veins station supported by Kim Goldby-Reffner, RN, BA as well as Kristen Jaworski. Prizes were distributed, games were played, a lot was learned and a good time was had by everyone who attended. Some members of our community infused for their first time, while others stuck to the practice station. The level of participation or skill set did not matter as this was a pressure-free learning environment and everyone was able to learn at their own speed and as their comfort allowed.

WPCNHF would like to offer a special thank you to the staff of HCWP for their contributions to the event. Each member of the HCWP staff took time out of their weekend to ensure a fun-filled Infusion Day. WPCNHF is incredibly grateful of their time and collaboration. Additionally, we would like to thank our sponsors for their support of our annual Infusion Day. Sponsors were as follows:

Accredo HHS
Baxter BioScience
Bayer HealthCare
BioPlus
BioRx
CSL Behring
Grifols
Hemophilia Center of Western Pennsylvania
Novo Nordisk
Pfizer
Walgreens

NIH Funding Cuts Could Limit Research Grants and Jobs

A recent article in The Fiscal Times offered some basic funding and job numbers associated with sequestration and federally funded research. Sequestration is a series of automatic across-the-board funding cuts to government agencies, totaling $1.2 trillion over 10 years. The cuts, which would be enacted if the White House and Congress do not reach an agreement on the federal budget, would be split 50-50 between defense and domestic discretionary spending. Potential cuts to vital research funding for the National Institutes of Health (NIH) are projected at $1.6 billion.

NIH currently supports approximately 402,000 jobs and $57.8 billion in economic growth or output. According to a new analysis by United for Medical Research (UMR), a coalition of research industry advocates and grant recipients, a 5.1% sequester would reduce the total number of NIH-related jobs by more than 20,500 and reduce economic activity by $3 billion. Industry, science and consumer health advocates warn that the fallout would hinder crucial areas of research such as cancer, heart, blood and AIDS.

According to the UMR study, California could lose the most jobs (3,028), followed by Massachusetts (1,736), New York (1,645) and Pennsylvania (1,209). Sue Nelson, vice president for federal advocacy at the American Heart Association and a former Senate Budget Committee official, cited an example of the far-reaching effects of such NIH cuts. "A lot of companies manufacture equipment they sell to researchers," Nelson told The Fiscal Times. "A research lab is like a small business. We employ everyone from the highest level researchers to persons who clean the test tubes. And then we all go out for lunch and buy from the corner lunch stand. So when a lab gets cut, it's like closing down a small business, and that's what's happening all across the country."

"Thousands of grants will be eliminated and cutting-edge research on blood and other diseases will be stifled. The lack of funding for new projects and the uncertainty of continued funding for current projects will have a long-term negative impact on biomedical research, slowing the development of cures and treatments for patients," said the American Society of Hematology (ASH) in a statement to its advocacy partners.

Source: *The Fiscal Times, February 6, 2013*

Inhibitor Insights

By Laurie Kelley

Will Inhibitor Reimbursement Change under Healthcare Reform?
Unless you've been living in a cave, under a rock, or on a cruise ship, or watching nonstop episodes of *Lost* and *The Tudors* without your cell phone nearby, you can't help but know that healthcare reform will continue with President Obama's reelection. Despite the controversy surrounding ACA, or the Affordable Care Act (its constitutionality was attacked and several states are trying to revoke it), the law offers several immediate positive results for families with bleeding disorders. Notably, children can stay on their parents' insurance until age 26 (regardless of whether they are in college or married); people with bleeding disorders won't be denied insurance just because they have an expensive medical condition; and lifetime limits have been eliminated.

In a nutshell, no insurance company can terminate you because you've used up your insurance money, or prevent you from being insured because you have a bleeding disorder. *Yes!*

But an inhibitor family may wonder, What about us? With higher-than-average hemophilia treatment costs, how will healthcare reform impact inhibitor reimbursement specifically? Because more people with bleeding disorders can now be insured and use as much factor as they need, treatment costs for these disorders will undoubtedly increase—with insurance companies mandated to pay for them. Will more costs be shifted to consumers? Will some treatments be restricted? Good questions.

Background first: How factor is covered
Every health insurance plan has two parts, representing two different budgets:
• **Medical benefit** (major medical) covers all clinical services, including doctor visits, diagnostic tests, surgery, and inpatient drugs.
• **Pharmacy benefit** covers outpatient drugs.

