

Hemophilia OUTLOOK

A Newsletter of

The Hemophilia Association of New York, Inc.

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THANK YOU FOR YOUR SUPPORT

Dear Friends:

I have successfully returned from my biking tour across the United States. All the hard work, which included going through the Mojave Desert, and climbing over the Ozarks, Rockies, and San Bernardino mountain ranges, contributed to memories that will last me a lifetime. I would live through all the pain and exhaustion all over again to feel the happiness that overwhelmed me when I completed my journey. This trip has proven to me that nothing worth having in this world comes easy. I just wanted to take this time to sincerely thank you for supporting and encouraging me on my campaign to help the fight against hemophilia. Your kindness not only means a great deal to me, but to those who will be aided by your generous donations. -Glenn Berke

MISSING MAIL...

Once again, the Association has determined that a number of pieces of mail containing contributions were not delivered to us, nor were they returned to the senders. *This problem has periodically plagued us and other postal patrons.*

If you have sent a donation and have not received a receipt, or your check fails to clear your bank account in a timely manner, or if a *cash* check does not bear our endorsement stamp, **PLEASE** call 212-682-5510 or e-mail us at hany@bestweb.net.

In Memory of

Frances Anne Patti

We are saddened by the death of trustee Frances Anne Patti. She past away on August 24, 2010.

Fran served on our Board since 1988. Over the years she participated in various support groups, serving as a resource for new parents seeking guidance. In the 1980s, she and her family even prepared a video tape for parents of young children with hemophilia. Most recently she was the first to volunteer for our trips to inform and educate our legislators in Albany. Frances will be missed.

She is survived by her husband Emanuel, son Alexander, daughter Amy, and grandchildren.

Si usted necesita traducción or interpretación en español de algún artículo en este letras de noticias por favor de llamar a Ann Pérez o Gabriela Blum al (212)682-5510.

FDA HEARING ON EXPANDING ACCESS TO ANTIVIRALS FOR HCV PATIENTS

(www.hemophilia.org)

On April 30, 2010, physicians and patients provided public testimony to the Food and Drug Administration (FDA) in favor of allowing seriously ill people with hepatitis C (HCV) access to “direct-acting” antiviral therapies.

Left unchecked, HCV can cause liver cancer, cirrhosis, end-stage liver disease and liver failure. The standard treatment combines pegylated interferon (P-IFN) and ribavirin (RBV). However, nearly 50% of patients do not respond to it. “Current control of hepatitis C is not working,” said Peter Lurie, MD, of the FDA’s Office of the Commissioner. Further, patients who do respond often experience debilitating side effects that can last the duration of the treatment—either 24 or 48 weeks. Interferon’s side effects include severe flulike symptoms, depression, fatigue and insomnia. Ribavirin can cause anemia, skin rash, fatigue and birth defects.

The hearing was held in response to a petition submitted in the Fall of 2009 by Mark Antell and Paul Brayshaw, representing the bleeding disorders community. In the petition, the advocates requested a public hearing for all stakeholders, plus expanded access to promising HCV drugs and combination regimens for patients unresponsive to standard P-IFN/RBV. Both spoke about the seriousness of HCV in the bleeding disorders community and the urgent need for expanded access to treatments. Others who could benefit from a broader regimen of drugs include patients with cirrhosis, HIV and those who are minorities.

Healthcare providers at the hearing expressed concern about limited access to clinical trials. Diana Sylvestre, MD, who treats HCV-infected intravenous drug users in the San Francisco area, mentioned that only a small subset of “real-world” HCV patients are included in trials of new drugs due to their drug use, co-morbidities and mental illness.

The FDA is creating a guidance document on the use of direct-acting antiviral agents, which is expected to be released this year. If approved, an expanded access program could be applied to several novel HCV treatments now in clinical trials. The therapy farthest along in development is the investigational drug Telaprevir, a protease inhibitor manufactured by Vertex and partner Johnson & Johnson. The results of a phase II trial published in *The New England Journal of Medicine* (NEJM) in April showed that HCV patients previously treated unsuccessfully with P-IFN/RBV had a much greater chance of clearing the virus when telaprevir was added to their treatment regimen. Success is measured by patients’ ability to achieve sustained virological response (SVR) for at least six months after completing therapy. In the *NEJM* study, more than 50% of patients in the P-IFN/RBV/telaprevir groups achieved SVR vs. only 14% in the P-IFN/RBV group.

NATIONAL HEMOPHILIA FOUNDATION’S ANNUAL MEETING

National Hemophilia Foundation’s 62nd Annual Meeting, from November 11th to 13th, 2010 in New Orleans, Louisiana.

To register, visit www.hemophilia.org or call toll-free 800-424-2634.

HANY SCHOLARSHIPS AWARDED...

Nine candidates are sharing \$36,000 in HANY scholarship awards this academic year. Five are first-time recipients and four are repeat grantees.

