

HACA News

February 2004
Volume 20 Issue 1

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

Mission Statement

HACA's Vision is to improve the quality of life for persons and their families affected by bleeding disorders.

HACA's mission is to:

- ◆ Educate, support and advocate for persons with bleeding disorders and their families.
- ◆ Network with healthcare professionals.
- ◆ Increase public awareness.

Hemophilia Association of
the Capital Area
3251 Old Lee Highway
Suite 3
Fairfax, Virginia
22030-1504
(703) 352-7641
Fax (703) 352-2145
E-mail:
hacacares@aol.com
www.hacacares.org
CFC #6022

Washington Days 2004

*NHF's Annual Legislative Advocacy Conference
March 10-12, 2004*

Please make plans to join your fellow bleeding disorders community colleagues at this year's National Hemophilia Foundation Annual Legislative Advocacy Conference in Washington, D.C. This year's program will include many of the successful components of last year's program, including:

Wednesday, March 10:

Evening Reception and Issue Briefing

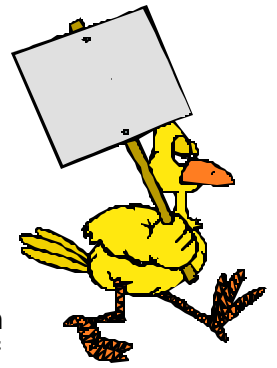
Thursday, March 11:

Breakfast and Briefing on Capitol Hill Followed by Congressional Visits

Friday, March 12:

Expanded State Advocacy Training Workshop with Stateside Associates, NHF's State Advocacy Consultants

You can register for Washington Days online at www.hemophilia.org



Chapter Survey

HACA's Board of Directors has scheduled a retreat for Saturday, March 13, 2004 to take an in-depth look at the strategic plan and any programs they might want to implement. In support of this event, a survey has been designed to elicit your input into the strategic planning process. When you receive that survey, please fill it out and return it to the HACA office by **February 16, 2004**. This will ensure that your voice will be heard in the process.

Von Willebrand Disease Workshop

A workshop for women with von Willebrand's disease has been planned for April 17, 2004 at George Mason University's Johnson Center. We will be taking a look at the genetics and treatment of the disorder, hearing from people living with the disorder, and sharing with one another. If you are a woman or teen living with the disorder or a parent of a young person living with the disorder, put this event on your calendar and plan to join us.

Golf Tournament

Our annual golf tournament has been scheduled for Monday, May 17, 2004. Golfers will tee off at one of the beautiful Ft. Belvoir courses at 2 pm. If you are a golfer, please plan to join us that day for a great day of golf. If you are not a golfer, but are interested in helping, please contact the HACA office at 703-352-7641. We need everyone's help to make this event a success.



Chapter News continued

Summer Camp

We have 15 spots reserved at the Hole in the Wall Gang Camp in Ashford, Ct. from Thursday, June 10th to Thursday, June 14th. Eligible campers must be between the

ages of 7 and 15. This year applications are available online at www.holeinthewallgang.org. If you do not have access to an internet connection and would like to receive a copy of the application, please call the HACA office at 703-352-7641. Please be sure that your completed applications are turned to the HACA office by **March 15, 2004**.

Porton Products
Genetics Institutes ReFacto*

Novo Nordisk
Nabi

Calendar of Events

- February 18 6:30 p.m.—
Venipuncture/Infusion Class at
Children's Hospital
- February 29—Blood Buddies
- March 10-12—NHF Washington
Days on Capitol Hill
- April 17—vWD Workshop at
Johnson Center, Fairfax campus of
George Mason University
- May 17—HACA Annual Golf
Tournament at Ft Belvoir

Novo Nordisk
Nabi

2004 Board of Directors Meetings

General Board Meeting
March 15, 2004

General Board meetings begin at 7:00 p.m. and are open to all interested HACA members. Because of security regulations at our meeting place, please notify the HACA office that you will be attending. Directions and site will be shared with you at that time.

Venipuncture/Infusion Classes

If you are interested in learning venipuncture techniques so you can give yourself or your child a factor, the next class will be held at Children's Hospital on February 18, 2004 at 6:30 p.m. If you are interested in taking part in this class, please call Nurse Coordinator Chris Guelchert at 202-884-3622 to reserve your spot. Additional classes are currently scheduled for June 2nd and October 20th.



Legal Alert!

Anyone who has been diagnosed with Hepatitis C as a result of their hemophilia treatment may have a claim against certain drug manufacturers. It is important for any person who is interested in pursuing such a claim to contact the attorney listed below to protect your rights. It is especially imperative for anyone in this category who has not yet reached the age of 18 to do so immediately.

