

HACA News

February 2003
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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.

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Washington Day—March 5 – 7

The National Hemophilia Foundation will be hosting Washington Day, the annual legislative advocacy program in Washington, D.C. on Thursday and Friday, March 6th and 7th. With the exception of legislative visits on Capitol Hill on Thursday, most of the sessions will be held at the Washington DC Marriott located at 1221 22nd St NW. HACA will be hosting the reception at the NHF training session for all attendees on Wednesday evening, March 5th.

The schedule for the sessions is as follows:

- Wednesday evening, March 5th, from 6:30 to 9 pm—light supper and briefing on talking points and how to make a legislative visit. There will also be time for groups to plan their visits on Capitol Hill.
- Thursday, March 6th. The day will be spent on Capitol Hill visiting members of Congress. That evening NHF will host a reception at the Marriott for all participants.
- Friday, March 7th. 8am- 2 pm—NHF will host a special training on state-based advocacy issues. NHF has spotted trends that include reduced reimbursement from Medicaid, insurance and access issues similar to the recent Children's Hospital and CareFirst stand-off, and State Medicare directors moving to a single provider. Many of these issues will require local organizations to get involved and educate state legislators. This session will be designed to help facilitate readiness.

If you are interested in making visits on Capitol Hill and/or attending the state advocacy training, please notify NHF's Director of Government Affairs, Cheryl Hayden at 212-328-3736 or email her at chayden@hemophilia.org. Please let Cheryl know of your interest by **February 21, 2003** so proper arrangements can be made for legislative visits. Please also advise the HACA office (703-352-7641) of your intention to participate.

Save the Date

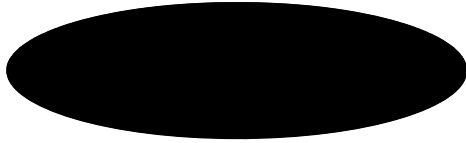
HACA's annual golf tournament has been scheduled for Friday, May 16, 2003 at the Ft. Belvoir Golf Club. We will be looking for people to help us recruit golfers, recruit sponsors, solicit items for our silent auction, and serve on a committee to organize the event. If you are interested in helping with this event, please call the HACA office at 703-352-7641.

Family Retreat

A family retreat is being planned for the weekend of February 21-22 at the Marriott in Tysons Corner. Attendance will be limited to the first 20 families. Call 703-352-7641 to reserve your space and watch the mail for more details.

Summer Camp

Applications for summer camp at the Hole in the Wall Gang Camp have been sent out to the prospective campers that we are aware of. We have 10 spaces at the first session, June 7-14. If your child is between the ages of 7 and 15 and is interested in camp, and you did not already receive an application, please notify the HACA office at 703-352-7641. Applications must be returned to the HACA office by **March 15th** in order for a child to be considered.



Congratulations, Dr. Kessler

HACA extends sincere congratulations to Dr. Craig Kessler who received the Ken Brinkhas Hemophilia Physician of the Year award at the NHF meeting in Orlando. The Ken Brinkhas Hemophilia Physician of the Year award honors distinguished physicians who have made major contributions to the advancement of care for individuals with bleeding disorders.

Board Members Elected in October

At our annual meeting held on October 19, 2002, several people were elected to HACA's Board of Directors. Those newly elected members include: **Keith Bushey**, Chief Safety Officer at George Mason University; **Amr El-Beshir**, a psychologist who works with middle school students in Prince George's County; **Nina Duggan**, (HACA's Secretary) mother of two affected young people and insurance specialist; **Sean Kevelighan**, public relations specialist with Hill Knowlton and former Legislative Director for Bob Shaffer (CO); **Anastasia Lee**, nurse coordinator at Georgetown Hemophilia Treatment Center; **Julie LeFevre**, mother of affected young person and Human Resources Director at HHS; **Richard Riccardelli**, (HACA's Treasurer) employed by Computer Services Corporation; and **Jack Shoff**, pharmaceutical company representative.

They join HACA's previously elected board members: **Susan Yamamoto** (President), **Paul Brayshaw** (Vice President), **Michael Garfield**, **Stephen Kulenguski**, and **James Romano**.

