

**in this
issue:**

MEDICAL NEWS	1
LEGISLATIVE UPDATE	2
BLOOD SAFETY NEWS	3
NHF NEWS	4

MEDICAL NEWS

June 7, 2004

HIV Vaccine Trial Initiated

The Walter Reed Army Institute of Research (WRAIR) has initiated a phase I clinical trial to assess the safety and immunogenicity of a HIV vaccine based on Avant Immunotherapeutics' Therapore technology. The trial will evaluate the vaccine in 18 healthy adult volunteers at three escalating dose levels. The trial, to be conducted in conjunction with the Division of AIDS, the National Institute of Allergy and Infectious Diseases, the National Institutes of Health and the US Department of Health and Human Resources will be placebo-controlled and conducted at the WRAIR Vaccine Clinical Research Center in Rockville, Maryland. The trial is being conducted under a cooperative research and development agreement between Avant and WRAIR. Therapore uses bacterial toxin proteins to deliver target antigens into human cells to induce significant cell-mediated immune responses.

Source: Pharma Business Week.

June 3, 2004

Research on Immune Responses to AAV and FIX Presented by MASAC Member

At the annual meeting of the American Society of Gene Therapy (ASGT), research was presented on the results of clinical trials using Adeno Associated Virus (AAV) vectors and an alternative therapy for HIV. Results from a study in which Katherine High, MD, of the Children's Hospital of Philadelphia, and colleagues infused two hemophilia B patients with a dose of AAV-FIX showed levels of 3% and nearly 12% of newly produced FIX levels. After several months, the levels dropped to undetectable levels. "The goal of future research is to determine how the immune response of the patients contributed to the elimination of FIX producing cells, in the hope to determine whether patients can achieve therapeutic FIX levels," says a release by the ASGT. High is a member of NHF's Medical and Scientific Advisory Council and co-chair of the organization committee for NHF's upcoming Novel Technologies and Gene Transfer conference.

Another study presented at the ASGT annual meeting was on an alternative therapy for HIV by Boro Dorpolic, PhD, of Johns Hopkins School of Medicine. He and his colleagues removed the viral genes from the HIV virus and substituted a DNA sequence that is complementary to the HIV RNA, called an antisense gene. In the first two patients, preliminary analysis has shown a significant reduction in the amount of HIV in their blood stream to nearly undetectable levels.

Source: *The American Society of Gene Therapy*.

June 2, 2004

Preclinical Data Suggest Potential for Oral Approaches to HCV Treatment

At the International Conference for Antiviral Research, researchers for Vertex Pharmaceuticals Inc., presented preclinical data on an investigational antiviral therapy for the hepatitis C virus (HCV). The researchers presented data showing that when dosed in combination in HCV replicon cells, merimepodib, Vertex's lead oral HCV therapy, and VX-950, an investigational protease inhibitor, enhanced antiviral activity.

Source: *Biotech Week*.

June 1, 2004

Scientists Describe Immune Responses in Patients with HCV and HIV

US scientists studying the virus dynamics and immune responses during treatment in patients with hepatitis C virus (HCV) and HIV coinfection found data to suggest that interferon efficacy at blocking virion production is correlated with treatment outcome; immunodeficient patients are able to respond to standard IFN-alpha; and "both innate and adaptive

immune responses may be important determinants of HCV RNA decline in response to interferon." The study can be found in the *Journal of Acquired Immune Deficiency Syndromes*, 2004;35(2):103-113.

Source: *Virus Weekly*.

LEGISLATIVE UPDATE

May 30, 2004

Ireland HCV and HIV Compensation Tribunal Report Claims

According to a report by Ireland's Hepatitis C and HIV Compensation Tribunal, more than half a billion euros (over \$600 million US dollars) in legal fees and compensation claims have been paid to 1,763 victims of blood tainted with hepatitis C (HCV) and HIV since 1996. New claims are still being received and nearly 800 are awaiting settlement following a 1997 admission of failures by the Irish Blood Transfusion Service Board that led to an infected blood supply. Four hundred patients with hemophilia and others who received blood transfusions with HCV and HIV and their spouses, long-term partners and dependants have received compensation.

Source: *Agence France Presse*.

June 18, 2004

NHF and NHLBI Post Request for Applications

After announcing its partnership for sponsorship of a collaborative research program to support studies leading to improved treatment and a cure for hereditary bleeding disorders last month, NHF and the National Heart, Lung, and Blood Institute (NHLBI) have listed the request for applications on the National Institutes of Health Guide for Grants and Contracts Web site. The requests can be viewed at <http://grants.nih.gov/grants/guide/rfa-files/RFA-HL-04-032.html>.

NHF and NHLBI each intend to commit \$6 million over four years to fund 8 to 10 grants from fiscal year 2006 to fiscal year 2009. NHLBI support will be provided by the National Institutes of Health (NIH) Research Project Grant (R01) award mechanism, while NHF monies will be drawn from the It's Time for a Cure campaign. NHF will continue to fund Career Development Awards and Judith Graham Pool Postdoctoral Research Fellowships, but will discontinue its Laboratory Grant program.

To learn about how to register your family to accept donations for the It's Time for a Cure campaign, visit www.hemophilia.org/cure or call 1-866-NHF-CURE for more information on how to donate.

July 7, 2004

NIH Article Reviews the Challenge of Emerging Infectious Diseases

The National Institutes of Health issued a news release on July 7, 2004, on the continual challenge of emerging infectious diseases. The release discusses an article written by David Morens, MD, Gregory Flokers, MS, MPH, and National Institute of Allergy and Infectious Diseases Director Anthony Fauci, MD, which appears in the current issue of *Nature*.