Roughly 75% of hemophilia patients have their factor covered through the medical benefit side of their insurance policy, which usually does not categorize drugs into tiers (see "Tiers" section). This means that copays for drugs are pretty predictable and stable.

Michelle Rice, mother of two with hemophilia and director of public policy at National Hemophilia Foundation (NHF), says, "ACA did not address reimbursement specifically as to prescription drugs. As each state is allowed to develop its own exchange,[1] we don't know exactly how prescription coverage will be addressed under the exchanges either. At this point we are not sure which benefit clotting factor will be covered under, major medical or pharmacy."

If we don't know, then what do the payers know? Are they knowledgeable about the special drugs needed to treat bleeding disorders, and specifically inhibitors?

Educating payers about hemophilia
NHF is on it, with a series of webinars that began in 2010 to educate payers about hemophilia treatment. The goal is to ensure that payers understand why these drugs are expensive; what they do; how they are used; why prophylaxis differs from on-demand, and how this affects long-term costs (and joint health). Payers also need to understand the differences in competitive brands of factor, and that one brand does not work with all patients. They must also know that biosimilar factors ("follow-on" recombinant factor products)

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Spotlight on The Member: The Clayton Family

When Darla and Gary Clayton's son Trent (now nine years old) was a baby, they noticed he was not using his right hand as he should and they began to suspect that something was wrong. They took him to their pediatrician and were told that their child was fine. When the Claytons did not see any noticeable improvement, they returned to the pediatrician who then referred them to a neurologist.

The neurologist ordered scans and discovered a mass in Trent's brain. The next day, at the age of 14 months, he underwent emergency brain surgery. The surgery exposed old blood that had pooled in his brain. Trent was in the ICU for almost a week. During this time, he received a blood transfusion, due to low blood oxygen levels. He was not tested for any bleeding disorders.

Over the course of the next several years Trent had many more bleeds in his brain, including micro bleeds that went unnoticed until evidence of them showed up in MRIs, which he had scheduled quarterly. Occasionally, he did show signs of bleeding, such as headaches, vomiting, and additional weakening on his right side. Eventually, the family traveled to Boston for a second surgery to remove another mass of blood in his brain. Although the surgery went well, they didn't get the whole mass. The surgeon in Boston was the first to use the diagnosis cerebral cavernous malformations to explain vascular differences in Trent's brain.

Around the age of three, after Darla received testing for an unrelated issue, she asked the doctors to test Trent for a clotting disorder. Trent finally received a diagnosis for his bleeding; he had Von Willedbrand Disease (VWD). At the time, Darla was told that there wasn't anything that could be done for his bleeds because they were spontaneous and couldn't be predicted. Trent was given a DDAVP challenge to determine if it could be given to him in the future for surgeries. The challenge appeared successful, at first, but shortly afterwards he developed three new lesions across the front of his brain.

The Claytons were new to VWD and wanted to know more. Darla told her story to just about everyone she knew, in an attempt to find answers and more information. One acquaintance put her in touch with Kim Ebsworth, one of our Chapter members. Kim talked with Darla and recommended that she contact the Hemophilia Center of Western PA (HCWP). Darla and Gary took Trent to the HCWP. By the time he was four years old, he had a port inserted and began receiving prophylactic treatments to help prevent bleeds.

The Claytons began infusing Trent at home and the process was sometimes stressful and frustrating. When the family attended their first Family Education Weekend with the Chapter, Darla attended a presentation about infusing at home. She left the session with a new outlook on home infusion. She says that when we are not calm, children can take the stress and frustration personally. Her advice to other parents is to not underestimate the importance of staying calm during infusions. If you need to, take a break and walk away.

Darla, a licensed psychologist, was determined to find a solution to help keep everyone calm during the infusion process. She decided to create a personalized photo book for Trent that would take him

through the process and remind him of his responsibility to remain calm. Darla also created a photo book for their daughter, Amarisa, who also has VWD (now age five), so that she would understand what her role was and the expectations her parents had for her. The book helped to keep her safe and calm during Trent's infusions.

During the 2012 Family Education Weekend, Darla co-presented a session on Calming Techniques. During the session, she reviewed her photo books, and also introduced the participants to a calming technique using a "Magic Glove."