HFA Annual Meeting

The Hemophilia Federation of America will be holding its annual meeting in Houston, TX, March 28-30 at the Marriott Houston-Intercontinental Airport. HACA will be offering limited scholarships. A maximum of \$500/family and \$350/ individual will be awarded. Please contact the HACA office at 703-352-7641 for a funding application. Your application must be returned to the HACA office by **February 20th**. You must be registered for the annual meeting by March 12th. The HACA office also has a copy of registration information or the form may be downloaded at www.hemophiliafed.org.

Thanks to Former Board Members

Each time we elect people to the Board of Directors, we must also bid farewell to others who have served the HACA community for at least two years and, in many cases, to those who have served for four years. Accordingly, we extend a huge thank you to the following individuals for their years of dedicated service to HACA: **Pam Bowers Anderson**--Pam served as HACA's Treasurer for four years. **Miriam Goldstein**--Miriam served on the board for 4 years. During that time she helped organize three educational seminars, served on a committee to update our by-laws, and wrote several position papers for issues confronting the bleeding disorders community including Social Security proposals and HOPPS issues. She has continued that service to HACA by drafting letters for HACA regarding the current CareFirst/Children's situation. **Linda Johnson**—Linda served on HACA's Board for two years. During that time, she organized two very successful golf tournaments to benefit HACA. **Janice Farmer**—Janice Served on HACA's Board for nearly two years until her retirement. Janice is currently completing a project to send copies of the chapter's brochure to public libraries in the area.

We're very grateful to these individuals for their gifts of time and for all they helped the organization to accomplish.



Calendar of Events

February 21-22	Family Retreat-Marriott at Tyson's Corner
March 5-7th	NHF Washington Day
March 28-30	HFA Annual Meeting-Houston, TX
May 16	Dc Hemophilia Open--Ft Belvoir Golf Club

Soda Project

Did you know that by placing a soda machine in your place of work, you could help to benefit HACA? HACA has a special arrangement with Dominion Vendors of Virginia Beach. We just notify Dominion Vendors about a company that might be interested in placing a soda machine and they take it from there. They talk to the company and if the company is interested and supports enough traffic to meet the minimum guideline of selling eight sodas a day, Dominion Vendors makes the arrangements to have the soda machine placed and serviced.

50% of the commission is donated to HACA and the other 50% of the commission is shared between Dominion Vendors and the company hosting the machine. The host company is able to deduct their portion of the profits given to HACA as a charitable donation.

HACA currently has such an arrangement with Cradle to Crayons Pre-School in Manassas. Their machine was placed in April of 2000 and has generated donations of \$841.50. No one has had to plan a special event or pay an entrance fee to receive that benefit. What could be an easier way to benefit the organization that benefits you? Please check with your office manager, your Pre-School Director or the manager of a new office building in your area as to their interest and notify the HACA office at 703-352-7641. We'll take it from there!

Sincere Sympathy

HACA extends our sincere sympathy to several families who have recently suffered the loss of a beloved member of their family. **Paul Nirdlinger** passed away on November 2nd. **Mark Leverone** passed away on November 4th. **Wallace Keene**, father of Melissa Hurtt and Chrissy Wick passed away on December 20th, and **Frances O'Brien**, father of Kathy Krug and Eileen Prohett passed away on New Year's Day.

May you all find comfort in your good memories of your loved one and strength in the care and support of family and friends.

Mark LeRoy Leverone—1953-2002

Mark Leverone was a kind, good, and loving man. Always thoughtful and caring, he continued to put others first, even as his own health declined. He bore years of illness with courage and grace and kept his sense of humor to the very end.

His wife, his parents, his grandmother, his sister and brothers and his friends will always be grateful to have had Mark as such a wonderful and special part of their lives.

God bless you, Mark.

Written in loving memory of Mark by his wife, Lucy McCoy

2003 Board of Directors Meetings

General Board Meeting
March 17, 2003
Executive Board Meeting
TBA

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.

Hemaware Magazine Seeks Your Input

Michael Coffino, editor of NHF's magazine, *Hemaware*, seeks input from any interested party for the following stories:

May/June Due 2/21/03

It is shown that exercise helps prevent joint problems. What's your favorite form of exercise and why?

July/August Due 3/28/03

What has been the most significant life transition you have experienced?

September/October Due 6/06/03

When hemophilia is cured, how will people in future generations describe the disorder?

November/December Due 8/01/03

What are the key issues that advocacy should focus on for those with bleeding disorders?