Their fields of study cover acupuncture, marketing, accounting, computer science, business and genetics, pre-dental, pre-law, biology, and nursing.

Among the stipends given are the ‘Isabel K. Brach Award’ (this year for nursing) and the ‘Dr. Margaret Hilgartner Memorial Award’ (this year for pre-dental).

Since 1981 the Association has awarded 85 competitive scholarships for higher education totaling \$237,710. From its beginning — providing \$500 to \$1,000 stipends to persons with bleeding disorders during their freshman year — the program has expanded to consider annual grants up to \$7,000 each for study at accredited academic institutions, including undergraduate and graduate schools, and certified trade and vocational facilities. Eligibility now extends from persons with certain diagnosed bleeding disorders to carriers and unaffected children of parents with qualified bleeding disorders.

Look for the announcement of our 2011-2012 scholarship program in the Winter issue of OUTLOOK.



Our address has changed but we haven't moved!

Our building has been **re-numbered** to:

110 East 40th Street

SCAMS ON RISE IN WAKE OF HEALTH CARE LAW

(AARP Bulletin, June 2010)

State insurance commissioners and attorneys general are warning consumers about a new wave of scams that are exploiting uncertainties about the new health care law.

Con artists may call, email, or show up at your door saying that under the law you must have health insurance or go to jail. They may even identify themselves as government officials and describe the policies they're selling as ‘ObamaCare’ insurance.

‘People are terribly confused and in some cases frightened’ about the new law, says Kim Holland, Oklahoma’s insurance commissioner and vice chair of the antifraud task force of the National Association of Insurance Commissioners. ‘And that’s something a scammer is going to look for—the opportunity to take advantage of someone who doesn’t know better.’

For the record: The requirement to have health insurance doesn’t begin until 2014, and even then you can’t be jailed for not having it. No real government official sells insurance, no ‘limited enrollment periods’ exist, and there’s no such thing as ObamaCare coverage.

Ways to protect yourself: Never sign up for an insurance policy without calling your state insurance department to find out whether the policy is legitimate and the seller is licensed. Never give your credit card, bank account or Social Security number to anyone you don’t know. And familiarize yourself with at least the main requirements of the health care reform law.

**OCTAPharma USA ANNOUNCES
FDA APPROVAL OF WILATE® - THE
FIRST REPLACEMENT THERAPY
DEVELOPED SPECIFICALLY FOR
VON WILLEBRAND DISEASE**

(Veinline, Summer 2010)

Octapharma USA announces the U.S. Food and Drug Administration has approved wilate® for the treatment of spontaneous and trauma-induced bleeding episodes in patients with all types of von Willebrand disease (VWD).

Wilate® is a newly developed, high-purity, double virus inactive von Willebrand Factor/Coagulation Factor VIII Concentrate (Human) that demonstrated efficacy for all types of VWD including pediatric patients in four prospective clinical trials utilizing both objective and subjective criteria.

Wilate® is indicated for the treatment of spontaneous and trauma-induced bleeding episodes in patients with severe VWD as well as patients with mild or moderate VWD in whom the use of desmopressin is known or suspected to be ineffective or contraindicated. Wilate® is the first double virus inactive VWF/FVIII (von Willebrand Factor / Factor FVIII), high-purity concentrate, utilizing the solvent/detergent (S/D) process and a special terminal dry-heating (TDH) system. The selected purification processes isolates the VWF/FVIII complex under highly protein-protecting conditions, resulting in a 1:1 ration of VWF:RCo (ristocetin cofactor) and FVIII activities that are similar to normal plasma. No Albumin is added as a stabilizer. Wilate® is exclusively derived from large pools of human plasma collected in U.S. FDA approved plasma donation centers.

**FACEBOOK AND FACTOR:
DONOR BEWARE**

(Parent Empowerment Newsletter, August 2010)

By anyone's standards, Americans are big givers. In 2009, Americans donated an estimated \$303.75 billion to charitable causes, according to the Giving USA Foundation. About half of this went overseas, to help with natural disasters, refugees, and social reforms. What's truly amazing is that about 75% was donated by individuals, not corporations or foundations. Whatever the world thinks of Americans, you can't deny that we have big hearts.

And when the US hemophilia community hears of a suffering child in an impoverished country, it's not hard to find someone to help. Most often, help comes in form of donated factor. The 4 million IU donated each year is a testament to the US hemophilia community's compassion.

But donating factor just got trickier. Social networking, through forums like Facebook, allows anyone from a developing country to ask hemophilia parents and patients directly for a factor donation. Not knowing international laws, Americans who want to donate factor can unwittingly put themselves in a dangerous, even illegal position, while putting the people they wish to help at risk.

Logistics: Customs is the official process of screening every package that comes into a country. Items must be declared in a specific way, or risk being delayed or seized. Biological products like factor are generally not allowed into a country without careful screening, and must be registered with the government.