Helixate S* (Mfg. by Baxter)
Stimate (for Type 1) (Mfg. by Schering-Plough)
Humate-P (Mfg. by Baxter)
ADVATE* (Mfg. by Baxter)
Proplex-T (Mfg. by Baxter)
Autoplex-T (Mfg. by Baxter)

You may contact George A Orlowitz, Esquire at (215) 545-0303 or (800) 787-5258. Mr Orlowitz has been actively involved with his local hemophilia chapter for over 17 years and will be very happy to assist you.

Scholarships Available

American Red Cross

For the tenth consecutive year, The American Red Cross is pleased to announce its scholarship program to the hemophilia community. Called HemASpheres™ University Scholarship Fund, three scholarships of up to \$5,000 will be awarded to persons receiving treatment for hemophilia or a child of a parent who is receiving treatment for hemophilia. Applications must be postmarked no later than **February 28, 2004**. Contact the HACA office at 7030352-7641 for a copy of this application.

Scholarships Available

Aventis Behring

Applications are now available for the Arthur B. Kane Memorial Scholarships, a program designed to help students with bleeding disorders pay for college, university, or vocational/trade school. The scholarships equal up to \$25,000 each. Applications must be postmarked no later than **March 15, 2004**. You can download an application at www.aventisbehringchoice.com

Factor Support Network Pharmacy

Factor Support Network Pharmacy is pleased to offer the Mike Hylton & Ron Niederman Memorial Scholarship to individuals with bleeding disorders and their immediate family members. The award will be ten \$1000 scholarships. All forms must be completed and postmarked by **April 30, 2004**. Contact the HACA office at 703-352-7641 for a copy of the application.

Hemophilia Health Services

Hemophilia Health Services Inc. will award scholarships from the Memorial Scholarship Program ranging from \$1,000 and up for persons with hemophilia and von Willebrand disease. This is also the inaugural year for the Scott Tarbell Scholarship. This scholarship will be awarded to persons with hemophilia A or B for education in computer science and math only. Completed applications must be postmarked no later than **May 1, 2004**. Contact the HACA office at 703-352-7641 for a copy of the application.

Wyeth

Wyeth is pleased to offer the Soozie Courter Hemophilia Scholarship Program for students with hemophilia A or B. Under this program, Wyeth will award fifteen \$5,000 college scholarships, two \$7,500 graduate scholarships, and four \$2,500 vocational scholarships. Completed applications must be postmarked no later than **April 15, 2004**. Contact the HACA office at 703-352-7641 for a copy of the application



Genetically Engineered Yeast Can Produce Proteins; Process could Create New Hemophilia Therapy

Dartmouth press release, August 28, 2003; Reuters Health, August 28, 2003

Bioengineers have genetically engineered yeast to produce humanized therapeutic proteins which could then be used to treat diseases ranging from cancer and multiple sclerosis to hemophilia and renal disease. In theory, any disease that has a faulty gene could be treated with a therapeutic protein.

How does a common household ingredient manufacture therapeutic proteins? Dartmouth researchers genetically engineered the yeast, *P. pastoris*, to perform a series of pathways that mimic the processing of proteins in humans. After eliminating the non-human pathways from the yeast, they inserted five genes that caused the yeast to synthesize a complex, human-like glycoprotein (or sugar).

Currently proteins for pharmaceuticals must be manufactured from living cells that are genetically engineered to produce proteins that mimic the ones synthesized by humans. It takes approximately two to three weeks to manufacture a protein using this method. With yeast, it takes three or four days.

The yeast process has been patented by GlycoFi. The company is producing therapeutic proteins directly and selling the technology to other biotech firms that make proteins. The scientists hope the technology can produce human proteins in large quantities that now cannot be mass-produced.

The process will challenge the fledgling industry of bioengineered animals. Pigs, sheep, and cows have all been genetically engineered to make human proteins in their milk.

Bloodstone Magazine, Winter 2004

New Hemophilia Therapy Begins Clinical Trial

Philadelphia Business Journal, September 22, 2003

A phase-1 clinical trial began this past September for a potential new hemophilia A treatment currently called OBI-1.

The study, conducted by Octagen Corp. and

(Continued from page 3)

Beaufour Ipsen, will take place in up to 20 clinical centers throughout the United States and the United Kingdom. Researchers will compare the safety of single infusions of OBI-1 against commercially available porcine factor VIII known as Humate-C®, which is derived from pig plasma and sold by Beaufour Ipsen.

OBI-1 is a genetically engineered molecule that was discovered by Dr. John S. Lollar, a hemophilia researcher and professor of medicine at Emory University in Atlanta. Five years ago, Octagen was founded to develop new therapies for hemophilia and other disorders related to the blood-clotting process. Octagen entered into an exclusive worldwide licensing agreement with Emory for the commercialization rights to the drug.