You can send your comments to: Michael Coffino, Editor, National Hemophilia Foundation, 116 W 32nd Street, 11th Floor, New York, NY 10001 or email your comments to: mcoffino@hemophilia.org.

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*Happy
Valentine's
Day*

*(Continued from page 3)***Scholarship Available**

AHF, Inc. announces the applications for the Beth Carew Memorial Scholarship are now available. AHF will award five (5) scholarships to full-time registered undergraduate students in good standing or entering freshmen, each in the amount of \$2,000. Awards are restricted to young adults and adults who have hemophilia, von Willebrand disease, or another related inherited bleeding disorder. Scholarship awards are designed to support full-time students entering or attending any accredited college or university in the United States.

Applications must be received at the AHF corporate offices no later than April 4, 2003 and awards will be announced by May 19, 2003. Application forms may be downloaded at www.afhinfo.com.

**Industry News****Monarc-M™ Available in High Potency Assay**

October 15, 2002

The American Red Cross announced that Monarc-M™ is now available in assays ranging from 1,500 to 2,000 International Units per vial. By extending the product's activity range, patients are less likely to require more than one vial of product when infusing.

Baxter Verifies Viral Inactivation of West Nile Virus

November 5, 2002

Baxter Healthcare Corporation presented the first data that verifies the inactivation of West Nile Virus (WNV) during the processes routinely used in the production of its plasma-derived therapeutics. WNV is a member of the *Fleoviridae* family of viruses and its structure is similar to previously identified viruses such as Hepatitis C. This family of enveloped viruses can be readily inactivated through proven inactivation processes such as pasteurization, vapor heating and solvent detergent treatment. Baxter used an infective assay with a 1999 New York isolate of the virus and confirmed that WNV was readily inactivated.

U.S.FDA Approves Wyeth Manufacturing Site for Hemophilia A Treatment

December 19, 2002

The U.S. Food and Drug Administration approved Wyeth's facility in St. Louis, Missouri, to manufacture its

recombinant factor VIII product, ReFacto®. This approval will allow Wyeth increased flexibility to meet the global demand for ReFacto®.

Baxter to Acquire Plasma Collection Centers from Alpha Therapeutics Corporation

December 20, 2002

Baxter Healthcare Corporation announced that it has entered into definitive agreement to acquire all of Alpha Therapeutic Corporation's plasma collection operations, which include 42 FDA-licensed plasma collection centers in the United States, and a central testing laboratory.

Baxter will also obtain Alpha's Alpha-1 antitrypsin (A1P1) product, which is currently undergoing FDA review, for the treatment of hereditary emphysema. Closing of the transaction is subject to approval by regulatory authorities. This transaction is expected to close in the first half of 2003.

NovoNordisk Announces Price Increase

January 6, 2003

NovoSeven® Coagulation Factor VIIa (Recombinant) will increase in price effective January 6, 2003. The suggested list price will increase from \$1.40 per microgram to \$1.47 per microgram.

Update on West Nile Virus: Blood Centers Initiate Voluntary Withdrawal of Frozen Blood Products

NHF Medical Advisory #395

December 20, 2002

From May through October 2002, 13 cases of meningoencephalitis, an inflammation of the brain and its membrane, due to West Nile Virus (WNV) have been confirmed to be the result of infection transmitted by blood transfusion or organ transplant. During this time period, surplus units of cryoprecipitate and fresh frozen plasma (FFP) were stored to be distributed at a later date. At present there is no licensed test to detect the presence of WNV in these units.

On December 12, 2002, the blood collection industry initiated a voluntary withdrawal of these frozen, untested units of cryoprecipitate and FFP. This withdrawal involves units collected and stored by the American Association of Blood Banks, America's Blood Centers, and the American Red Cross.

For the complete transcript of this Medical Advisory, please visit NHF's web site at www.hemophilia.org.

The Latest Findings in Gene Therapies

By David Page

The U.S. National Hemophilia Foundation (NHF) held its Fifth Workshop on Gene Therapies for Hemophilia at the Children's Hospital of Philadelphia, on April 12 and 13, 2002.

