



Dateline Federation

NEWSLETTER OF THE HEMOPHILIA FEDERATION OF AMERICA

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Jens and Family

The following article was submitted by Lisa Freese, a member of the hemophilia community who is living in Vienna, Austria, with husband Axel (Hemophilia A Severe), son Jens (7), daughter Annika (carrier, 4) and son Maximus (8 months). Another son, Kristofer (14), lives in Denver with his mother.



This summer, our family, already no stranger to medical difficulties and chronic illness, experienced anew the shock and dismay associated with an unwelcome diagnosis when our 7 year old son Jens was diagnosed with Burkitt's (B-cell) Lymphoma, a type of Non-Hodgkin's Lymphoma. The situation was complicated by the fact that we are currently living overseas in Vienna, Austria, where mom, Lisa, is stationed with the State Department. We were lucky in that Europe's premier facility for pediatric oncology is also located here in Vienna: St. Anna's Children's Hospital (or Kinderspital, as the Austrians call it). Returning home was not an option, as Burkitt's is a particularly aggressive form of cancer, and required immediate chemotherapy. However, as fate would have it, both dad, Axel, and Lisa speak German, so an already difficult situation was made a little easier.

As background, on June 13, a Thursday, Jens complained of a sore throat, but not too badly, so we figured he must have slept with his mouth open and left it at that. Two days later, he was still complaining. This time, Lisa took a look inside with a flashlight, and saw that his right tonsil was swollen to almost twice its normal size. We knew this was not normal, but since he had no accompanying indicators of infection, we took him to the Embassy doctor, who prescribed Amoxicillin and told us to come back the following Tuesday. When we returned, the tonsil had not changed, so the nurse made an appointment for us with an Austrian ENT, who decided it needed to come out. Since Jens had never been sick in his life, this sounded like major surgery to us, so we questioned the doctor pretty thoroughly. He assured us that that the surgery would be relatively easy, but that the tonsil clearly needed to come out. Lisa was due to return to the States for a training course, so we scheduled the surgery for after she returned. But two days later, Jens woke up complaining his throat hurt even worse. We peeked inside and saw that the tonsil had grown and was changing colors. That decided it. The doctor scheduled an emergency tonsillectomy for the following morning, and told us the tonsil would be taken to the General Hospital for testing. We were told to report to St. Anna's on Tuesday, 25 June for further testing. Alas, Jens would be receiving more than tests that day.

When the doctor at the hospital informed us of the diagnosis, we could hardly believe it. How could this happen? Jens never got sick, it must be

•Continued on page 15•

HFA Mission Control - Houston, 2003

We are counting down to blastoff for symposium 2003 in Houston, Texas. Reserve the dates on your calendar - March 28 - 30, 2003. The place is the Houston Airport Marriott at Intercontinental Airport. Room rates for a single or double are **\$82** per night.

There is a new feature this year. On Friday morning from 9:30 to 12:30, we will hold a Chapter Development workshop. The subjects will be board recruitment and development, organization, volunteer recruitment and direction, fund raising, budgeting, financial management and design and production of newsletters as a key tool in communication. Presenters will be Star Tyree, former Executive Director of Indiana Chapter; Laurie Kelley of Kelley Communications; and Jan Hamilton, Executive Director of HFA. This session will be open to anyone interested in these topics from chapters or those interested in forming chapters.

Topics of interest to be presented are:

General Sessions: Heptatis C, Hari Conjeevarum, MD; Living and Laughing with a Chronic Illness, John Valusek, PhD; Motivational Speaker, Ray Hollister; Women with Bleeding Disorders; Nutrition; Government Relations Update, Patrick Collins and Jan Hamilton.

Breakout Sessions: Yoga with Lana Bienvenu; Art Therapy with Carol Lancon; Pediatric Dentistry & Hemophilia, John Hendry, DDS; Home Infusion Techniques; Couples Communication; Pain Management; Sports and Hemophilia; Coagulation Disorders for New Families, Keith Hoots, MD; Robert Keller, MD; Dads in Action Panel.

Other Special Features: Women's Tea, New Families 1st Time Attendees group, Break out sessions for Spouses, Parents, Patients and Siblings.

Of course, we will also have our Teen Connection program and HFA Kids will feature some wonderful new educational activities that will also be fun.

Keep your eyes and ears focused for a surprise guest.

Registration is going to be available on our web site, www.hemophiliafed.org, very soon. A limited number of travel scholarships are also available. First preference will be given to first time attendees and prioritized after that on a first come, first served basis.

**• Call our office for additional details:
1-800-230-9797.**





*HFA would like to invite
you to join us at booth #121
in Orlando, Florida for
the NHF 54th
Annual Meeting on
October 31st through
November 2nd.*



Chapter Chat



Chapters and Member Organizations, if there is anything
you would like to share, email us at
m.scricca@cox-internet.com

PHOTOGRAPHY PAINTING
ART SCULPTURE DRAWING
CONTEST

Express Yourself!!

March 28-30

at the Hemophilia Federation of America's next Symposium

in

HOUSTON, TEXAS

All media welcome.

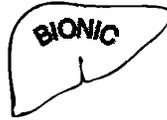
Bring your artwork to the symposium to enter contest.

Email or call the Federation with questions. Or contact

Shannon Pennington at spennington@rock-grove.k12.ok.us

- Art contest for Symposium •
Accepting all media.
Contact Shannon Pennington at
spennington@rock-grove.k12.ok.us or
the HFA Headquarters with any
questions. Artwork can be picked up
at the end of symposium.