Bloodstone Magazine, Winter 2004

COBRA Don't Be Afraid Pick It Up!

COBRA is not a deadly snake. It is a friendly, important, "can't live without" continuation of your health insurance policy after you leave your place of employment. But you must ask for it and "pick it up". In other words, you must choose it. Don't let the premiums scare you. Just ask Patient Services Incorporated (PSI) for help before you say no to COBRA.

COBRA (Consolidated Omnibus Budget Reconciliation Act of 1986) is a government acronym for a law that allows you to take your employer's health insurance policy with you when you leave the job for any reason (other than being fired for misconduct) if they employ more than 20 persons and have a group health insurance policy. Before you depart, you need to tell your company's benefits office that you would like to have COBRA. They will then send you papers to fill out and return. You must send these signed papers back within 60 days, or you will lose the opportunity to get COBRA. After signing up for COBRA, you are required to pay the first premium within 45 days. Don't be shocked by the high cost because it could be three months worth of premiums. Call PSI, and we may be able to help you with the expense.

COBRA coverage is good for 18 months, but there are some exceptions that would allow you to keep it longer. If you turn 21 and are not a full-time student, you may keep COBRA for 36 months. If you become legally divorced or separated, you and

your children may keep your COBRA for 36 months. If the insured person dies while employed, family members may keep the COBRA for 36 months. If you become disabled within the first 60 days after you leave your job, you may keep your COBRA for 29 months. If you are approved for Medicare while working, you may keep your COBRA for 18 months after leaving your employment.

So don't be afraid of the COBRA. It only bites if you don't "pick it up" and use it. The bite it can inflict is financial ruin, medical devastation, and possible death due to lack of necessary treatment. Please remember that assistance from PSI can help you become friends with the COBRA.

Post Script Informer, Fall 2003

Editor's Note: you can reach PSI by calling 1-800-366-7741 or email uneedpsi@uneedpsi.org.

Their web site is www.uneedpsi.org.

Introducing the Patient Services Items Program

Specializing in Medically Necessary Items and Services Assistance

The October 7, 2002, U.S. Inspector General's Opinion (No. 02-14) prohibits homecare companies and specialty pharmacies from providing "free items and services," primarily to patients receiving public assistance. In response to this opinion, Patient Services Incorporated (PSI) has created and is offering a program to address the continuation of this service to patients in the bleeding disorders community.

The Patient Service Items Program (PSIP) provides much-needed services items to those who cannot afford them. PSI will receive requests and consider them independently based upon a consensus list and need. A notarized signature form will be required for legal reasons to protect all parties involved. So please cooperate with this one-time only request.

PSI has worked hard to develop a program to address the needs of those affected by this U.S. Inspector General's Opinion. We believe our program provides a more legal and fair way of addressing these necessary items and services. Presently, 80% of the "providers of service" are utilizing the Patient Service items Program.

If you are in need of an item that your homecare company can no longer provide for you, please contact Jason toll-free at (866) 367-ITEM or by mail at P.O. Box 1928, Midlothian, VA 23113, or via e-mail at PSIsp@uneedpsi.org.

Post Script Informer, Fall 2003

Insurance and Dental Procedures

By David Linney

Several times a year, I am contacted by clients with bleeding disorders who need help sorting out insurance coverage for dental services when a bleeding disorders treatment is also involved.

“I’ve got a bleeding disorder. I’m scheduled to have a dental procedure. I need a treatment. I’ve got health insurance and dental insurance. Who pays for what?”

Confusion often results from determining which insurance, health or dental, pays for what services and how, if at all, benefits are coordinated.

This article will focus on commercial health and dental insurance coverage. It will not discuss coverage through Medicaid or Medicare.

Background Information

Health Insurance provides coverage for approved health services typically provided by a health plan, including hospital, physician, diagnostic lab and x-ray, physical therapy, prescription drugs (if not covered through a drug plan), etc. Health insurance may or may not provide coverage for a very limited number of dental procedures.

- Health plans typically provide no coverage for dental checkups, cleanings, routine x-rays, simple extractions, etc. Actual coverage for dental services will vary from health plan to health plan and will be established by each plan.
- Health plan coverage for dental services may commonly be limited to one or more of the following: removal of bony impacted wisdom teeth; the removal of a number of natural teeth; and dental services for care of acute traumatic injury or cancer.

Dental Insurance provides coverage for dental services only. It does **not** provide coverage for health services.

Coordination of Benefits between Health and Dental Insurance (for dental services and a bleeding disorders treatment)

- Dental offices commonly verify dental services coverage with health plans and dental plans and submit all claims for charges as well.

The information to follow is not typically something that you have to act on, but rather it is something that is good to know.