The workshop brought together researchers from the U.S., Canada, and Europe to present their latest findings in gene therapy and bleeding disorders. The scope of the 47 papers presented during the two days was very impressive. Dr. Margaret Ragni of the University of Pittsburgh Medical Center identified the challenge for gene transfer therapy: effective treatment which avoids the disabling complications of past innovations. The ideal therapy would be effective in a single dose, would not create inhibitors, would be non-toxic and non-infectious, and would be affordable.

Ongoing Trials

Dr. Amy Patterson of the U.S. National Institutes of Health (NIH) reported that of the 522 research protocols reviewed by NIH, only five were on hemophilia – three on factor VIII and two on factor IX. All of these are in Phase I clinical trials, during which the therapy is tested for harmful responses with a very small number of patients. Only at Phase II and Phase III are therapies tested for their effectiveness in treating the target disease.

Two updates on these clinical trials were presented. Dr. Gilbert White of the University of North Carolina at Chapel Hill presented the first update on a trial which uses a gutted adenovirus vector (a modified virus used to deliver the desired gene to the proper cells in the body) to insert the factor VIII gene into the liver. One patient has received this experimental therapy with the result that factor VIII levels rose to 1 – 1.5 percent of normal. However, the patient developed an inflammatory response to the vector, with high fever and increased liver enzymes. This trial was placed on hold but will not continue with lower doses. Another concern with this vector is the development of circulating antibodies to the vector, a situation which makes repeat treatment much more difficult.

Dr. Bertil Glader of the Stanford University Medical Center in California presented an update on another clinical trial, which uses a recombinant adeno-associated viral (rAAV) vector to deliver the factor IX gene into the liver via the hepatic artery. He presented early results from a Phase I clinical trial with two patients. Both patients received low doses of the gene therapy, and liver, kidney and blood tests remained normal. Factor IX levels rose slightly to around one percent and no inhibitors to factor IX were detected. However, the rAAV vector was found in the semen of both patients. Although there is no evidence

that the vector can be transmitted from one generation to the next, this highlights the need to take great precautions against potential germline transmission. In the first person, traces of the vector disappeared after 10 weeks. In the second, it was still present after eight weeks. The next stage of the trial, in which four patients will receive a higher dose, will not go ahead until all signs of the vector in the semen have disappeared.

These two early clinical trials represent only a fraction of the data presented at the workshop. Participants learned of a wide variety of research using mice and dog models, different vectors, such as the lentivirus (a form of HIV which has been modified so it cannot be reproduced) and different modes of delivery. Some show great promise while others point out potential dangers.

One case was discussed where a patient (who was not in a hemophilia trial) received a very low dose of the adenoviral vector and died as a result of his body's immune response. This highlights the danger that gene therapy may carry a severe risk of toxicity at a level that does not provide therapeutic benefit. As one immunologist warned, "You are working against an excruciatingly sensitive immune system designed to fight against just the kind of invasion you are engineering."

Alternative Approaches

Other presentations, however, suggested that genetic research with entirely different approaches may yield positive results.

Dr. Steven Sommer of the City of Hope Medical Center in Duarte, California, described "translation bypass therapy" in which small-molecule drugs, taken orally, can be used to bypass factor VIII or factor IX deficiencies in individuals with a nonsense gene mutation – about 10 percent of people with hemophilia. This holds special promise for people in developing countries as the treatment would be both low-cost and easy to administer.

Dr. Steven Pipe of the University of Michigan described research in which genetic manipulations of the factor VIII gene, either in the manufacturing process or in gene insertions, could lead to benefits – increased secretion, longer half-life, and increased specific activity of factor VIII.

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The Orlando Report

HACA was able to help 7 individuals/ families attend the NHF annual meeting in Orlando. The following are highlights of the summaries submitted by the DeArmon family, the LeFevre family and by David Stone. David has also submitted a wonderful full-length report containing his notes from every session he attended and pictures of many of the events he took part in. If you would like a copy of his report, please contact the HACA office at 703-352-7641.

Jim DeArmon

Jim reported attending six sessions including Partners in Care: Enhancing Long-term Outcomes; Opening Session: Focus on Research—Past and Future; NHF Research Fellows and Providers Roundtable; National Prevention Program: Prevention Teams Report Out; Point/Counterpoints: Prophylaxis, Ports and Sports; and Nursing Case Studies.