FYI CORNER



The Bionic Liver

The shortage of liver donors has become the mother of invention, prompting the creation of a bioartificial liver (BAL) device designed to maintain patients until a donor can be found. The apparatus circulates plasma through a bioreactor that houses liver cells sandwiched between artificial plates or capillaries. If needed, the BAL device could support a failing liver on a long-term basis (similar to the way a kidney dialysis machine supports a failing kidney). Liver cells normally synthesize clotting factors, produce bile, regulate carbohydrate, fat and protein metabolism and break down alcohol/drugs. The BAL device could not perform all the functions of a healthy liver, therefore researchers are trying to develop liver cells that will perform as many of these tasks as possible.

The first clinical use of a BAL bioreactor was in 1987 to treat a single patient with acute liver failure. Although consequent studies have shown that widespread use of the devices was safe, it has been more difficult to prove their efficacy. Results from clinical trials are forthcoming, and while preliminary data from one such study in England showed no clinical survivor benefit, a similar study in the United States and Europe had results that were promising.



Information Quality Standards

Legislation was passed by the U.S. National Academy of Sciences (NAS) called the Information-Quality Act that will go into effect in October 2002. The Act will require federal agencies to follow standards regarding the quality of disseminated scientific information in an effort to hold the government accountable and avoid poor-quality notices.

The Act requires that agencies adhere to a standard of quality, develop peer review procedures and develop an appeals process in case there is a dispute with any of the information the government disseminates. If it is found that a complaint is valid, the data must be removed from all print and Internet sources. It is this third provision that has health experts wary, as it is felt that such criticisms may be used to bring many frivolous lawsuits to courts.

(Source: *The Lancet* 2002: Vol. 359:1133)

Combating Negative Sibling Reactions

If siblings react negatively to their brother's hemophilia, there are ways to give them the attention they need. In her book, **Raising a Child with Hemophilia**, author Laureen Kelley recommends three strategies: treating each child uniquely, spending time alone with each child, and acknowledging the child's feelings.

Since all children are different, it is impossible to treat every one equally all of the time. Each child has special needs, talents, expectations, and developmental patterns. When children compare themselves to a sibling with hemophilia, you should attempt to address their individual needs. When they realize that their needs are being met, they will compare themselves less frequently.



The siblings of a child with hemophilia might feel ignored or less loved by their parents, because their brother requires extra time and attention. But showing your children that you love and value them does not have to mean a big outing or special event. You can also reassure them throughout the day with affection and other special gestures or words of kindness.

It is important to validate your children by letting them know that their feelings are okay. Once you have acknowledged their feelings, you can cope with those feelings in a positive manner. Try not to play "referee" to end an argument, but rather help your children find solutions on their own.

(Source: *HemophiliaGalaxy.com*)

Deltec Safety Needle Receives 510k Clearance

August 19, 2002

Deltec, Inc. has received 510(k) clearance by the Food and Drug Administration (FDA) for its GRIPPER PLUS™ Safety Huber Needle.

The new GRIPPER PLUS™ Safety Needle is used to deliver medications intravenously through a patient's implanted port. Its unique feature is a safety arm that is lifted to lock the needle into a protected position when de-accessing it from an implanted port. An audible click provides clinicians with confirmation that the de-accessed needle is in its locked safety position. The GRIPPER PLUS™ Safety Needle is safe for clinicians, comfortable for patients, and easy to use. It also allows institutions to comply with NIOSH / CDC criteria for sharps safety.



The GRIPPER PLUS™ Safety Needle is based on the input of many clinicians and the design of the familiar GRIPPER® huber needles, which are recognized as the gold standard in huber needles. In the last five years, Deltec has sold over 10 million GRIPPER® needles worldwide.

Deltec, Inc., the manufacturer of PORT-A-CATH® and P.A.S. PORT® implantable access systems and CADD® ambulatory infusion pumps, is committed to developing the highest quality, most reliable products to provide innovative, convenient and cost-effective drug delivery world wide. Headquartered in St. Paul, MN, Deltec is part of the Medical division of Smiths Group plc, London and manufactures a wide range of infusion and access systems for domestic and international markets.

New Consumer Website from NHeLP



Help us spread the word! The National Health Law Program (NHeLP) has officially launched HealthCareCoach.com: www.healthcarecoach.com. Dedicated to helping consumers get the most out of their health care, HealthCareCoach.com features hundreds of articles with information about everything from keeping health care costs down and coping with emergencies to dealing with denied claims and what people can do when they lose coverage.

In addition to lots of helpful facts and information, visitors will also find links to many other useful web sites as well as an opportunity to voice their opinions on national health care issues.

Our Deepest Sympathies

Ernest Charter ("Ernie") Died peacefully in his sleep August 16th, prematurely at the age of 52. Ernie died after a long battle with AIDS inflicted on him by the Anti-hemophilic Factor he used to lead a normal life. Ernie is survived by his wife of 17 years, Rene, and two daughters, Alicia and Danielle Charter of Santa Rosa.

Tom Fahey, the co-founder of the Committee of Ten Thousand and one of our first Board Members, died Thursday September 26th in Pittsburgh awaiting a liver transplant. He leaves behind his wife Fran Fahey, a large and loving family and many friends.

All of our thoughts and prayers are with the families in their time of need.

Hemophilia Association of New Jersey Memorial

The title "Prayer Feather" signifies a remembrance and healing and a prayer for the future. The "wound" at the heart is for all those lost and the suffering.



The "Prayer Feather"

A nation's blood supply is a unique life-giving resource. However, in the early 1980's blood became contaminated by HIV. Tainted blood and blood products transmitted this devastating, often fatal disease to more than half of the 16,000 persons with hemophilia in the United States. An additional 12,000 individuals contracted the disease through blood transfusions.