- Bleeding disorders treatment for a dental procedure will commonly involve one or more of the following: a prescription for Stimate; a prescrip-

tion for Amicar; infusion(s) of DDAVP; or infusion of factor product.

Charges for these services should not be submitted to the dental plan. Charges should be submitted to the health plan or drug plan. The reason for this, simply stated, is that these treatments are for the care of a medical condition (i.e., the bleeding disorder). Billing for bleeding disorder treatments should be separate from the billing of dental services (i.e., two separate bills should be generated).

- Dental bills, for services that are likely covered by a health plan, should usually be submitted to the health plan first before being submitted to the dental plan.
- Dental plans may or may not coordinate benefits with health plans. More dental plans coordinate benefits than do not, but a significant number do not coordinate benefits.

Dental plans that coordinate benefits with health plans will consider payment for all covered dental services not paid or paid only in part by the health plan.

For example, if a health plan pays \$1,000 of a \$2,000 dental services claim, the dental insurance that coordinates benefits will consider payment of the \$1,000 balance under the coverage provisions of the dental contract.

Dental plans that provide dental “carveout” or supplemental benefits will consider charges only for covered dental services that are denied by the health plan. They will not consider any charges for dental services paid for in whole or part by the medical plan.

For example, if a health plan pays \$600 for dental care on two teeth for which the dentist charged \$1,000 and nothing on another tooth for which the dentist charged \$500, then the dental insurance will pay nothing on the first two teeth for which there is \$400 balance and will consider payment of the \$500 (under the coverage provisions of the dental contract) for the charges of the other tooth.

Dental plans that **do not** coordinate benefits will pay for covered dental services regardless of any medical plan benefits paid for these services.

Understanding insurance coverage for dental procedures involving bleeding disorder treatments should be helpful for those few times when you have to have a more involved dental procedure.

- from *Hemaware*, Volume 8, Issue 5, Sept/Oct 2003

Manufacturers of Factor Products

Accurate as of January 28, 2004

The following is a list of companies that manufacture factor products and the Factor VIII, Factor IX, anti-inhibitor, and von Willebrand's Disease products that they produce. The majority of products are derived from human sources, the recombinants, Stimate (high concentrate DDAVP), and Hyate:C being the exceptions (recombinant products are genetically engineered, Stimate is synthetically derived and Hyate:C is made from porcine factor). All human derived products are virally attenuated, using a variety of methods. Your HTC physician can explain these methods.

Many of the product manufacturers offer financial and other auxiliary services for consumers using their products. The names and phone numbers of the local representatives are listed for anyone interested in finding out more about the individual companies.

Company	Factor VIII Products	Factor IX Products	Anti-Inhibitor Products	Von Willebrand's Disease Products
Grifols Grifols	Alphanate Alphanate Alphanate (Off label usage.)	AlphaNine-SD AlphaNine@SD Profilnine@SD		Alphanate (Off label usage)
American Red Cross	Monarc-M (Mfg. by Baxter BioScience)	Baxter		
Aventis Behring LLC Aventis Behring LLC Bayer Corp.	Humate-P Monoclate-P Humate-P Monoclate-P Koate-DVI Kogenate FS* Koate-DVI (Off label usage.) (Mfg: Bayer, Inc.)	Helixate FS* Moberg (Inc.)	(Mfg. Stimate (for Type 1) Humate-P	Stimate (for Type I) Humate-P
Baxter Bioscience Bayer Corp. Porton Products	Hemofil-M Recombinate* Koate-DVI Kogenate-FS*	ADVATE*	Proplex-T Hyate:C	Koate-DVI (Off label usage)
Genetics Institutes Baxter Bioscience Novo Nordisk Nabi	ReFacto* Hemofil-M Recombinate* ADVATE*	Benefix*	Feiba-VH NovoSeven* Autoplex-T	Novo Nordisk
Porton Products			Hyate-C	
Genetics Institutes	ReFacto*	Benefix*		
Novo Nordisk			NovoSeven* (also used for factor 7 deficiency)	
Nabi			AutoPlex-T	

* Denotes Recombinant Product

LOCAL MANUFACTURER'S REPRESENTATIVES

AMERICAN RED CROSS

John Strasinski (610) 662-0605

AVENTIS BEHRING LLC

Gina Raymond-Duncan (800) 394-1290x6384

WYETH

Kelly Wren (703) 837-0894

BAYER, INC

Jack Shoff 1-800-246-5551X84807

BAXTER BIOSCIENCE

Kevin O'Conner (800) 777-5513x7204

NOVO NORDISK

Elizabeth Tawil (410) 349-8962

HOME CARE COMPANIES

Home Care Companies provide numerous services for persons with bleeding disorders. Though all Home Care Companies can provide the major brands of products, there are individual differences between the companies and the services they provide. As with any purchase, it is wise to compare the companies to find the one that best suits your needs. It is also recommended that you consult with your physician before making your final choice.