Like most kids, Trent participated in various sports over the years. However, he was having some level of difficulty with most sports because his arm and leg on his right side are weak, as a result of the brain bleeds. Since Trent liked to run, his parents decided to let Trent focus on running, instead of playing other sports.

Trent began to participate in regional and national competitions through Wheelchair & Ambulatory Sports, USA. At age eight, he entered his first competition and placed first in his events! Trent, the only athlete who competed from the state of Pennsylvania, really enjoyed the experience. Most participants from other states attended as part of a team; however, Trent attended the competition as an individual, since there weren't any teams in our region. On the drive home, Trent suggested that the family start a team, and Strong As Steel Adaptive Sports was born! The organization is open to children in Western Pennsylvania who have a physical disability or a visual impairment. Strong As Steel Adaptive Sports will be hosting its first event on May 4, 2013, at Robert Morris Sports Complex, on Neville Island. For more information, visit www.strongassteeladaptivesports.org.

The Claytons are active members of the bleeding disorders community and participate in Chapter events regularly. Their favorite Chapter event is the Walk. Darla has volunteered on the Walk Committee for the past two years and has helped with the Kid's Zone on Walk day. In addition, she participates in the Take A Bough fundraiser and has led jewelry making sessions for both Take A Bough and the Women's Group. The Claytons are very active outside of the bleeding disorders coming, too, and are always on the go! In addition to their commitment to Strong As Steel Adaptive Sports, the children participate in numerous activities including gymnastics, archery, and scouting. As a family, they also enjoy camping, hiking, swimming, and watching movies together.

Meet The HCWP Staff

My name is Jennifer Warner, and I am the newest Social Worker/Mental Health Professional at HCWP. I started working at the center on February 26, 2013. I previously worked for Mercy Behavioral Health as a Clinical Psychologist for 12 years, and as one of their HIV and Liver Disease Early Intervention/Prevention Therapists for the last 4 years I was there. Most recently I worked with the West Penn Allegheny Health System as a Mental Health Worker in one of their Hospice programs in the 2 years before coming to HCWP.

I received my Bachelor's Degree from Waynesburg College with focus on Clinical Psychology and Biology, and my Master's Degree in Clinical Psychology from Duquesne University. I am currently pursuing certification as a Nationally Certified Psychologist and a Licensed Professional Counselor through the State of Pennsylvania.

Michelle Alabek is in the new genetic counselor at HCWP. She graduated with her Bachelor's in Biology from Virginia Tech and her Master's in Genetic Counseling from Virginia Commonwealth University. Previously, Michelle worked in Louisville, KY as a cancer genetic counselor before returning to her hometown to work as the first genetic counselor at HCWP. Michelle will provide genetic counseling to patients and their family members, which includes documenting family history, explaining the underlying genetics of the diagnosis, discussing available testing options, interpreting test results, and reviewing implications for other family members. In addition to genetic counseling, she is interested in research, ethics and public health as they relate to genetics. She also enjoys being involved in education of the community and health care professionals.

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Staff office hours are Monday through Friday from 9 a.m. until 4 p.m. Every attempt will be made to return calls received during regular office hours on the same day.

Our Mission:

The Western Pennsylvania Chapter of the National Hemophilia Foundation is leading the way in Western Pennsylvania in improving the quality of care and enriching the lives of those with bleeding disorders through education, advocacy, resource, and referral.

WPCNHF Wish List

The Chapter is always doing fundraisers to raise money for our educational programs and member support activities but sometimes we just need a few small things for the office. WPCNHF has a list of items needed in the office. If you, or anyone you know, is interested in donating any of the following please contact the office at info@westpennhemophilia.org or call us at 724-741-6160.