(Continued on Page 6)

NEW YORK STATE'S HEALTH INSURANCE PLAN FOR PEOPLE WITH PRE-EXISTING MEDICAL CONDITIONS IS NOW AVAILABLE

In March 2010, Congress passed the health care reform law, the Patient Protection and Affordable Care Act. The law created a temporary program until January 2014 that makes coverage available for individuals who have a pre-existing medical condition, have not had insurance for six months, and who are legal US residents.

This new program is called the Pre-Existing Condition Insurance Plan (PCIP) and will be available throughout the country. In New York State, the PCIP plan is the NY Bridge Plan, administered by GHI, an EmblemHealth company.

PCIP plans will operate until the year 2014. At that time other provisions of health care reform will go into effect and state health benefit exchanges will be available for individuals to purchase coverage.

Highlights of the NY Bridge Plan Covered Services:

- No Deductibles
- Preventive Care
- \$20 Office Visit Copay
- \$500 Inpatient Per Occurrence Copay
- \$100 Emergency Room Copay
- Pharmacy Benefits
- Vision Care

For Eligible New Yorkers, you must:

- Be a legal US resident
- Be a resident of New York State
- Have one or more pre-existing medical condition(s), and
- Not have had health care coverage for the last six months.

There is no minimum age to qualify — even newborns may be enrolled if they meet the eligibility requirements. However, once you turn 65 years of age and have Medicare coverage, you will no longer be eligible for the NY Bridge Plan. Applicants who transfer from another state's PCIP program will be eligible if they are a resident of New York and have less than a 63 day lapse in coverage from their prior PCIP coverage.

With the NY Bridge Plan, your eligibility is not based on income. The NY Bridge Plan offers premium rates that are significantly lower than other individual coverage, plus low copayments and no deductibles.

Premium rates are standardized depending on where you live. These rates are for individuals only, as there is no family or dependent coverage offered with the NY Bridge Plan. If other family members qualify, they may also apply for coverage separately. Accepted members will be billed individually and be fully responsible for paying the monthly premium. For information on the NY Bridge Program rates, go to:
www.ghi.com/nybridgeplan/index.html.

Information about hemophilia won't be found anywhere on the NY Bridge program's website. Hemophilia is a qualifying condition. GHI/Emblem Health will cover factor products, both inpatient and outpatient, as well as other necessary services (nursing, etc).

For more information on the NY Bridge Plan, call from 8:30 am to 5:30 pm, Monday through Friday at 1-866-693-9277.

For information on factor coverage call GHI at 877-444-9622.

FACEBOOK AND FACTOR:
DONOR BEWARE

(Parent Empowerment Newsletter, August 2010)
(Continued from Page 4)

If you're not connected with a nonprofit and don't have paperwork filled out correctly, chances are your package won't get through, and valuable factor will be lost.

Are you even allowed to ship it? Factor is normally shipped only by companies licensed to sell pharmaceuticals. They know the correct assay size for the patient, and can make sure the "cold chain" is intact—correct temperature is preserved from the time the product leaves the manufacturer. That's important because most requests come from countries in the tropics. Without a license, nonprofit status or designated program, you could find yourself in a "gray market" of factor distribution, outside the law—not a great place to be!

Who's doing the asking? In an effort to "do good," Americans can be naïve. A friendly person from a developing country on Facebook befriends you, chats with you, shares photos...and then asks for factor. It's exciting to offer help. But what do you really know about this person?

Does he have hemophilia? Which type? Has he been properly tested? Many countries don't have proper screening to accurately determined factor deficiency and levels. Can he administer factor by himself? Who is supervising his care? If he lives far from a hospital, he may not be under a doctor's regular supervision. In many developing countries, few hospitals specialize in hemophilia care, and most patients do not self-infuse. Do you want to risk sending an injectable drug to someone you know almost nothing about?

What will happen to your factor if it does arrive? Will it be used by the patient, or sold to other patients? Sound cynical, but once the factor leaves your hands, it's out of your control and enters the grey market, where anything can happen. Factor is like gold in developing countries: sometimes it brings a hefty price that can pay for food or rent. Deep need can bring deep desperation.

Whose factor is it anyways? If you want to donate a few vials of your child's in-date factor to help impoverished children, don't do it. Donating factor meant for you or your child, without a valid medical reason, is insurance fraud—a felony in most states, punishable by a fine and prison time.

Before you take any action to donate factor, contact project SHARE. They are the US experts in screening international requests for factor. They do extensive background checks on each patient, his physician, the national hemophilia organization, and the country's customs requirements. If you want to donate factor, talk to them first. They will ensure that it's done properly and legally.

They are also the experts on factor shipping. From one vial to a million IU at one time, they know how to package and process the paperwork, ship it and track it. And they follow up to make sure the factor is used properly.

Americans are generous, but they need to know that donating factor isn't simple. When you donate through project SHARE, you can make your compassion work for you and for those you're trying to help—efficiently, effectively and expertly.

For more information on project SHARE, go to www.kelleycom.com/projectshare/ or call 978-352-7657.