Blood safety is a shared responsibility of many diverse organizations including the federal government, blood collection agencies and blood product manufacturers, as well as other private sector organizations. The tragedy of the early 1980's was a direct result of failed leadership and greed.

In terms of devastation to a specific population, this was the worst medically induced tragedy in history.

"Prayer Feather", commissioned by the Hemophilia Association of New Jersey, memorializes the innocent victims whose memory remains in our hearts, honors those still struggling to survive and reminds us that the mandate from this tragedy is clear-

Forever Vigilant!



TEEN CONNECTION



Want to feel a part of something bigger? Want the support and friendship of teens that feel the same as you? Here's your chance! Hemophilia Federation of America's Teen Connection, sponsored by Apex is accepting new members and we would love to have you join the ranks of this teen leadership program.

If you meet the qualifications listed below and you would like to participate in leadership development programs while gaining the friendships of others in the hemophilia community, then The Teen Connection is for you!

- Are you between 13 and 19?
- Do you, a family member or a friend have a bleeding disorder?
- Are you willing to commit a minimum of two hours per month?

If you can answer yes to these questions, call or email us today!

800-230-9797

hemophiliafed.org

Product Availability Improves

With recent communications from our manufacturers, we have learned that they are “back in the saddle” and that all sizes of product should be available at this time. Supplies of both monoclonal and recombinant products are back with fairly steady production for increased availability.

NHF’s MASAC has said they are recommending at least a 15% increase in usage over their last recommendation. Numbers showing ratio of inventory to distribution from the manufacturers show monoclonal to be at a pretty steady level and recombinant getting better everyday.

If you had delayed surgery or immune tolerance therapy or voluntarily switched to monoclonal from recombinant, you should be able to return to normal usage at this time, being cautious not to stockpile. When a patient attempts to stockpile product it hurts everyone so we continue to ask that you order within reason.

Any patients who have difficulty obtaining product should first speak with their provider and ask why and then call HFA to let us know where the problem lies.

To those who cut back on usage during the dire periods of shortage and those who postponed elective surgery or immune tolerance, thank you for helping us through this serious shortage. When we work together it is best for all concerned.

To Port or Not to Port????

One of the hottest topics today in the hemophilia community with parents of young children is whether or not to have a port for their child. It is an emotional decision for some and one that should be evaluated thoroughly with your family and with your physicians.

There are advantages – such as ease of access and reduction in stress level in the administering of clotting factor. However, there are other things to consider as well – such as keeping the site sterile, cleaning the access area thoroughly after use to keep the line open.

Some things to consider in making the decision:

1. Is the patient on immune tolerance?
Frequent sticks could mean a port would simplify the infusion procedure.
2. What is the quality of the patient’s veins?
Are the veins small and/or fragile? If so, a port may be an answer.
3. What is the comfort level of the adult administering the factor? This could also be a point in favor of getting a port.
4. If you are not now performing infusions yourself, then the convenience of not having to visit an Emergency Room, HTC, or physician’s office for each infusion may also be an advantage – unless your home care nurse is performing this service for you.
5. Speak with other parents on both sides of the fence before making a decision.
6. Read articles on the subject.
7. Speak in depth with your HTC staff or physician about the pros & cons.
8. It’s not a bad idea to take a sheet of paper and list the pros on one side and the cons on the other – in your own situation – and then evaluate.

There is no universal right or wrong on this subject. Some physicians highly recommend a port even to the point of suggesting it before a child experiences their first bleed. Others prefer to steer clear of a port altogether. The ultimate decision is yours. Make it an informed decision.

TALKING TO YOUR BRAIN

A Few Reminders About Living with a Chronic Illness

John E. Valusek, Ph.D.

When and if we become aware that we have the ability to choose how we will respond to any situation or event which happens to us, we can then permit ourselves to react, initially, with normal fear, anxiety, anger, grief or depression for a short period of time. Then, by tuning in to our thoughts, we can learn to change these reactions by the simple process of changing our thoughts which will also change our feelings as well as our behavior. We all have this power but most of us, no matter how intelligent we may be, never learn to be aware of it and therefore never use it.

Here are a few reminders about living with a chronic illness:

1. When serious illness or injury strikes anyone in the family, the whole family is affected by it. Everyone will, therefore, need support and understanding.
2. Many people believe their illness/disease is a punishment for misdeeds or sins. This belief is destructive and needs to be eliminated.
3. Many children and adults will react to their condition with fearful, regressive, dependent behaviors which lead to inactivity or lack of interests which can lead to overprotective responses from family and friends. Caring does not mean we have to expect our loved ones to be helpless just because they have a serious chronic illness.
4. Some people, especially teenagers, try to ignore or deny their illness which can lead to reckless, rebellious, or risk-taking behaviors. This attitude does not mean they are stupid, rather it is a sign that a good, developing adult brain is still being guided by an immature thinking/emotional status.
5. Mothers can spend a lifetime blaming themselves for causing a child to have hemophilia. The blame is pointless, harmful and doesn't change anything.
6. As much as possible, the child with a chronic illness must be actively involved in the treatment process. This means learning as much as can be understood about all of the important aspects of caring for oneself safely while hopefully developing a positive mental and emotional outlook.
7. Some people respond to chronic illness by adopting a victim role which leads to isolation and a negative attitude of resentment toward all others who are healthy. If you stay in this mood, you will end up "shooting yourself with chemical bullets."
8. The most effective coping requires knowledge, understanding, practice, making mistakes and learning from them while receiving reliable support from others.
9. You do yourself a disservice if you try to pretend that nothing is wrong or different about your state of health but "different" does not mean "oddball."
10. If others laugh at you, criticize or call you names, remember they are telling you a lot about themselves and nothing, really, about you.