AHF, INC.

Shirley Moorhead (866) 243-4621

CAREMARK THERAPEUTIC SERVICES

Vickie Strange (800) 670-6782 x 3305

E BIO CARE

Carletha Gates (301) 249-3291

HEMOPHILIA HEALTH SERVICES

VA-Becky Cohn (703) 817-7707

MD-Sharon Walker (410) 296-7463

OPT CARE PLUS

Renee West (703) 499-9440

Jane Newman (703) 585-8611

ALPHA THERAPEUTIC SERVICES

John Butram (800) 552-1150

CORAM HEMOPHILIA SERVICES

Amy Judge (804) 375-3400

FACTOR SUPPORT NETWORK

Paul Brayshaw (202) 271-4252

HEMOPHILIA RESOURCES OF AMERICA

Van Warrington (302) 245-1360

NuFACTOR

Terry Stone (703) 791-2115

ADDITIONAL FACTOR SUPPLIERS

GEORGETOWN FACTOR CONSORTIUM

Profit from the Georgetown Factor Consortium helps to support the Hemophilia Treatment Center at Georgetown

Lisa Jacobs (202) 687-4861

FACTOR FOUNDATION OF AMERICA

Factor Foundation of America is a non-profit pharmacy. A portion of the profits from factor sales are directed back to the Hemophilia Community.

Dave Maderios (561) 289-3614

POSITUDES

Positudes is also a non-profit pharmacy. A portion of the profits from factor sales are directed back to the Hemophilia community.

John Degiorgio (804) 789-1368

Prophylaxis Q&A Compilation

By Mary G. Hudson, RN et al.

A collaboration of nurses has come up with some of the most commonly asked questions concerning prophylaxis.

1. What is prophylaxis in the treatment of hemophilia?

The term "prophylaxis" is defined as treatment provided as a preventive or protective measure. With regards to hemophilia, prophylaxis entails routinely infusing clotting factor to keep factor levels high enough to prevent most, if not all, bleeds. The goal is to keep factor levels above 1% to prevent joint bleeding and, hopefully, prevent joint damage.

Primary Prophylaxis: Prophylaxis treatment started before bleeding begins or before a joint bleed has occurred.

Secondary Prophylaxis: Prophylaxis treatment started after some joint bleeds have occurred, but before a "target joint" has been established. A target joint occurs when repeated bleeding occurs in the same joint over a short period of time. The Center for Disease Control and Prevention (CDC) definition of a target joint is = four bleeds into the same joint over six months or = 20 bleeds in the same joint in a lifetime.

Event-Related Prophylaxis: Prophylaxis treatment before a specific event, such as sports activity or school trip. It can also be before certain procedures such as surgery or dental work.

THE CANDIDATE FOR PROPHYLAXIS

2. Who is a candidate for prophylaxis?

Individuals with severe hemophilia A or hemophilia B (factor activity level = 1%) or with moderate hemophilia (1% to 5% factor activity level) who have frequent bleeding episodes may be candidates for prophylaxis. If you are unsure or would like to find out if prophylaxis is the right treatment for you or your child, it is best to contact your hemophilia treatment center (HTC) or hematologist.

3. What are the advantages and disadvantages of prophylaxis?

Determining risks and benefits of any treatment option is important when deciding whether the option is right for you or your child. Some of the advantages and disadvantages of prophylaxis are:

Advantages

- Reduces bleeding and decreases the potential for joint damage;
- Allows for a "normal" lifestyle;
- Family and patient have more control over their hemophilia;
- Reduced anxiety when leaving children in day care, school or with a babysitter;
- Participation in a greater variety of physical activities;
- Children become familiar with infusion techniques;
- May reduce time spent in hospitals due to procedures related to bleeds/surgery;
- May be more cost-effective in the long term.

Disadvantages

- Requires adherence to established plan;
- Usually requires long-term commitment;
- Individuals may ignore early signs and symptoms of bleeding episodes;
- More frequent "pokes";
- Venous access may be a concern;
- Intravenous access device (IVAD) management and related problems;
- May give family false sense of security;
- Cost.

4. Will my child still bleed?

Yes. Factor levels peak and fall during prophylaxis. Depending on the patient's actual factor level and physical activity, he may have a bleed. Prophylaxis is intended to prevent spontaneous bleeding episodes, though bleeding episodes may still occur as a result of injury. These episodes, referred to as breakthrough bleeds, should be reported to the HTC.