Jim reported that the most helpful session for him was the session on Nursing Case Studies because the session was quite interesting and gave him new perspectives on a variety of coagulopathies and nurses' reaction to them. He found the Fellows Roundtable the least helpful session he attended because, while the session was very interesting, most of the information was beyond his understanding.

The highlight of the Annual Meeting for Jim was discussing new meds and approaches to care with medical and vendor personnel. Jim shared that he enjoyed being in an environment where there is complete understanding and much sympathy for hemophilia patients and parents.

Rick, Julie, James and Tori LeFevre

Julie writes: I am the type of person who learns only enough to make it through the current crisis—then learn enough for the next and so on—(like when taking a test in school—study for that test—take the test—get an A and then promptly forget 70% or so of what was learned). When I was first told about James' condition—I used this same learning process. I knew that the resources were out there should I need to learn more and I knew my husband was retaining some of what we needed to know as well.

Rick and I haven't made a really big issue out of James' hemophilia and have not educated him about what *his medical condition really is*. We know that he is the one who will deal with this only a daily basis when he grows up, but we felt we were helping James by not bogging him down with all the medical issues and limitations that come

with having hemophilia. One of the sessions we attended touched on this very issue so I gave James a "test on hemophilia and here is how it went—

1. What kind of hemophilia do you have? "Factor 8" (correct)
2. What does that mean? "I don't know."
3. What happens when we give you factor? "It gives me more blood."
4. What is factor? "A medical thing."
5. What is your port for? "To take some of my blood out and give me Factor 8."
6. Why is it important that you get factor? "So my blood clots."
7. What does clot mean? "I never looked blood up."
8. Well, what does it mean to you? "It doesn't work—bleeding".
9. What type of hemophilia do you have—mild, moderate, or severe? "Severe" (correct)
10. What does severe mean? "I don't know—I guessed."
11. Do you know what a target joint is? "No"
12. Do you know what a joint is? "Yes, I think it's in your neck"—then pointed to his forearm and said, "I think it is here."
13. Do you have more than one joint? "No, only one."
14. Where did you get hemophilia? "Virginia"
15. How? "I don't know, I was born with it."
16. Does everybody have it? "No"
17. Will you have hemophilia all your life? "Yeah, I mean, no—yes"
18. What do you mean? You will or you won't? "Yes, I will."
19. (After explaining the definition of cure) Do you think there will be a cure for hemophilia in your lifetime? "Yeah"
20. Do you like having hemophilia? "Yeah"
21. Why? "A couple of years ago you said if I don't cry you would give me Pokemon cards—can we switch to Yu gi go cards?"

At that point I stopped the questioning—feeling very sad and overwhelmed that in our efforts to allow James to have a normal life, I now felt that we will need to provide more education to him as he has lived with hemophilia for 9 years and really only knows that he gets factor replacement every other day. If we don't start helping James now by getting him educated about hemophilia, his hemophilia will be our issue in his later years.

The highlight of the meeting for Julie was being able to talk to a large number of "Exhibitors" and "Experts" in one place. Another highlight was being able to attend as a family (Tori is now old enough to take part in the Youth and Adolescent Session along with James.) Julie also reported that the Opening Exhibition Trick or Treating

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Does Your Child Need A “504 Plan” or an “IEP”?

By Doreen Rousseau, MS

As a parent of three children, two of whom have severe hemophilia, I never really considered the need for an accommodation (504) or individual education plan (IEP) for my sons. They had not experienced any difficulties when they went to school, and they are both fine students. Now, as a school psychologist, I have a working knowledge of what these special plans can do for a child, and I view things differently.

When my first son entered school, I educated his teachers and nurse every year. We were very fortunate that the school nurse had some knowledge of hemophilia and had even attended a local camp supporting children with hemophilia. So, educating staff and having my sons' medical needs met, were never issues.

More recently, our school district has done some re-districting which resulted in my youngest son having to attend a new elementary school. When I shared his medical diagnosis, the principal requested a “504 accommodation plan”. My eldest son entered the high school this year, where gym classes are deemed more physical, and he was denied participation in some activities. I was also not getting the response I wanted for classroom accommodations, which he has had since middle school, so this led me to request a 504 accommodation plan at the high school level.

There is basic information parents need to know regarding the needs of their children in the school environment.