Our feelings can be described as emotional sensitivities which function much like the strings of a musical instrument. When we let others get us upset, we give them an unjust power to pluck our emotional strings whenever they wish to do so. We upset ourselves by plucking our own strings.

We, ourselves, are the architects of our future selves. This means we have the ability to change how we act and react to any person or situation, past or present. We can stop being prisoners of the past as soon as we become aware of the power of our own thoughts and of our ability to change them by conscious choice.

Your mind can cause you joy or pain, can make you sick or keep you sane. Be careful what you say in the presence of your brain. Your brain is always listening to you!

NOTE: Dr. Valusek is a popular speaker in many circles including hemophilia chapter annual meetings. You can hear more from him at the HFA Symposium in March, 2003 in Houston, Texas.

GOOD NEWS FROM BAYER

Mary Bauman, Director International Strategic Marketing for Bayer, made the following announcement in the most recent issue of BP Pulse.

Additional 250 IU Vial Sizes Released to Marketplace

Delivering on the promise made earlier this year to make all three vial sizes available, Bayer BP recently released three lots of 250IU vial sizes of Kogenate® FS. These releases, totaling over 11,000 vials, were released to the United States and New Zealand. This is in addition to the May release of 250 IU vials of KOGENATE® Bayer for Europe. Europe also is scheduled for another release of 250s very soon.

At the Global Supply Summit held in April 2002, and in the special WFH Congress edition of *BP Pulse*, it was announced 1000IU vials were being reliably supplied, 500IU vial sizes would become available in May, and 250IU vials would follow shortly thereafter. Bayer BP is pleased to have met that timetable. Bayer BP continues to increase releases with a plan

to exceed year 2000 average quarterly releases by the second quarter of 2003. These latest releases reflect the continuing progress being made toward returning to normal release schedules for all three vial sizes.

Recent Positive News from FDA: July 2001 Warning Letter Closed, 200 Liter Fermentors Approved in the U.S.

In a letter dated July 12, 2002, the U.S. Food and Drug Administration (FDA) informed Bayer BP that they were satisfied with responses provided and progress made following the inspection of late-2000, and the subsequent July 2001 Warning Letter. As a result, the Warning Letter is now officially closed. Additionally, in another letter dated July 30, FDA informed Bayer BP that Bayer's responses and corrective actions following the March 2002 Berkeley inspection are acceptable. These communications reinforce findings of compliance by Canadian and European regulatory authorities following inspections earlier this year. Carol Moore, vice

president of regulatory affairs, commenting on the letters received from the FDA, said, "We are very pleased that the FDA recognizes the significant amount of work done to correct issues identified in these inspections. Many people have worked very hard to achieve this success, and as a result, we have been able to significantly increase the amount of Kogenate® FS and KOGENATE® Bayer released around the world for people living with hemophilia."

Further good news was received from FDA. Achieving a long-term key manufacturing objective for Kogenate® FS, Bayer BP recently received approval for the 200 liter fermentors at the Berkeley, Calif., manufacturing site. Fermentors are used to grow the hamster kidney cells that have been genetically modified to produce human Factor VIII. This approval increases flexibility for Bayer BP in allocating product to the U.S. market.

Bayer BP remains committed to maintaining the highest possible manufacturing standards and providing reliable supplies of Kogenate® FS and KOGENATE® Bayer.

Baxter Launches Super High Potency Hemofil M AHF

Deerfield, IL. September 2002. Baxter Bio-Science announced the launch of a new, super high potency of its Antihemophilic Factor (Human) monoclonal antibody purified preparation, Hemophil M AHF, that will enable patients to reconstitute and infuse fewer vials of the plasma-derived Factor VIII during bleeding episodes.

The super-high potency provides between 1701 and 2000 IU's per vial, ideal for high-utilization applications. Currently, HEMOFIL M AHF is available in potencies ranging from low (220-400 IUs per vial), medium (401 - 800 IUs per vial) and high (801 - 1700 IUs per vial.)

Kristen Nelson, HEMOFIL M AHF marketing manager said, "We developed the super high potency version of this leading FVIII therapeutic to make life easier and better for hemophilia patients who require more frequent infusions, or for applications requiring high doses, such as surgery." Other convenience features include 30 month storage at room temperature and reconstitution with the recently introduced BAXJECT needle-less transfer system.

"Those who use HEMOFIL M AHF are primarily adult men, often with joint damage, who require more frequent infusions," explained Kathy Bosma, nurse coordinator at Michigan State University. "This new potency reduces the need to mix several vials of therapeutic for a single infusion, making it easier and more convenient for patients to perform home treatments."



HFA BOARD MEETING



Our Executive Board L to R: Carl Weixler, Barbara Chang, Alan Converse
Rich Vogel(standing), Bob Marks, Donnie Akers and Jan Hamilton
Not Pictured: Peter Bayer and Randy Ratcliff

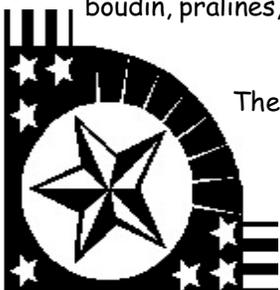
First Annual Mid-Year Board Retreat

Marking another milestone in HFA history, our Board of Directors gathered in Lafayette, Louisiana September 19 - 22 for our very first Mid-Year Board retreat. Food, fun and fellowship were sandwiched in between two day long sessions and an Executive and Finance Committee meeting.

