

# Hemophilia OUTLOOK

A Newsletter of

The Hemophilia Association of New York, Inc.

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❄️ **WINTER 2010/2011** ❄️



**DON'T FORGET!**

**SUPPORT THE  
ANNUAL APPEAL**

Please use the enclosed  
donation form.

## COMPREHENSIVE CARE

The concept of comprehensive care is to treat the whole person and the family, through continuous supervision of all the medical and psychosocial aspects of bleeding disorders. Comprehensive care is total care because every facet of the person is addressed, including their physical, emotional, psychological, educational, financial and vocational factors.

The development of comprehensive care over the past 30 years, has greatly improved the quality of life for people with bleeding disorders, helping them to be more independent and productive. The treatment center care network has also lowered patients morbidity and provided cost-effective care in the long term.

*(Continued to page 6)*

## INTRODUCTION...

The Hemophilia Association of New York welcomes Linda E. Mugford as our new Executive Director. Ms. Mugford holds degrees in Business Administration and Organizational Management, and has 25 years experience in not-for-profit and health-related careers.

As former manager of Comprehensive Hemophilia Services of New York Blood Services, Ms. Mugford not only brings knowledge of the issues facing those with bleeding disorders, but has a track record of action, working with both consumers and health care providers, and as an advocate for affordable access to medical treatment.

Ms. Mugford joined the staff in mid-November and will assume the full responsibilities of the position when Thom Harrington retires in early 2011.

**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.**

## **SUMMER CAMPS FILL UP EARLY**

OUTLOOK readers know how enthusiastic we and the parents of former campers are about the developmental benefits camp offers to children with chronic disorders. Campers have had wonderful experiences at the three specialty camps nearest to our area, listed below. All three provide on-site medical care and supervision.

Transportation and travel arrangements to camp are the responsibility of the camper's family. HANY offers help with "camperships" and the cost of transportation for those who require it.

### **Camp High Hopes**

**Where:** Aldersgate Camp, Brantingham, N.Y.

**Ages:** 7-17

**Tuition:** free

Sessions are available for boys and girls in August 2011.

Siblings and carriers are welcome if space is available.

For more information about attending or volunteering at camp, call 315-463-5354.

### **Double "H" Ranch**

**Where:** Lake Luzerne, N.Y.

**Ages:** 6-16. Coed.

**Tuition:** Free.

Application deadline: April 15, 2011.

Call 518-696-5676 for applications or information on volunteering.

### **The Hole in the Wall Gang Camp**

**Where:** Ashford, CT.

**Ages:** 7-15. Coed.

**Tuition:** Free

Application deadline: April 1, 2011.

For application or volunteering at camp, call 860-429-3444.

## **LAW SIGNED TO IMPROVE ACCESS TO CLINICAL TRIALS**

([www.hemophilia.org](http://www.hemophilia.org))

On October 5, 2010, the Improving Access to Clinical Trials Act (P.L. 111-255) was signed into law. This legislation helps reduce barriers for certain people wanting to participate in clinical trials. Often, clinical trial sponsors compensate participants financially for their time. The money is regarded as personal income for tax purposes. This payment, however, may threaten the eligibility of some people to be enrolled in Medicaid or to collect Supplemental Security Income. With a small number of potential trial participants, and the possible loss of Supplemental Security Income and Medicaid benefits for many who wish to participate, clinical trial research for rare diseases and conditions becomes exceptionally difficult and may hinder research on new treatments and potential cures. The new law will allow compensation up to \$2,000 to be excluded from taxable income, thereby making it easier for people to participate in clinical trials.

Biogen Idec Hemophilia now has two long-lasting, fully-recombinant product candidates in Phase 2/3 global clinical trials:

*\*A-LONG, Study of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in Individuals with Hemophilia A*

More info at

<http://www.clinicaltrials.gov/ct2/show/NCT01181128?term=rFVIII Fc&rank=2>

*\*B-LONG, a Study of Recombinant Factor IX Fc Fusion Protein (rFIX Fc) in Individuals With Hemophilia B*

More info at

<http://www.clinicaltrials.gov/ct2/show/NCT01027364?term=b-Long&rank=1>

## **HANY SCHOLARSHIP PROGRAM**

The Association has again renewed its scholarship program. For the 2011-2012 academic year the maximum award set by the Board of Trustees will be \$7,000, although lesser amounts may be awarded.

The program is for qualifying full-time study at an accredited college, university, graduate school, or certified trade or vocational school for the coming school year. Applicants will be judged on their achievements and financial need.

HANY scholarships are available to persons with inherited genetic bleeding disorders (as diagnosed by a board-certified hematologist) and their children. Considerations will also be given to females who are carriers attested to by a board-certified hematologist.

Applicants must be registered with HANY and live within the fourteen counties of southeastern New York (the five boros of New York City, Long Island, Dutchess, Orange, Putnam, Rockland, Sullivan, Ulster and Westchester counties).

June 3, 2011 is the deadline for applying. Call HANY for an application at 212-682-5510.