The first is that any need that a student has is better supported with a legal document. When medical and/or educational issues require special accommodations, these legal documents take the form of either a 504-Accommodation Plan (504), or an Individualized Education Plan (IEP). These documents are derived from federal legislation and all states have them. The method in which they are used, or determined necessary, vary from state to state, and school district by school district. Information requiring the processes and procedures in your child's district can be obtained from your State Education Department or your local school district. In school districts, call the Special Education Department or the School Psychologist and ask for parts 200 and 201 relating to the education of students with disabilities. These parts contain the procedures for IEP's. A similar request can be made for the 504 procedures.

IEP's are typically given to students who meet the requirements for receiving special education service; they have a learning disability, and emotional disturbance, exhibit traits of mental retardation or have other health impairments, etc. that hinder their ability to learn. The

federal government supports these services by reimbursing the school for their provision.

504's are given to students who don't qualify for special education services, but have needs that require some accommodation to place them on a “level playing field” with other students. These services are not federally funded and come directly out of the school's budget. Some schools will go the route of an IEP for financial reasons.

If your child is experiencing difficulties with the learning process (acquiring the information taught), then an IEP is the avenue you should consider. For example, a child with a bleeding disorder who has sustained brain hemorrhaging or perhaps bleeding that has affected their vision, etc. would want to pursue an IEP. This process requires an “educational-psychological assessment”, which typically includes cognitive and achievement testing.

If your child has needs more along the physical route: a home tutor, wheelchair, splints, immobilization, additional set of books at home to prevent back and shoulder bleeds or restricted gym activities, etc. then a 504 accommodation plan will do nicely, and an educational-psychological assessment is not required. There are advantages to having one of these documents in place:

- They are binding and legal documents and will be upheld in a court of law
- The information contained in them must be carried through
- As your child progresses through the school system, this document will help inform all teachers and staff of his or her special needs
- This document must be reviewed once each year, and parents must be invited to participate in the review
- Older students are asked to participate in the development of their own plan

Both 504's and IEP's are recognized at the college level if your child has been determined to have a “disability” (cognitive or physical). Some states recognize “hemophilia” automatically as a physical disability. 504 plans, as the public school writes them, may pose problems on this level because the public school is required by law to provide a Free Appropriate Education (FAPE) and colleges are not. This should not stop you, or your child, from pushing to obtain the modifications needed for success in college.

All parents are encouraged to be knowledgeable regarding their (parent and student) rights before a need arises. Unless parents ask, these two avenues of assistance may

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Transition and Insurance

By Laura Lews,
Apex Therapeutic Care

Transitioning your insurance plan can be a daunting and scary experience. There are so many things to consider, and any mistake that is made can have long lasting repercussions. Any best strategy involves planning. The more time that you have to make the change, the greater your options become. There are a few options that you have, and the more information that you possess, the smarter decision you can make.

A transition involving coverage can involve a variety of scenarios. A person who is 18 and will not be continuing to college, an employment change or loss of employment, exceeding the lifetime maximum for insurance benefits, changes in plan coverage, or temporary or permanent disability are a few. Timing is the most important thing to consider involving any change. A pre-existing condition clause can be imposed on a plan if there has been a lapse of continuous coverage for a period greater than 63 days of termination. Keep this number in mind when weighing your options. You don't have much time.

There are primarily five options for health insurance coverage. Discuss your options with someone that is qualified to assist you and weigh all pros and cons prior to making a choice.

1. Evaluate the Group Health Insurance offered by your employer. Evaluate the different plans being offered to choose the best plan for you and your dependents. Look at out-of-pocket expenses, access to care, spectrum of benefits, and lifetime maximums when making a decision. Ensure that there is not a waiting period for benefits that would prevent access to care for a period. There is generally more than one choice, and if you are getting close to your lifetime maximum consider changing plans during the open enrollment period offered by your employer.
2. Consider applying for individual health insurance. An application would be made to a private insurance company. Be aware that each state has different laws governing the acceptance of

certain individuals. In some states, insurance companies may have the ability to deny coverage based upon medical diagnosis.