Budget discussions and symposium planning accompanied the launch of four new programs for HFA. The enthusiasm for Dads in Action (expansion), Putting a Face on Hemophilia, Emergency Factor Tracker and Emergency Room Triage Education ran rampant throughout the weekend.

Board members were given the opportunity to tour our headquarters and meet and get to know our staff. They were treated to culinary delights most had never experienced including crawfish, alligator, boudin, pralines, beignets and stuffed bread.

The weekend drew to a close at the home of Dr. and Mrs. Charles (Jan) Hamilton.





L to R: Sue Dill, Rich Vogel, Joe Caronna, Judy Igelman and Christy Argo



L to R: Maria Rubin, Rich Vogel, Shannon Pennington and Sharen Haddad



L to R: Randy Ratcliff, Donny Akers, Barbara McKeown Burke and Joey Privat



L to R: Star Tyree and Carl Weixler



L to R: Trudy Stringer, Christy Argo, Debbie de la Riva, Phill Blomquist
Judy Igelman, Alan Converse and Lew Collins



Public Health Dispatch: Investigation of Blood Transfusion Recipients with West Nile Virus Infections



An investigation conducted by CDC, the Food and Drug Administration (FDA), the American Red Cross and state health departments in Georgia and Florida has confirmed transmission of West Nile virus (WNV) from a single organ donor to four organ recipients .

During treatment for injuries that eventually proved fatal, the organ donor received numerous transfusions of blood products. However, the source of the organ donor's infection remains unknown.

Subsequently, CDC has been informed of four other patients with WNV infection diagnosed after receiving units of blood in the weeks before WNV diagnosis. Because each of these patients resided in areas with high levels of WNV activity, the most likely mode of infection is mosquito exposure. To rule out blood transfusion-associated transmission, investigations are ongoing and efforts are under way to contact donors of blood given to these patients and other recipients of blood from these donors for follow-up and WNV testing. In each instance, precautionary measures have included a withdrawal of any remaining blood products obtained from the donors whose blood was given to these patients.

The investigations of the organ donor and four other transfusion recipient patients involve follow-up of approximately 100 donors. Initial testing by a quantitative polymerase chain reaction (PCR) assay (TaqMan[®]) of stored blood specimens obtained from the blood donors at the time of donation has been completed for two investigations. In one investigation, both donors tested negative for WNV. In the second investigation, in which specimens for 15 of 17 donors were available, specimens from three donors had evidence of WNV viral RNA, suggesting that these donors might have had WNV infection at the time of donation. In addition, plasma derived from a donation by one of these three donors also had evidence of WNV RNA. These results are preliminary because all of the specimens available for testing in both investigations were initially processed and stored as part of routine blood collection procedures, which could affect test validity. Therefore, additional follow-up testing and epidemiologic investigation of these donors are necessary.

In cases of suspected WNV meningitis or encephalitis in recent (e.g., ≤ 4 weeks before onset of illness) recipients of blood or organs, clinicians should contact local public health authorities to initiate an investigation. Serum or tissue samples should be retained for later studies.

•Updated: September 13, 2003•

West Nile Virus Blood Transfusion Q & A

West Nile virus is an arthropod-borne virus first isolated in 1937 from the West Nile region of Uganda. The natural transmission cycle of West Nile virus includes mosquitoes (Culex species) and birds (crows and blue jays), with humans and other animals (such as horses) as incidental hosts. West Nile virus infections are associated with significant mortality in horses and certain domestic and wild birds. Historically West Nile virus has been found primarily in Africa, Asia, southern Europe, and Australia. It has been associated with several epidemics such as those in Israel, South Africa, France, Romania, and most recently in the metropolitan area of New York City. In humans, most West Nile virus infections are subclinical. Febrile disease is the most common clinical presentation in young adults. Other symptoms may include fatigue, headache, weakness, gastrointestinal problems, and altered mental status. Neurologic involvement, which occurs in a small percentage of infected individuals, may produce encephalitis and/or meningitis. Symptoms indicating potential central nervous system involvement include confusion, nuchal rigidity, and loss of consciousness. Mortality rates of 5% to 14% have been reported in individuals who demonstrate neurologic disease.

Q. Is West Nile virus transmitted by blood transfusion?

A. To date, there are no documented instances where West Nile virus was transmitted through transfusion. However, the virus may be transiently present in the blood of those infected, so it is possible that this could occur.

Q. What is being done about this possibility?

A. On August 17, FDA issued an alert to blood banks and organizations to be vigilant in excluding individuals who may have early symptoms of West Nile virus from donating blood. In addition, the Food and Drug Administration and the Centers for Disease Control and Prevention have asked that blood banks and others report any cases where a blood recipient develops West Nile virus for investigation of whether transfusion could have been involved. Such an investigation is now being conducted by CDC to determine whether blood transfusion or donor organs may have been a source for the possible West Nile virus cases in the current transplant recipients.

Q. What else can be done to protect people against this possible threat?

A. In this case, even though a blood source is unproven at this time, as a precaution, to protect others, blood banks are recalling any remaining units of blood products from all donors whose blood was administered to the transplant donor. There is currently no validated blood test to screen donated blood. If it is shown that people without symptoms can transmit West Nile virus through donating blood, public health partners will work with others to help facilitate availability of such a test.

Q. Should people avoid donating blood or getting blood transfusions?

A. Blood is lifesaving and is currently in short supply. Donating blood is safe and we encourage blood donation now and in the future. For patients who need a blood transfusion, the benefits far outweigh any risks.