5. How would I recognize a bleed?

Recognizing a bleed while on prophylaxis is a learned process. Most bleeds result in pain, and eventually, restricted use of the bleeding site (either a joint or muscle.) Some people describe their bleeds as a "tingling, bubbly or butterfly" feeling when the bleed is occurring. Swelling, redness and/or warmth at the bleeding site may occur. If your child is irritable, limping, dragging his foot or leg or favoring one limb, check to see if bleeding may be

present. Always contact the HTC whenever breakthrough bleeding is suspected.

6. Will prophylaxis give my child a “normal” life-style?

Prophylaxis will afford the opportunity for a child and parent to have more stable lives. Prophylaxis does not “cure” hemophilia, though it can decrease the complications from repeated bleeding when used appropriately.

7. Will my child be able to go out and do anything and everything?

No. High-risk activities that lead to bleeding, such as contact sports, should still be avoided for people on prophylaxis. Safety measures, proper fitting equipment and supervision are still necessary.

8. How long can my child stay on prophylaxis?

The length of time varies for each person and that is a decision that is made by the HTC staff and family. Many children stay on prophylaxis through their adolescent years.

9. What is the risk of developing an inhibitor when on prophylaxis?

An inhibitor is an antibody to the clotting factor concentrate. Some individuals (about 20% with severe hemophilia) will develop an inhibitor, but most won't. Many treaters believe an inhibitor will appear within the first nine exposure days to factor concentrate. Being on prophylaxis does not increase the risk of developing an inhibitor.

It is important to monitor bleeding episodes while on prophylaxis. Increased bleeding or breakthrough bleeding may mean an inhibitor is present or the activity of the clotting factor being infused is inadequate. (Recently this has been seen with one of the recombinant factor VIII products.) The HTC should be called for further evaluation if bleeding occurs.

10. What if my child currently has an inhibitor?

If you or your child currently have an inhibitor, your physician will develop a plan for treatment. This may include treating with higher and more frequent prophylactic doses. People with inhibitors have also been on prophylaxis with anti-inhibitor concentrates. Check with the HTC to see if this may be an option.

VENOUS ACCESS

11. What is venous access?

Venous access is a term that refers to how and where factor is actually infused into the body. Because prophylaxis requires frequent “pokes”, good access is very important. Access is generally through venipuncture using a butterfly needle. If venous access is a challenge, an IVAD may be used. Examples of IVADs are:

- Port-a-cath
- Peripherally Inserted Central Venous Catheter (PICC) line

12. What is port placement and is my child a candidate?

A port is a small IVAD that resembles a drum with a soft flexible tube attached. During surgery the port is placed at the chest with the tube going into a large vein leading to the heart.

A child may be a candidate for a port if he has hard-to-find veins and needs to infuse frequently.

Parents should have adequate time to learn about ports. Special teaching and care are required whenever a port is used. An HTC will help determine if a port is the right choice.

Advantages

- Does not require a “poke”;
- Can be used to draw some laboratory tests;
- Easily accessible.

Disadvantages

- Requires surgery for placement;
- May get “line” infections;
- May get catheter-related clots;
- Proper care required with use;
- Often will “outgrow” the line and need a new one;
- Must take antibiotics before some types of dental work.

13. What are the advantages and disadvantages of an external central venous catheter (e.g. Broviac or Hickman)?

An external central venous catheter is another type of alternative access device. Like a port, a catheter is usually placed in the chest area. Instead of being under the skin, external catheters extend outside the body. These devices also require special teaching and care.

(Continued on page 10)

(Continued from page 9)

Advantages and disadvantages of external venous catheters are as follows:

Advantages

- Does not require a poke;
- Can be used to draw some laboratory tests;
- Easily accessible;
- Able to be repaired if torn;
- Can be a needleless system.

Disadvantages

- Requires surgery for placement;
 - May get "line" infections;
 - May get catheter-related clots;
 - Can get pulled out accidentally;
 - Proper care required with use;
 - Must change dressings/caps using proper technique;
 - May be considered cosmetically appealing.
 - Unable to swim in lakes;
 - Must take antibiotics before some types of dental work.
- An HTC can provide information about external venous catheters.

EXPECTATIONS

14. How will this affect our daily life?

Prophylaxis can be a major change in a patient's daily life. For some families, prophylaxis will be a rewarding experience while others may find it adds stress to their lives. Taking a close look at the responsibilities that go along with prophylaxis can help a family decide if prophylaxis is an appropriate treatment option.

15. What will be the parents' responsibility?

- Learn proper infusion technique;
- Appropriate care of venous access device (when applicable);
- Learn to recognize and treat a bleed if one occurs;
- Compliance to treatment schedules – same time each day for best results;
- Keep current and accurate records;
- Responsible storage, use and disposal of factor concentrate and related supplies;
- Report follow up HTC visits to evaluate the treatment and education;

- Report all breakthrough bleeding and all fevers > 101 degrees to HTC;
- Keep insurance information up to date.