Broadly based prevention strategies, as well as new and improved countermeasures, including surveillance tools, diagnostics, therapeutics and vaccines, must continually be tested, refined and upgraded," says Fauci. "This will require a strengthened relationship between public health and basic and clinical science."

The authors outline three types of emerging infections (newly emerging, re-emerging/resurging and deliberately emerging) and consider methods for their control, while asking the question "Why do infectious diseases emerge and re-emerge?"

To read the release, visit <http://www.nih.gov/news/pr/jul2004/niaid-07.htm>.

BLOOD SAFETY UPDATE

July 13, 2004

FDA's Blood Products Advisory Committee Will Hear Key Updates at July Meetings

On July 22, 2004, the Food and Drug Administration's Blood Products Advisory Committee will hear updates on the current thinking of transfusion-related acute lung inflammation and donor blood pressure determination. The committee will also discuss and provide recommendations on the dating of irradiated blood on the new standards for platelet evaluation and experience with monitoring of bacterial contamination of platelets.

On July 23, 2004, there will be an update on West Nile virus. The committee will also hear presentations, discuss and provide recommendations on hepatitis B virus nucleic acid testing for mini-pools. There will also be an informational

presentation on current trends in plasma product manufacturing.

The meeting will be held at the Holiday Inn Gaithersburg in Gaithersburg, Maryland.

For more information, visit <http://a257.g.akamaitech.net/7/257/2422/06jun20041800/edocket.access.gpo.gov/2004/pdf/04-13727.pdf>.

June 1, 2004

Pan European Blood Safety Alliance Founded

The Pan European Blood Safety Alliance was founded in Paris to raise awareness about the importance of blood safety, to advocate for increased blood safety in Europe and to promote the rights of all patients to receive the safest blood and blood products available. The founding members of the alliance come from the European Haemophilia Consortium, Haemoglobinopathy Association of Counsellors, European Cancer patient Coalition, Thalassemia International Federation, Foundation of the Promotion of Bone Marrow Transplantation Switzerland, European Organization for Rare Disorders, Hepatitis Aid Austria, International Myeloma Foundation and Sickle Cell Society UK.

Source: *PR Newswire European*.

July 6, 2004**FDA Seeks Comments on Factor VIII Inhibitor Workshop**

The Food and Drug Administration (FDA) announced on July 6, 2004, the opening of a docket to receive information and comments on the November 21, 2003, public workshop entitled "Factor VIII Inhibitors." FDA is opening the docket because there was insufficient time available during the workshop for a full discussion of the many important topics being covered.

Those interested in submitting written or electronic comments on the workshop, related regulatory and scientific issues and comments on information submitted to the docket by other interested parties, should do so by January 6, 2006 to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Room 1061, Rockville, MD, 20852-1448, or electronically to <http://www.fda.gov/dockets/ecomments>. (See the "Supplementary Information" section for electronic and other access to the slide presentations and transcript from the workshop.) Those who have already submitted written comments and information to FDA should resubmit the same comments to the docket to ensure their adequate consideration, as this information was not previously submitted to the docket.

For more information, visit
<http://www.fda.gov/cber/genadmin/fctviii.pdf>.

NHF NEWS

July 6, 2004**Register for NHF's Annual Meeting and Save**

To register online visit hemophilia.org/annualmeeting and learn more about the Annual Meeting, which will be held November 4 to 6, 2004. This year's highlights include:

- ◆ Special educational programs and networking opportunities for parents and new families, women with bleeding disorders and carriers, individuals with bleeding disorders and many others
- ◆ A fun-filled Youth & Adolescent Program with field trips, education and meeting and making life long friends
- ◆ Provider-developed preconference symposia on women with bleeding disorders and other CME and CEU credited sessions for physicians, nurses, social workers and physical therapists
- ◆ A wide variety of social and recreational activities including a final night country western extravaganza with the best barbeque in Texas!

Those who reserve rooms at NHF's headquarter hotel, the Hyatt Regency at Reunion, can take advantage of NHF's group rate of \$169/night (make sure to indicate NHF affiliation when making reservations). Hotel rooms go very quickly so don't forget to make your reservations early before hotel sells out.

Hyatt Regency at Reunion, 300 Reunion Boulevard, Dallas, Texas, direct telephone: (214) 651-1234, toll free telephone: (800) 233-1234.

To make travel arrangements in advance using NHF's official travel agency, call Association Travel Concepts (ATC) at (800) 458-9383. ATC will allow meeting goers to save 10% to 15% on airline tickets (restrictions and service fees may apply).

June 24, 2004**Hemophilia Center Conducting Study on Women with Bleeding Disorders**

The Mary M. Gooley Hemophilia Center is conducting two studies on women with bleeding disorders entitled Open Label Continuation Study for the Use of Cyclokapron® for Treatment and Management of Women with Menorrhagia Related to an Inherited Bleeding Disorder and A Study of the Levonogestrel

Intrauterine Device for Menorrhagia Related to an Inherited Bleeding Disorder. The medication for the first study will be dispensed free of charge through Pharmacia, and the second study is sponsored by the Aventis Foundation. Premenopausal women diagnosed with von Willebrand disease who are interested in participating in the study should call (585) 922-5041 for more information and to see if they qualify.

June 13, 2004

Victory Junction Camp Opens

The Victory Junction Gang Camp near Randleman, North Carolina, opened its doors this month to kids with hemophilia and juvenile rheumatoid arthritis from the Carolinas and Virginia. The 75-acre camp was established to serve children with chronic and life threatening illnesses. Victory Junction has a racing theme and was founded by NASCAR driver Kyle Petty and his wife Patty. This camp is the seventh of the Hole in the Wall Gang Camps, which were founded by actor Paul Newman.

Source: The Associated Press.