Q. How can blood banks avoid collecting blood from donors who may have West Nile virus?

A. Most people who have West Nile virus do not show symptoms, making it difficult to defer them from donation. However, some individuals develop minor symptoms of fever and headache. Blood banks need to be vigilant to defer all of those who may have minor illnesses, especially in areas where West Nile virus is most active.

Q. If a person has had West Nile virus, can they still donate blood?

A. With West Nile virus infection, the viremia usually is transient and people recover very quickly. Blood centers will take precautions to be sure that donors who have been diagnosed with West Nile virus have fully recovered before donating.

Q. If I recently had a transfusion, should I be concerned about getting West Nile virus?

A. There have been no cases where transmission by blood transfusion has been documented. Although, by natural routes, infection would probably be expected in 2-14 days, it is unknown what incubation period would be expected if transmission were transmitted by transfusion. If you have symptoms of West Nile virus or other concerns you should contact your physician.

Q. I am on a list to receive an organ transplant. Should I be concerned?

A. We are not at a point in the investigation where we can determine whether WNV can be transmitted through an organ transplant. If someone needs a transplant, he or she should get it.

Industry Confirms Plasma Therapies Safe from West Nile Virus

(Annapolis, MD – September 6, 2002)- The Plasma Protein Therapeutics Association (PPTA), concerned about the numerous reports in the media surrounding the West Nile Virus, has issued the following statement for consumers worldwide. Plasma-derived products do not represent an increased risk for the transmission of the West Nile Virus. This is because West Nile Virus is a member of a family of so-called lipid enveloped viruses, which are very susceptible to a wide variety of inactivation procedures already in place for the manufacture of plasma protein therapies. Inactivation steps such as solvent/detergent treatments, pasteurization, low pH incubation and solvent extraction have been extremely effective inactivating lipid enveloped viruses.

The PPTA member companies have evaluated their manufacturing processes for the ability to inactivate or remove potential viral contaminants and found that they have been shown to provide significant inactivation and removal of these type of viruses. While to date no cases of West Nile Virus have been directly attributed to blood products, reports of four Florida organ-donor recipients contracting the disease is a cause for concern. The FDA has already provided safety alerts for the collection of blood, including stringent donor deferral practices. Moreover, the Centers for Disease Control (CDC) continue to investigate the Florida infection cases to pinpoint the exact cause of transmission.

Existing plasma donor screening practices are likely to reject donors who show symptoms of West Nile Virus. Moreover, PPTA will facilitate discussions among industry experts and scientists to provide an ongoing assessment of any health risk West Nile Virus may pose. The industry stands ready to take whatever appropriate action is necessary to assure the ongoing safety of plasma therapies.

About the Plasma Protein Therapeutics Association

The Plasma Protein Therapeutics Association (PPTA) is the primary advocate for the world's leading source plasma collectors and producers of plasma-based and recombinant biological therapeutics. The medicines produced by PPTA members are used in treating life-threatening diseases and serious medical conditions including bleeding disorders, immune system deficiencies, alpha-1 antitrypsin deficiencies, burns and shock. For more information visit the association's Web site, www.pptaglobal.org.

a mistake. And indeed, because all we had to go on was the word of an unfamiliar doctor (the hard copy test results arrived several days after we were told of the diagnosis), in the backs of our minds niggled the idea that there must be some misunderstanding, for how could a 7 year old get cancer? Of course it was wishful thinking—the cancer was there, it was growing, and it would take Jens unless we got our acts together and began helping him to fight this new and unwelcome visitor. For Jens it was equally traumatic: one minute he's healthy except for a swollen tonsil, the next minute he's in the hospital having tonsils removed, then the next thing he knows he's checked in at yet another hospital having numerous tests done, not to mention having an IV inserted and receiving a lumbar puncture (spinal tap). By the end of the first day, he looked like he had been run over by a truck. As for us, we were stunned. Lisa went home and wrote the first of what became regular updates—the only means we had of keeping in touch with our relatives and friends around the world. Axel stayed with Jens at the hospital, in what became a routine. Whenever Jens went into the hospital to stay, one of us went with him.

For us, the first week or so after the diagnosis consisted of absorbing as much information as we could about this new disease called Burkitt's lymphoma. (Many of you know the feeling—Hemophilia? What the heck's that? I thought that was something royalty got!) Luckily, our family members back in the United States searched website after website for information to send us: on Burkitt's; on therapies, both conventional and alternative; and on how best to get Jens well as quickly as possible. Being members of the hemophilia community helped so much: from Rick Nagler we learned how to handle mouth sores, nausea, and pain, not to mention received lots of distracting videos; from Ellis and Linda Leigh Sulser we received undaunting and ceaseless support, prayers, and compassion; from Jan Hamilton we received inspiring and helpful emails; and from our friends at HACA good wishes and lots of prayers. All these folks know what it is like to live with illness, with pain, with suffering, and yet all of these folks really know how to LIVE! And that's what kept us going—knowing that we had support from so many people who knew, inside and out, what it is like to take drugs that sometimes seem worse than the disease they're intended to treat. That for us was the hardest—having to watch Jens suffer, knowing we could ultimately do nothing other than reassure him of his own strength and his ability to endure.

