FORMERLY COMMUNITY ALERT

NHF e notes

A MONTHLY ONLINE NEWS BULLETIN

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

AUGUST 2004

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MEDICAL NEWS

August 1, 2004

Avigen Remains Optimistic on Prospects for Gene Transfer Therapy

In May of this year, Avigen, Inc., a California-based biotech company conducting research into gene transfer therapies for a variety of conditions including hemophilia B, announced the suspension of clinical trials for its hemophilia treatment and its decision to focus instead on "serious and life threatening neurological disorders, including Parkinson's disease and chronic neuropathic pain." The decision, which was reported by NHF on May 29, 2004, was intended to "make the best use of current financial and scientific resources," according to a company statement. However, the company made clear at the time that they did not believe efforts to date would be wasted, and that discussions with current and potential funding partners for additional hemophilia research would continue. Since that time, communication between NHF leadership and Avigen representatives have maintained an encouraging tone. "Furthering our research on the gene therapy technology will enable us to develop next generation clinical trials for hemophilia and other diseases during the next one to two years," says Glenn Pierce, PhD, MD, vice president for Research and Clinical Development for Avigen and a past NHF president. Similarly, a recent company statement said that they "remain optimistic that with adequate time and resources, a cure for hemophilia B will be found." NHF leadership will be continuing a dialogue with Avigen representatives and their funding partners to help ensure that resources will be available and research can continue. In addition, NHF will continue to raise and distribute funds though the "It's Time for a Cure" campaign to support research into a wide variety of potential cures for bleeding disorders.

July 29, 2004

Factor and Supply Donations Help Kids at Summer Camp

Many pharmaceuticals donate factor, portable coolers and other supplies to summer camps for kids with hemophilia during the summer months. For the last eight years, Wyeth has donated supplies of clotting factor through its Summer Camp Product Donation Program. In 2003 alone, 26 camps received up to 30,000 units each of ReFacto and BeneFix. Applications for the donations can be obtained from local Wyeth sales representatives. If the representative is unknown, call the Wyeth Hemophilia Hotline at (888) 999-2349.

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July 12, 2004

Hepatitis C Treatment Recommended for Shorter Course of Therapy

Schering Plough recently announced that the European Medicines Agency's Committee for Medicinal Products for Human Use issued a positive opinion for the approval of a shorter, 24-week course of peginterferon alfa-2b and ribavirin combination therapy for the treatment of hepatitis C virus genotypes 2 and 3. Previously, peginterferon alfa-2b was approved in the European Union for a 48-week course in combination with ribavirin. The safety of effectiveness of the 24-week treatment was compared with that of historical control patients receiving the 48-week course.

Source: R&D Focus Drug News.

July 11, 2004

New Method Enables Researchers to Make Human SARS Antibodies Quickly

Scientists at the National Institute of Allergy and Infectious Disease (NIAID), part of the National Institutes of Health, are developing a cocktail of SARS-specific antibodies that may help protect people who have recently been exposed to the SARS virus or who are at high-risk of exposure. The technique could also make possible the development of a similar approach to prevent or treat other illnesses, such as HIV/AIDS and hepatitis C. The antibodies could be used as a potent defense for healthcare workers and others at high-risk of exposure and as an effective treatment for those newly exposed to the virus.

The report describing these findings appears in the July 11, 2004, online issue of Nature Medicine.

This NIH news release is available online at: http://www.nih.gov/news/pr/jul2004/niaid-11.htm.

July 6, 2004

HIV/HBV Patients at Increased Risk for Virologic Failure after HAART

According to a Taiwanese study, HIV and hepatitis B virus (HBV) coinfected patients had an increased risk for virologic failure and death after highly active antiretroviral therapy (HAART) was initiated. The study was conducted between 1994 and 2003 with 111 patients to assess the impact of HBV infection on outcomes of HIV-infected patients. The study can be found in *Clinical Infectious Disease*, 2004;38(10):1476-1472.

Source: Virus Weekly.

July 6, 2004

FDA Issues Warning Letter to Makers of Norvir

The US Food and Drug Administration's (FDA) Division of Drug Marketing and Advertising Communications recently issued a warning letter to Abbott Laboratories regarding information on its Web site for Norvir (ritonavir capsules) Soft Gelatin capsules. The letter objects to a chart on the site comparing the cost of common anti-retroviral therapies, which might be interpreted to imply that Norvir could be used by itself at a sub-therapeutic dose for the treatment of HIV. The letter also delineated concerns about the presentation of risk information in certain Norvir materials. According to a company release, Abbott will work with FDA to ensure that all Norvir-related materials provide appropriate balance of risk information.

Sources: Company release and Virus Weekly.