16. What will be the child's responsibility?

- Tell an adult whenever there is a bleed;
- Help with setting up infusion supplies and record keeping;
- Keep venous access device safe and clean;
- Wear appropriate safety equipment;
- Avoid high-risk activities that may lead to bleeding;
- At an appropriate age, learn to self-infuse.

17. What are the financial issues related to prophylaxis?

Occasionally, lifetime insurance maximums/caps can be exceeded with prophylaxis. Knowing insurance coverage and lifetime cap will help determine if cost will be a problem should one decide to begin prophylaxis. A simple example of how prophylaxis may affect a lifetime maximum is as follows:

Factor IX Anti-Inhibitor Von Willebrand's Disease

Alphanate Alpha-Nine@SD Profiline@SD

Helixate FS* (Mfg. Stimate (for Type 1) Bayer, Inc.) Humate-P

ADVATE* Berlex-T

Hyate:C

Benefix*

Cost of factor/unit
 X # units/dose
 X # of treatments/week
 X 52 weeks/year
 = annual cost of prophylaxis

18. Will there be issues with my insurance?

Insurance companies are different and may need to be contacted before beginning prophylaxis therapy. Be sure to talk with HTC staff if there are concerns. Prophylaxis, while expensive, may actually be more cost effective than episodic treatment. Most times the HTC can help with insurance company issues before problems arise with justification of a prophylaxis treatment plan and the cost of factor.

OTHER RESOURCES

19. What reading materials are available on prophylaxis?

There have been some articles and booklets written about prophylaxis. For information, contact NHF's information service, HANDI, at 800-42-HANDI. HTCs will have a list of reading materials that may be helpful before deciding on prophylaxis as an option. One excellent book is *Raising A Child With Hemophilia* by Laureen Kelley.

-from *Hemaware*, Volume 8, Issue 5, Sept/Oct 2003

Transgenic Pigs – A Factory for Factor Production

By Judy Berlfein

“Do you prefer your factor by intravenous infusion or sipped slowly from a glass of chocolate milk?” That’s the question William Velander of Virginia Polytechnic Institute and State University hopes to ask the children of Latin America who have hemophilia. And if all goes according to plan, he’ll respond to their desires.

For 17 years Velander and his colleagues have been developing methods of producing large quantities of factor. The key, Velander repeats several times in an interview, is abundance. If we have enough factor, he believes, we can provide it to all who need it at an affordable price. Using today’s methods of generating recombinant factor, quantity remains an issue. But if Velander’s methods are proven sound, scarcity will no longer be a stumbling block.

What’s the trick? With nearly two decades of research on the project, Velander has successfully inserted the gene for factor IX (FIX) into developing pig embryos. When the pigs mature, the female pigs are able to synthesize large quantities of FIX in the mammary glands. According to Velander, each liter of milk produced by the pigs contains up to a million units of activity, far exceeding the levels found in today’s recombinant preparations. These animals have become an efficient factory for FIX. “Factor purified from the milk of 10 to 20 animals could provide hemophilia B treatment for all of Latin America,” Velander asserts. Velander does not envision children drinking milk straight from the animal. Once purified, the protein could be reconstituted for an intravenous infusion, similar to the approach used with today’s recombinant factor. However, another possibility exists that Velander and Oral Alpan of the National Institute of Allergy and Infectious Diseases have begun to study. It may be feasible to mix purified factor with pasteurized cow’s milk or any other desired drink and provide it as an oral treatment.

Independent teams of researchers in Japan and the Netherlands demonstrated two decades ago that factor could be given orally. The large protein successfully navigated the harsh conditions of the gut and made it safely to the bloodstream. Velander says the experiments weren’t pursued further because the protein was not available in sufficient

quantity to study. “But now abundance is no longer an issue,” he states. With nearly unlimited FIX available from his pigs, Velander and colleagues plan to complete the work initiated by the Japanese and Dutch scientists.

At first glance, many are skeptical about the possibility of oral administration. It sounds a bit too simple. Velander notes that proteins won’t routinely pass through the gut, especially if they’re taken on an empty stomach. When food is absent, the high acid content of the stomach and enzymes designed to destroy proteins in the intestine will quickly break proteins down into constituent parts. However, if the preparation is taken after a full meal and inhibitors are used to discourage enzyme activity, the odds of pushing intact protein into the circulation improve. Milk, he comments, provides a useful vehicle for carrying factor, as it contains casein, a natural inhibitor of enzymes aimed at proteins. Other foods, such as soy, also have natural inhibitors, and could be a favorable addition to the factor mixture.