Interestingly, as the treatment progressed, Jens gradually took ownership of his cancer, of his treatment, and of all aspects of his well being. He learned to read his blood work results, demanding to see "his counts" as soon as they arrived in the room. He learned the best ways to handle his ravenous appetite (result of the cortisone he received) and his nausea (result of the rest of the chemo he received), managing to eat enough to stay off a feeding tube. He learned which nurses were funny, which were grumpy, which would give him a kiss or some extra time or a cuddle. And he learned that in crisis, our family sticks together, and that there are lots of people in the world who love him. As of the 3rd of September, Jens is cancer free—the ultrasounds and CT scans show no tumors. For now, he is healthy, but the doctors warn there is a 10 percent chance the cancer can recur. If it does come back, it will likely to recur in the throat, and rather quickly. So for the next several months we must remain vigilant, keeping watch over Jens. This won't be a problem. The problem will be backing off, giving him space, letting him get back into the swing of things. This is a struggle for all parents of children who live with illness of some form, whether it be cancer or hemophilia: not to identify your child with the disease he or she happens to have. And when Jens gets that first cold, as he certainly will, it will take all our strength not to rush him back to the hospital, to let his own immune system do its job, to trust that he is truly well.

(Editor's note: So often we get so embroiled in the day to day struggles of living with Hemophilia that we forget other tragedies can visit our lives. This is a touching story of a father who has hemophilia, the trauma their family encounters when a child is diagnosed with a catastrophic illness and the family dynamics that ensue.)



Hemophilia Gene Therapy: 2002 Update

By: Chris Walsh, M.D.

This is a brief vignette summarizing the current and future genetic therapeutic interventions for interested patients and their families.

Clinical Trials

All the current trials are corporate sponsored by small biotech firms. The trials involve patients with both Hemophilia A and B using different approaches to genetically correct the diseases.

Transkayotic Therapies Inc. sponsored clinical trial showed some modest success. In this report, Hemophilia A patients underwent a skin biopsy (a small piece of skin is removed) and the skin cells grown in the laboratory. The cells were then exposed to the factor VIII gene that is introduced into the cells by the use of a small electric current. The cells that retain the gene are grown for several weeks until a sufficient number can be injected into the patient. Using laparoscopy, a small hole is made in the abdomen and the cells injected into the omentum or fat pad covering the abdomen. The cells take up residence and grow into the tissue. Over time the cells secrete factor VIII protein, which winds up in the bloodstream. Twelve patients underwent this procedure. According to a published report in the *New England Journal of Medicine*, all 12 patients had no serious reaction and no complications. In some patients, factor VIII was detected in the blood in levels ranging from 0-to-4%. Factor usage decreased in those patients with detectable factor. However, it appeared that the plasma levels of factor VIII dropped along with an increase factor usage over a period of several months. Bottom line-low levels of factor VIII can be produced but don't last more than a few months. Although the results are modest, this hemophilia clinical trial indicates that the potential promise of gene therapy is around the corner. This trial is closed but another Phase II trial is being considered.

Another factor VIII trial supported by Chiron used a retroviral vector carrying the factor VIII gene that was delivered by an intravenous injection. Although the results are not yet publicly available, 13 patients have received different doses of the virus. In one patient treated at UNC-Chapel Hill, his levels reached 1% of normal and his factor requirement has dropped—now over one year. Again suggesting a small increase in FVIII can lead to a modest clinical effect in a small percentage of patients although not long-lasting. No additional data has been presented and the trial is now closed.

The Factor IX trial sponsored by Avigen found 3 of 8 patients with modest levels of factor IX after an adeno-associated virus was injected into skeletal muscle. Once again, requirement for factor use dropped in those patients but in the absence of what would be considered therapeutic factor levels. Similar to the other trials mentioned above and due to ethical considerations, no patients received a sham or fake vector to determine a 'placebo effect'. This study is now closed.

A new trial sponsored by Avigen uses adeno-associated virus carrying factors IX that is introduced through the artery leading into the liver. The rationale for this is that in studies in mice and dogs, the best factor IX levels were obtained if injected directly into the liver. Patients with Hepatitis C must undergo liver biopsy before being considered suitable candidates. This trial has enrolled 4 patients. The 2 patients given the lowest dose of virus did not produce detectable factor IX. This trial is open.

• Gene Therapy Cont'd •

A trial sponsored by Genstar for Hemophilia A patient's uses a 'gutless adenovirus.' A few patients have enrolled. Adenovirus has received terrible press after the death of an 18-year old boy last fall in a gene therapy trial at the University of Pennsylvania. In that trial, an early version adenovirus (developed in the early 1990's) was directly injected into the livers of patients with a rare inherited liver disease. The adenovirus directly injured the liver and precipitated the boy's death. Since then modification of the adenovirus to reduce or eliminate its toxicity have led to 'gutless adenovirus'. It remains to be seen in humans whether this 'gutless' virus is effective and non-toxic. It is known that even this gutless vector still triggers an inflammatory response causing a transient increase in liver enzymes and a decrease in platelets. Such has been observed in one patient that has received this vector. Clearly the risk-to-benefit ratio using this vector should be weighed carefully by patients because there are clearly known side effects with this vector.

New Approaches

Both genetic and cell-based strategies loom on the horizon. Genetic approaches correcting the RNA rather than the DNA appear to be useful for large genes such as the factor VIII gene. Viruses are limited in how much genetic material they can carry. Using this RNA system, only the portion of the gene that is defective need be corrected. This is very new and more work is needed to improve the system.

New vectors such as those based on the HIV-1 virus appear to be headed toward the clinic. Given the historic relationship of Hemophilia patients and viral infections, only the demonstration that this vector is highly effective and superior to all others will offset the safety and psychological issues posed by this vector. The introduction of non-viral vectors DNA-based methods to avoid the concerns of viruses is appealing and being pursued.