- White copy paper by the ream or by the case
- Colored copy paper by the ream for invitations and newsletter inserts
- Legal pads for note taking
- Sticky Notes
- Forever U.S. Postage stamps
- 10 x 13 Ready-seal envelopes for newsletter mailings
- Paper towels
- Apartment-sized refrigerator

Washington Days 2013

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Usually, Tier IV drugs require exorbitant patient cost-sharing – patients must pay a percentage of the cost of these drugs – from 25% to 33% or more co-insurance – rather than a fixed, co-payment. Treatments for hemophilia, known as clotting factor therapies, are typically placed on the specialty tier. The yearly cost for clotting factor can be as high as $300,000 per year for a person with severe hemophilia, and can exceed $1 million for a person who develops an inhibitor. People with bleeding disorders simply cannot afford to pay 25% of this cost. This legislation, co-sponsored by a democrat and a republican, would benefit many people nationwide with expensive chronic conditions. While the bill has not yet been scored by the Congressional Budget Office (CBO), it is anticipated that the score would be very low since the bill only affects private insurance plans and not Medicare and Medicaid. Further, it is anticipated that the bill would have a small impact on insurance premiums.

Our chapter had several meetings throughout the day, including those with Senator Bob Casey and Representative Keith Rothfus (see photo) as well as with representatives from Senator Toomey's Office. All of our meetings went exceptionally well, and we're hoping that our voices were heard!

Inhibitor Insights

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are not all the same—each is made from a different cell line, uses a different fermentation process, and undergoes different purification and viral inactivation processes; any of these can cause the drug to act differently than the similar brand-name drug. And payers must understand the need for access to all therapies. To date, NHF has educated more than 400 participant payers in this valuable series. So, are they getting it?

Rice notes, "The general feeling we receive when talking to payers is that most understand hemophilia, and particularly the uniqueness of an inhibitor. They recognize that there is little they can do to manage the cost. Their understanding might be as simple as 'an inhibitor is a complication that while expensive, if not treated, could lead to increased costs long term, and potentially fatal outcomes.' They seem to understand that it's important to let the physician guide this treatment."

So far, encouraging. Instead of looking only at per-unit cost, or total cost per year, it's vital that payers know why a treatment is advised; know the medical and lifestyle outcomes of following a physician's prescribed regimen; and know the cost over the long run of not accepting a course of therapy. This means educating payers about the cost of lost productivity at work when a patient has a bleed and the cost of joint replacement when a patient is older.

Tiers always win?

Still, as healthcare reform progresses and as costs rise for payers, prescription drug tiers become a way to manage costs. Tiers are classifications of drugs within an insurance formulary that allow insurance companies to charge varying out-of-pocket expenses—the portion of costs you pay. There are four tiers:

- **Generic drugs** (tier 1)
- **Brand-name drugs** (tiers 2 and 3)
- **Specialty drugs** (tier 4)

Tier 1 requires the lowest copayment, usually $10 to $50. Copays tend to go up with higher tiers, with tier 4 the highest. Specialty drugs account for only 1% of total drug prescriptions but represent 17% of drug spending by private insurers.[2] Tiers are a good tactic to encourage consumers to choose lower-cost generic drugs.

But there are no generic drugs for factor. And specialty drugs that fall under tier 4 can incur coinsurance charges, instead of flat copays, for drugs that cost more than $500 and/or for injectable therapies. Currently, factor is not considered a specialty drug; our national hemophilia organizations are working hard to keep this from happening. But could factor—specifically inhibitor factor—eventually become a specialty drug?

"Unfortunately, I think the answer to this question is yes," says Rice. "Currently in most plans, all clotting factors are treated the same." Inhibitor drugs may not be carved out as special, untouchable by healthcare reform. Where hemophilia drugs go, so go inhibitor drugs—at least for now.

What can you do to protect inhibitor reimbursement?

While you wait to see how ACA evolves, there is much you can do to prepare for coming changes and to protect the coverage you have and need. Your first stop should almost always be your HTC social worker. Next stop: meet with your local or state hemophilia organization.

You can also meet with your state health officials, Medicaid director, insurance commissioner, and legislators.