## **OTHER SCHOLARSHIPS AVAILABLE**

Pfizer pharmaceutical is offering Soozie Courter hemophilia scholarships to undergraduate, graduate and vocational school students. For more information, visit [www.hemophiliavillage.com](http://www.hemophiliavillage.com).

For a full list for the various scholarship programs look at the website of LA Kelley Communication ([www.kelleycom.com](http://www.kelleycom.com)) or call HANY.

## **TAX DEDUCTION REMINDER**

If you itemize your tax return remember that medical and many healthcare-related expenses that cumulatively exceed 7.5% of your adjusted gross income are deductible. There is a long list of items which are deductible (check Internal Revenue Service publications or visit [www.irs.gov](http://www.irs.gov)), including your out-of-pocket cost for prescriptions, health insurance premiums and health-related transportation.

If you used your personal auto to get to doctors, a pharmacy, therapist or other medical destination, you may deduct 16.5¢ (.165) per mile driven from January to December 2010. If you traveled by taxi, bus or other transport, that verifiable cost is also deductible.

## **VICTORY FOR WOMEN WITH BLOOD DISORDERS**

The National Hemophilia Foundation (NHF) announced their new women's health and bleeding disorder initiative – *Victory for Women with Blood Disorders*, an endeavor that builds upon and expands the former Project Red Flag. *Victory for Women with Blood Disorders* program will incorporate education, advocacy, and support for women diagnosed with a blood disorder as well as raising awareness among women who have not yet been diagnosed. For more information on this program visit [www.hemophilia.org](http://www.hemophilia.org).

***Our Best Wishes  
for a Happy and  
Healthy New Year!***

**PLANTS HELP PREVENT  
INHIBITORS AND ALLERGIC  
REACTIONS**

*(NHF eNotes, November 2010)*

Using genetically modified plants, researchers from two Florida universities are developing a technique that could help prevent treatment-related complications such as inhibitors, an immune reaction that neutralizes infused factor, and anaphylaxis, severe allergic reactions, in people with hemophilia B. The lead authors of the study were Dheeraj Verma, PhD, Department of Molecular Biology and Microbiology, College of Medicine, at the University of Central Florida (UCF) in Orlando and Babak Moghimi, MD, Department of Pediatrics, College of Medicine, at the University of Florida (UF) in Gainesville.

Inhibitors result in approximately 25% of patients with hemophilia A and up to 4% of patients with hemophilia B. Clinicians often use immune tolerance (IT) induction to eliminate an inhibitor. By administering daily doses of factor over time, the body begins to tolerate the therapy. The process is similar to desensitization therapy used to treat food and environmental allergies. The technique is less effective in individuals with hemophilia B than in those with hemophilia A. In addition, because of the large amounts of factor used, IT becomes very expensive. The approach being developed by Verma, Moghimi and colleagues could be more cost effective.

The researchers used a so-called “gene gun” to insert the genetic material that manufactures factor IX (FIX) into chloroplasts, the energy production centers of plants. They then fed the modified plants to mice with hemophilia B for a prolonged time period. Insulated from digestive acids and enzymes by durable plant cell walls, the

FIX protein traveled through the stomach and into the small intestines. Once inside the small intestines, bacteria then broke down the cell walls and released the protein, which induced tolerance by the immune system.

“We have made them develop tolerance, and removed the allergic part of this treatment,” said coauthor Henry Daniell, PhD, a Pegasus professor and University Board of Trustees Chair in the College of Medicine at the UCF.

Later the mice were infused with factor product, which triggered little to no inhibitor responses and no anaphylactic events. “I think this is a milestone — nobody has previously achieved such levels of robust immune tolerance by any means using a noninvasive procedure,” explained Thierry Vandendriessche, PhD, an associate professor of medicine at the University of Leuven in Belgium, who was not involved in the study. He is president of the European Society of Gene Cell Therapy.

Investigators will conduct follow-up studies to test the approach in mice with hemophilia A and then carry out trials in humans using lettuce to produce the therapeutic proteins. “We’re hoping that our research will, in the future, result in better and more cost-effective therapies,” said study co-author Roland Herzog, PhD, an associate professor of pediatrics, molecular genetics and microbiology in the UF College of Medicine and a member of the UF Genetics Institute.

The study, “Oral Delivery of Bioencapsulated Coagulation Factor IX Prevents Inhibitor Formation and Fatal Anaphylaxis in Hemophilia B Mice,” was published in the April 2010 issue of the Proceedings of the National Academy of Sciences.

## **DRUG RECALLS**

([www.patientnotificationsystem.org](http://www.patientnotificationsystem.org))

*Please sign up for the Patient Notification System (PNS) to be notified directly about the latest recall or withdrawal of recombinant and plasma products. The system is confidential and time sensitive. It is administered by an independent third-party organization and is free of charge.*

### **About the Patient Notification System**

#### *Ensuring Confidentiality*

Maintaining registrant confidentiality was one of the primary considerations in developing the system. An advisory panel made up of representatives from consumer groups helped design the system to safeguard sensitive registrant information. To ensure confidentiality, the Patient Notification System is operated by Stericycle, Inc. an independent organization that specializes in pharmaceutical notifications.