Methods for generating stem cells from organs such as marrow, skin, fat, etc. that can be potentially transformed in the lab to liver cells offers a totally new avenue of research. Recent studies in mice suggests that rare populations of cells can be transformed suggesting stem cell 'plasticity' similar to 'morphing' that we now take for granted in the latest movies (see *The Matrix*). This work will take several years to define but in theory an example would call for the isolation of bone marrow cells that are cultured in the lab to induce them to form liver cells. These cells still carry the factor mutation and are then corrected by the various gene transfer methods; the gene-corrected cells are then reimplanted back into the patient. Since the cells were originally derived from the patient, this would bypass many of the transplantation hurdles and need for immunosuppression.

Interest in hemophilia gene and cell-based therapies are now growing. Research support from a number of private and public-funded sources suggests that many more methods of gene transfer will be available. All together this ushers in hope that soon a safe and effective treatment for hemophilia patients will be at hand.

About This Publication

Dateline Federation is a publication of the Hemophilia Federation of America (HFA). It is published four times a year. The material in Dateline is provided for your general information only. We do not give medical advice or engage in the practice of medicine. The HFA recommends that you consult your physician or local treatment center before beginning any form of treatment. Send all comments, suggestions, and article submissions to: Dateline Federation, 102-B Westmark Blvd., Lafayette, LA 70506.

Mission Statement

Hemophilia Federation of America is a national nonprofit advocacy organization established for and directed by persons with hemophilia and other coagulation disorders and their families of origin and/or families of choice. The Federation exists for the sole purpose of serving its constituents as a patient advocate for, but not limited to, product safety, treatment, insurance and quality of life issues in a positive and proactive manner. Its mission is to serve the needs of all families with coagulation disorders and complications of treatment, such as HIV.

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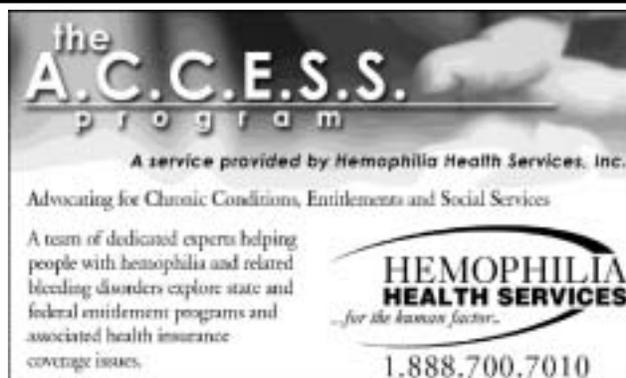
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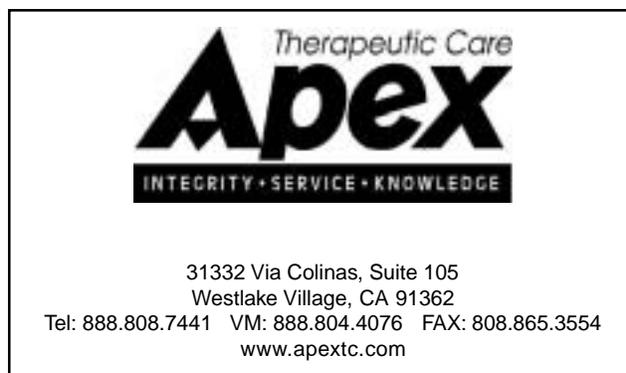
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Community Calendar

OCTOBER

October 26-30 the **American Association of Blood Banks** will hold their meeting in Orlando, Florida. Contact AABB Meeting Services at 301-907-6977 for more information.

On October 31 to November 2, the **National Hemophilia Foundation** will hold its 54th Annual Meeting at Disney's Coronado Springs Resort in Lake Buena Vista, Florida. Call (800) 424-2634, ext.4 or you can email them at meetings@hemophilia.org for more information.

Calling all Walkers, Bikers, Rollerbladers and Runners. Join the **McHenry County Walk-A-Thon** for Hemophilia October 26 at Petersen Park in Illinois. For more information contact Pam Doughty at 815-759-1636 or go to the web site at www.hfi-mcherny.org.

NOVEMBER

On November 4-5, the FDA will sponsor a Public Scientific Meeting in Bethesda, MD to further discuss development of donor screening tests for the West Nile Virus.

DECEMBER

The 2002 Annual Meeting of **The American Society of Hematology** to be held December 6-10, in Philadelphia, Pennsylvania. Call 202-776-0544 or e-mail ash@hematology.org.

The **Maine Holiday Party** will be held on December 8 with the location to be announced at a later date. See next issue for more information.

The **Blood Product Advisory Committee (FDA)** will hold their meeting in December from the 12th-13th in Bethesda, Maryland. Call 800-741-8138 code #19516.

JANUARY 2003

The 32nd **Critical Care Congress** Meeting titled *Cutting Edge Therapeutics* will be held in San Antonio, Texas from January 28 to February 2, 2003. For more information, you can call 847-827-7157.

FEBRUARY 2003

National Home Infusion Association will hold their

12th Annual Conference February 2-5 in San Antonio, Texas.

The **American's Blood Centers** Annual Meeting will be held in Washington, D.C. February 22-26. Contact 202-393-5725. This meeting is open for members only.

MARCH 2003

Hemophilia Federation of America holds its Annual Symposium in Houston, Texas on March 28-30 at the Airport Marriott. For more information call us at 337-991-0067.

Self-acceptance

*Have patience with all things
but first with yourself. Never
confuse your mistakes with
your value as a human being.*

*You're a perfectly valuable,
creative, worthwhile person
simply because you exist.
And no amount of triumphs
or tribulations can ever
change that.*

*Unconditional self-acceptance
is the core of a peaceful mind.*

- St. Francis de Sales

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