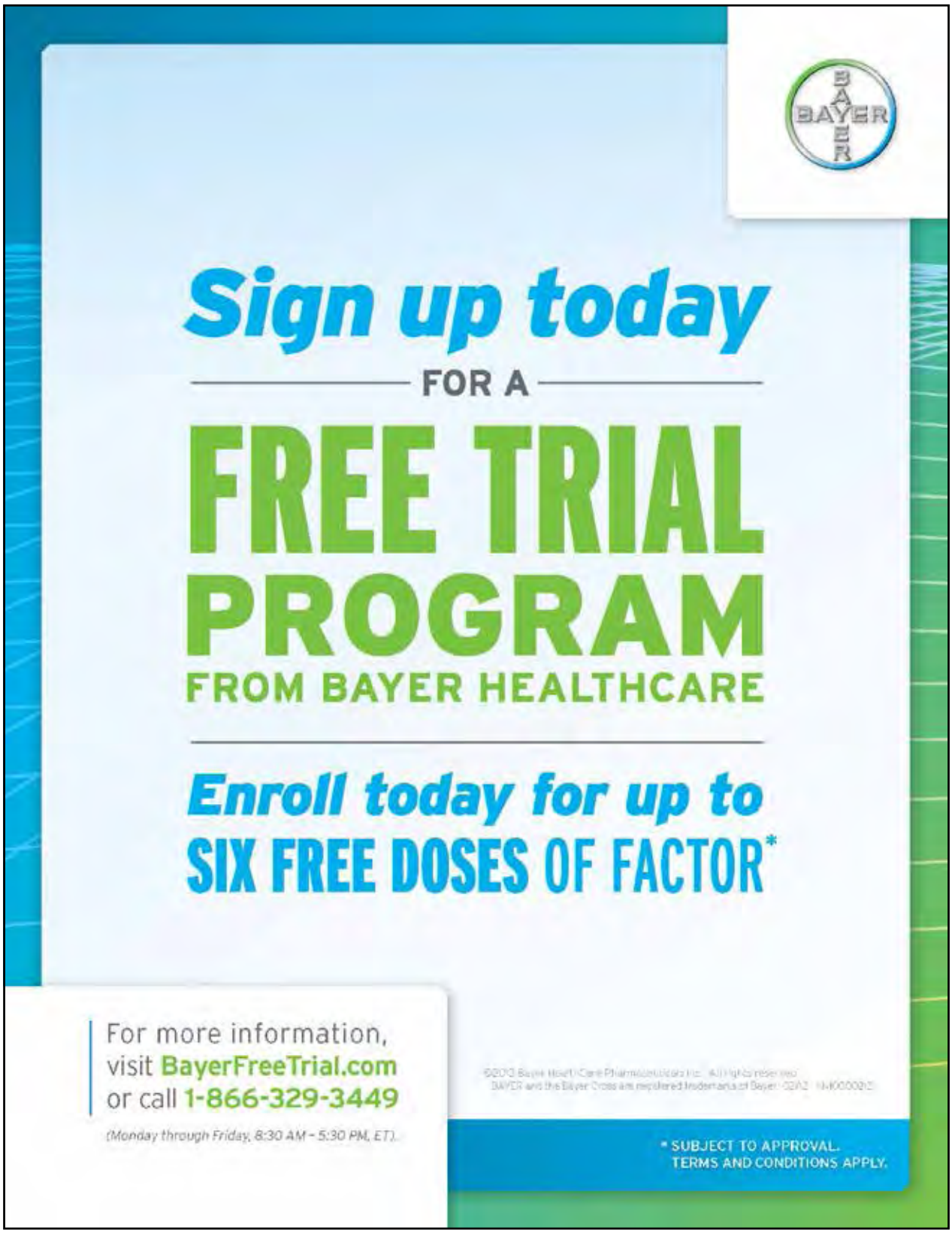
You'll need to educate these officials on inhibitors, on the importance of being able to choose therapy, and on specialty-tier and out-of-pocket cost issues. Tell your story—most state employees and representatives want to know.

Rest assured that your national organizations are working to educate payers on these issues, too. "When NHF speaks with payers, we try to address the entire spectrum of bleeding disorders," says Rice. "We are sure to include information on… inhibitors."

If you're feeling unsure about insurance terms and how to approach your payer or state representatives, ask for help. NHF, Hemophilia Federation of America (HFA), and state hemophilia organizations all have tools, glossaries, and training manuals to get you started. Make it your New Year's resolution to get informed and proactive. Netflix will still have your favorite TV shows, and you'll have more peace of mind—and, we hope, coverage.

1. Under ACA, every American must have health insurance (with exceptions). To help you choose a health insurance policy, states have constructed a web-based exchange designed to compare many different health insurance policies in your state, based on the personal parameters and financial information you provide. This allows you to find the best plan at the lowest cost that meets your health and financial needs.

2. IMS Health.



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