#### **All registrant information will be held in strict confidence by Stericycle, Inc.**

#### *Free to Consumers*

There is no fee to participate in the Patient Notification System. The system is funded by the manufacturers of plasma-derived and recombinant analog therapies and is free to consumers and health care providers

#### *Cooperative Effort between Industry and Consumers*

Key consumer groups, including Alpha-1 Association, Alpha-1 Foundation, Canadian Blood Services, Committee of Ten Thousand, Hemophilia Federation of America, Immune Deficiency Foundation, and National Hemophilia Foundation, worked closely with the plasma protein therapeutics industry in designing the Patient Notification System. The system is

administered by PPTA and is supported and funded by all major plasma-derived and recombinant analog therapy manufacturers and distributors, including: Baxter Bioscience, Bayer Healthcare, Cangene Corporation, CSL Behring, Grifols USA Inc., Novo Nordisk Pharmaceuticals Inc., Octapharma USA Inc., Octapharma Canada Inc., Talecris Biotherapeutics, and Wyeth. An advisory panel made up of consumer groups and industry representatives provides input on the system and makes additional recommendations for future enhancements.

#### *How the System Works*

Anyone interested in participating registers with the Patient Notification System and provides general contact information, including their preferred method of notification. Registrants have the opportunity of being notified by email, telephone, or fax, whichever is most convenient for them. Please consider email as your method of notification for the following reasons: instantaneous, trackable, accessible, and even on travel. If a therapy is withdrawn or recalled, the company involved immediately contacts Stericycle, Inc. which then directly notifies the registrant. Every effort will be made to notify registrants within 24 hours. Each registrant will also receive a letter by first-class mail to ensure receipt of the information.

In addition, consumers can go online to [www.patientnotificationsystem.org](http://www.patientnotificationsystem.org) or call a 24-hour, toll-free number (1-888-UPDATE-U) for current information on therapy recalls or withdrawals. To maximize the usefulness of the system, it is important for consumers to keep accurate infusion logs and record the lot number, therapy name, and manufacturer for all therapies used.

*For additional information contact HANY's office at 212-682-5510.*

## **COMPREHENSIVE CARE**

*(Continued from page 1)*

### *Hemophilia Treatment Centers*

Having a chronic disease means spending a lot of time and energy negotiating the health care system. The hematologists, nurses, psychosocial professionals and physical therapists not only help consumers with their medical care issues, but also lend tremendous emotional support. Many people with bleeding disorders use the resources of their hemophilia treatment center (HTC) for many years because the staff understands their unique needs.

*Members of the care team at HTCs include:*

- *Hematologists* are specialists in blood disorders.
- *Pediatricians* are specialists in caring for infants, young children, and teenagers.
- *Nurses* are medical specialists in hemophilia care. The nurse is probably the person you will see most frequently.
- *Social Workers* are specialists who assist you with the issues of daily living, such as adjusting to hemophilia and locating resources (e.g., insurance, transportation, housing, etc.).
- *Physical therapists* are specialists in activity, exercise, and rehabilitation.
- *Orthopedists* are specialists in disorders of the bones and joints.
- *Dentists* are specialists in disorders of the teeth and gums. The dentists at HTCs are experts in treating children with oral bleeding problems.
- *You* are also an important member of the treatment team. The staff needs your input to develop a plan of care that will ensure you remain healthy, active, and able to live successfully with added challenge of hemophilia.

*\*Improved access:* HTCs offer extensive outreach and assistance for uninsured and other underserved populations. HTC

comprehensive care provides access to the latest treatments, information, research, and clinical trials.

*\*Low-cost medication management:* Select nonprofit, HTC-based pharmacies provide factor replacement products and other therapies, often at prices much lower than commercial providers. Treatment costs of blood products may be reduced through a federal discount program available at many HTCs.

*\*Family-centered care:* HTCs work closely with families to educate and encourage adherence to care plans and treatment regimens.

*\*Diagnosis:* Specialized diagnostic services include the availability to coagulation laboratories, personal and family medical histories, viral testing and hepatitis vaccines, and genetic testing and counseling.

*\*Research:* HTCs participate in CDC's surveillance program to monitor for bloodborne infections and to collect data to measure health outcomes. In addition, HTCs conduct clinical trials to find new therapies for people with bleeding disorders.

*\*Advocacy and education:* HTC staffs provide advocacy and education in a number of ways:

- Collaboration with local consumer organizations
- Community outreach to underserved population
- Patient and community education on state-of-the-art practices in bleeding disorder management
- Financial counseling and advocacy with insurance payers

*\*Information provided by Centers for Disease Control and Prevention (CDC).*

*For Hemophilia Treatment Centers out of our immediate area, call HANY at 212-682-5510.*