Velander still has several hurdles to cross. He has completed preliminary experiments in healthy dogs, feeding them milk from his pigs and achieving measurable FIX levels in the blood stream. The next step is to purify the factor and administer it intravenously to hemophilic dogs to determine if it is effective in correcting the bleeding disorder. All of this requires funding. Agreements are in the works with the ministry of health of the Brazilian government to provide that support. The ministry would administer the experiments in animals and conduct the ultimate trials in humans. If therapy is successful, they would then market it throughout Brazil and other Latin American countries.

Velander dreams big. But the mantra of all research applies to his plans as well. Perseverance, time and good luck will determine if his dreams come to fruition.

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Guidelines for Emergency Department Management of Individuals with Hemophilia
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MASAC Document #155

Individuals with bleeding disorders who present to an emergency department for care may not receive appropriate, expeditious management. Therefore MASAC has developed guidelines for that appropriate care.

You can receive a copy of the guidelines by visiting NHF's web site at www.hemophilia.org or by calling the HACA office at 703-352-7641.

A Powder to Halt Nosebleeds Quickly

By Dawn Wotapka

Newsday (New York), January 20, 2004

The product and what it is marketed for: NosebleedQR is a topical powder that bonds with blood to halt bleeding almost instantly.

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Users open a blister pack and load a swab with the brown powder—a blend of hydrophilic polymer, a water-absorbing resin used for food processing and water purification, and potassium salt, a binding agent. If the wall between the nostrils is covered with QR, a protective, flexible scab that mimics a natural one forms. The faux scab is a Hematrix, the trademarked name for the unique barrier, a “web that forms on top of the blood.” according to Doris E Goodman, vice president of marketing for producer Biolife LLLC of Sarasota, FL.

A similar product, UrgentQR, contains the same ingredients but is poured onto cuts. KidsQR (quick relief) is a gentler version, Biolife said.

Florida scientist James A. Patterson accidentally discovered the product in 1999. While exploring water purification, he slashed his hand on a glass shard. He noticed the potassium salt covering his hands clotted the blood.

The solution was first called Eco-cure, but the name was changed after the Food and Drug Administration objected to labeling the nascent product a "Cure."

Since then, the company has worked to make Nose-bleedQR as common as gauze giant Band-Aid. QR is considered an over-the-counter bandage, but Biolife asked the FDA to permit stating that the product kills bacteria, is usable by those with bleeding disorders and promotes healing. Goodman expects permission to use the bacteria promise in the next few months, while touting healing could take two years.

To prove the product's effectiveness, in 2000 Biolife conducted a study in a Venice, FL, emergency room environment of 45 patients with a range of "frequently encountered wounds on different parts of the body." Forty-three patients' bleeding stopped within a minute, including seven of the eight with a bloody nose. Of that, 37 percent stopped within five seconds of application, 33 percent ended within five to 15 seconds. Thirty percent ended within 15 to 60 seconds.

The powder is manufactured in compliance with FDA Quality System Requirements, and testing showed the product is nontoxic and safe for external use, Biolife added. However, it should not get into the eyes or in the posterior nasal cavity, and the powder should not be inhaled or ingested.

In its February issue, *Consumer Reports* magazine labels the product a "quick solution to an occasional messy problem." But it points out that if bleeding is severe, frequent or coming from both nostrils, a doctor should be consulted. The magazine says more peer-reviewed studies are needed to confirm the studies' conclusions.

Dr Brian S Skrainka, a pediatric ER doctor in Indiana, agrees that the product seems to work very well. He said the product, although slightly messy, is well tolerated, including by those with bleeding disorders, and has prevented more severe procedures. "When other measures failed, I was able to...stop the bleeding," he said. "This obviously would save moms and dads from making a trip to the ER."

I'll Bet You Didn't Know...

Interesting and Totally Useless Information

- There are more than 200 chili pepper varieties, none of which belong to the pepper family.
- Iron weighs more after it rusts because the materials are combined with oxygen.
- Man is not the only creature that favors his right side over his left side. Elephants are believed to be "right-handed" as well, since most of them wear down their right tusk more than their left tusk.
- The geographical center of the North American continent is Rugby, North Dakota.
- Egypt wasn't the only place that produced mummies. The original natives of the Canary Islands, the Guanaches, were adept at mummifying. Mummies have also been found in the high mountains of Chile.
- In the course of a year, a man is likely to shave off a pound of whiskers.
- The greyhound has the best eyesight of all breeds of dogs.

Taken from Chantilly Printing and Graphics' *Business Fourm*, December 2003

"A man always has two reasons for doing anything; a good reason and the real reason."

____ John Pierpont Morgan



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