3. Evaluate state coverage options through Medicaid. Many states have plans for specific diseases that are more 'forgiving' of the financial requirements. Generally, Medicaid programs are offered by each state to provide medical care to persons with low income. There is generally a program to guarantee coverage to children, which is also based upon need. Most Medicaid plans offer comprehensive coverage and offer plans with spend downs or co-payments if you exceed the financial requirements.
4. Medicare may be an option for coverage. If you have become disabled and qualify for Social Security Disability Insurance (SSDI). Medicare may provide medical benefits. There is a waiting period prior to when your Medicare coverage becomes active, so consider a continuation of benefits through the COBRA option of your group health plan. If you are accepted to the Medicare program, your COBRA coverage period will be extended to the full 29-month period prior to when Medicare coverage begins. If COBRA is not available, alternative coverage needs to be found during the waiting period. Also, be aware that Medicare does not offer comprehensive coverage, and many enrollees are found looking for supplemental or gap coverage.
5. Many states offer a High-Risk Health Insurance pool, provided for uninsurable individuals. There are also guarantee issue plans mandated in many states.

There are many other issues to consider when transitioning your current health insurance coverage. We provide health insurance counseling by knowledgeable insurance experts to help with the navigation through these complex issues. From *The Advocate*, Fall 2002

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at times be overlooked by school staff. It is also important to recognize that each child is unique, and that what works for one won't necessarily work for another, hence the term "individual". More importantly, you must work with the school to educate them. Hemophilia is rare, and most schools will not have any prior experience dealing with the issues it brings until your child enters the district. Don't forget to seek help from your hemophilia treatment center. What you do today will certainly pave the way for

your child to have what all parents want for them: a happy and successful school experience.

Remember, advocacy must begin at home. No one cares as much about the physical and mental well-being of your child but you. If you don't take the steps to educate yourself on what can be done, you run the risk of an unhappy school experience. YOU are your child's best advocate.

Health Insurance Checklist

- ✓ Does the policy cover physician/hospital services only?
- ✓ Does it also cover prescription drugs?
- ✓ Does it also cover blood derivatives?
- ✓ Does it also cover rehabilitative therapy?
- ✓ Does it also cover ambulance services?
- ✓ Does it also cover durable medical equipment?
- ✓ Does it also cover home care services?
- ✓ Is separate coverage available for drugs under a drug plan?
- ✓ Does the policy require prior authorization for coverage of medical services or procedures?
- ✓ Does the policy cover ongoing health maintenance needs?
- ✓ Does it only provide coverage for emergency or acute care?
- ✓ Does the policy provide adequate coverage in the event of catastrophic illness? What is the annual deductible?
- ✓ Does the policy have low annual limits (CAPS)?
- ✓ Does the policy have a lifetime limit (CAP) on benefits? What is the lifetime limit?
- ✓ Does the policy restrict the delivery of medical services to specific types of healthcare facility providers?
- ✓ Will the policy cover medical services provided by physicians of healthcare providers other than a designated provider?
- ✓ Will the policy cover medical services provided in other states?
- ✓ Will the policy cover medical services provided in other countries?
- ✓ Does the policy cover pre-existing conditions?
- ✓ Is there a long waiting period associated with the pre-existing condition? How long is the waiting period?
- ✓ If there is a waiting period, can it be waived?
- ✓ Does the policy cover pre-existing conditions once the waiting period has been satisfied?
- ✓ Will premiums be higher because of a pre-existing condition?
- ✓ Can the policy be canceled as a result of an undisclosed pre-existing condition?
- ✓ Can the policy be canceled when the lifetime cap is exceeded?
- ✓ Does my employer provide group insurance for employees and dependents?
- ✓ Can the policy be transferred if my employment situation changes?
- ✓ If my employer changes insurance carriers, will I still be eligible for coverage under the new policy?
- ✓ Does the policy have separate deductibles for different services?
- ✓ Can insurance claims be denied if the insurance carrier decides that they are "medically

unnecessary"?

- ✓ Can reimbursement for medical care be denied if you are not expected to "improve" or "recover" as a result of that care?
- ✓ Are out-of-pocket costs for services not covered by the policy "credited" toward your deductible or stop-loss?
- ✓ What medical services or treatments are included in calculations for lifetime limits?
- ✓ What medical services or treatments are *not included* in calculations for lifetime limits?

The following questions are especially important if you have a managed care plan.¹

- ✓ Do restrictions apply when accessing specialty care? If yes, what are the restrictions?
- ✓ If I am unhappy with the service I receive from my Primary Care Physician (PCP), may I file a formal grievance?
- ✓ What are the procedures for filing a formal grievance for unsatisfactory service provided by any healthcare provider under this managed care plan?
- ✓ What steps must be followed to obtain an outside referral by my PCP to my Hemophilia Treatment Center?
- ✓ How many years of experience does my PCP have in treating my particular disorder?
- ✓ How many patients with my particular disorder have been treated on a regular basis by my PCP?
- ✓ Is my PCP allowed to consult with my former physician to ensure the accuracy and continuity of my care?
- ✓ Is so, can my former physician bill and receive reimbursement for this consultation?
- ✓ Will my necessary lab work be restricted to designated providers?
- ✓ If so, can I appeal based on lack of experience in relationship to my particular disorder?

¹Know Where You Stand: A Checklist of Questions for Your Managed Care Provider, *Hemalog*, July 1995

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Orlando Report (continued)

experience made this one of her childrens' "best Halloweens ever"!

David Stone

No parent ever expects to have a child with a chronic condition. Although we certainly did not imagine that our son would be born with hemophilia, we take great comfort in the fact that we are part of a strong and educated community whose mission is to ensure a high quality of life for those with bleeding disorders, while supporting medical advances in gene therapy and treatments, and providing support and friendship. Nowhere was this more evident than at the NHF Annual Convention in Orlando, Florida last fall.

Our son Matthew is 15 months old and has hemophilia b-severe. As new parents having gone through a crash course in hemophilia, we thought it was imperative that we attend the convention so that we continue to learn more about our son's condition. Rather than making it a family trip, we decided I would go solo to concentrate on the educational task at hand. So, with a full itinerary in my hand, I put on my walking shoes, and off I went to experience it all at my very first convention.

Before I chronicle the sessions I attended, I should share with you that all of the meetings I attended offered valuable information. I had prior knowledge of some, but learned new things every time. For me, the highlight of the convention in addition to the gene therapy update (critically important) was the exhibit hall. I felt the opportunity to ask questions and network with the representatives of the pharmaceutical and home health care companies was invaluable. I learned of their products and services one on one and learned a great deal more than I could have ever possibly read about.

The most valuable information came from the pharmaceutical companies. Most have programs that will supply you with factor free of charge in the event of a hardship situation, such as if you have a period where you have a brief lapse of insurance, possibly due to starting a new job. I hope to never need such assistance, but I'm so glad to be advised of such programs. I would like to extend a heartfelt thank you to all of the companies who participated in the exhibit hall. All were extremely generous with their time and the memorable and useful gifts. They especially took care of the kids and made them feel special. What more could you ask for in the Magic Kingdom!!!

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The Latest Findings (continued)

Dr. Assem Ziady of Copernicus Therapeutics presented his work with mice in which the factor IX gene is transferred through inhalation. This approach has the benefit of not needing a viral vector. Administration with an inhaler, a method commonly used by asthmatics, might have added benefit of a lesser immune response, thus avoiding the development of inhibitors.

Dr. William Velander of the Virginia Polytechnic Institute and State University presented the most promising research, at least for people in developing countries. He spoke about the results of his work with transgenic pigs that produce human factor IX in their milk (see *Haemophilia World*, September 2001, Volume 8, No. 3). The production of factor IX is so abundant that a small herd of pigs could provide sufficient factor IX for the entire developing world. Dr. Velander hopes to achieve the same abundant level of production from his factor VIII pigs within two years.

In addition, Dr. Velander and his associates presented data showing that mice, when they drink pig's milk containing human factor IX, absorb 10 percent into their bloodstream. This parallels research conducted 20 years ago in humans. This finding has two possible benefits. Factor therapy could be by mouth, instead of by intravenous infusion. This would be an immense advantage for small children. What's more, the product, taken orally from a very early age, could be used to "tolerize" small children to factor VIII and factor IX, and possibly avoid the development of inhibitors.

However, these are not today's products. Extensive clinical trials with humans still need to be done and approval from regulatory authorities needs to be obtained. As well, production facilities still have to be built.

It was clear from the presentations that gene therapy treatments are still years away. Many challenges remain, but much has been accomplished since the NHF held its first workshop in 1996. The variety of research and the innovative ingenuity exhibited by the scientists give us much reason to hope. Reprinted from WHF's *Hemophilia World*, Vol. 9, No. 2, June 2002