July 5, 2004

Anemia in HIV Patients Explored

Members of the Anemia in HIV Working Group, an expert panel of physicians in the care of HIV-infected patients, recently published a study on the preva-

lence of anemia in the highly active antiretroviral therapy era. The study showed that anemia in HIV-infected patients can have "serious implications, which vary from functional and quality-of-life decrements to an association with disease progression and decreased survival." The study can be found in *Clinical Infectious Diseases*, 2004;38(10):1459-1468.

Source: Hematology Week.

July 2, 2004

Caremark Receives Civil Investigative Demands

Caremark Rx, Inc., recently announced that it had received Civil Investigative Demands from the attorney general of Washington state seeking information on business practices. The attorney general also indicated that demands would be issued by 18 additional states. "Caremark believes that its business practices comply in a material respects with applicable laws and regulations," says a company release.

The request comes two months after a $29 million settlement was paid by Medco Health Solutions over allegations it pressured doctors to switch patients' medications to benefit the company. A spokesman for the Washington attorney general said the type of request that was sent to Caremark was spawned by the Medco case.

Sources: Company release and Associated Press.

June 26, 2004

Trial Demonstrates Effective Treatment of Intracerebral Hemorrhage

Results from a clinical trial conducted by Novo Nordisk of intracerebral hemorrhage showed that treatment with NovoSeven significantly improved neurological and functional outcomes. As a result of the trial's success, which involved 400 patients in 20 countries, Novo Nordisk will immediately begin to seek approval from regulatory agencies for the treatment.

Source: Company release.

June 21, 2004

Elevated Plasma Levels Linked to Increased Recurrent Early Pregnancy Loss

According to an Austrian study, elevated maternal plasma levels of factor VIII are associated with an increased risk for recurrent early pregnancy loss. Researchers evaluated the relation between the loss and factor VIII levels in 49 unrelated women with a history of two to six early pregnancy losses. The study can be found in *Thrombosis and Haemostasis*, 2004;91(4):694-699.

Source: OBGYN & Reproduction Week.

June 18, 2004

Australian Senate Establishes Hepatitis C Fund

Without establishing an actual compensation claims process, the Australian Senate recommended that a fund be established to help people with hepatitis C "access health and related services." The action disappointed many who were calling for a victims' compensation fund and declined to pinpoint actual blame for the spread of the virus before the nation's blood was tested for it.

In March 2004, NHF issued a statement regarding compensation and improved treatment research for patients with hepatitis C. To read the statement, visit www.hemophilia.org/News/nhfnews/nn_03_08_04a.htm. Also, look for articles on hepatitis C compensation and advocacy in the November/December issue of *HemAware*.

Source: Sydney Morning Herald.

LEGISLATIVE UPDATE

July 22, 2004

Hearing Examines Genetic Non-Discrimination and its Implications for Workers and Employers

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The House Employer-Employee Relations Subcommittee's Education and Workforce Committee held a hearing on July 22 to examine the current federal and state laws and regulations that govern genetic discrimination, privacy and the use of genetic information in employer-sponsored health plans. The hearing was intended to "provide an overview of this complex area of law and science to ensure that any legislation enacted is precise and measured in its impact."

While workers and employers agree that employment decisions should be based on the qualifications of employees, not on genetic factors, questions remain on the issue including: the extent to which current laws such as the Americans with Disabilities Act, HIPAA and state laws already protect individuals with genetic predispositions toward illnesses from employment discrimination; the extent to which genetic testing is practiced by employers; what enforcement mechanisms and penalties apply; and what are the unintended consequences of overly broad definitions of genetic information and testing.

For Further Information on this hearing, visit: http://edworkforce.house.gov/mediaadvisory.htm.

July 19, 2004

New Database Focuses on Genetic Policy and Laws

The National Human Genome Research Institute, part of the National Institutes of Health (NIH), unveiled a new Web-based resource that will enable researchers, health professionals and the general public to more easily locate information on laws and policies related to a wide array of genetic issues. The free data currently focuses primarily on the following subject areas: genetic testing and counseling; insurance and employment discrimination; newborn screening; privacy of genetic information and confidentiality; and commercialization and patenting. The resource allows users to view state legislation for any of the 50 states and the District of Columbia.

The NIH news release is available at: http://www.nih.gov/news/pr/jul2004/nhgri-19.htm.

The database is available at: http://www.genome.gov/LegislativeDatabase.

July 14, 2004

Health and Human Services Appropriations Bill Passes Committee
The House Committee on Appropriations passed the Labor, Health and Human Services and Education Bill, allocating $492 billion for mandatory spending and $142 billion for discretionary spending. Under the House version, the National Institutes of Health will be provided a $28.5 billion budget, $727 million more than fiscal year 2004. The Centers for Disease Control and Prevention will be funded at $4.48 billion, $101 million less than last year. The Ryan White AIDS program is increased by $35 million over fiscal year 2004, with total funding of $2.1 billion.

The Senate will take up its version of the bill in September when they return from recess.

Highlights of the Appropriations Bill are available at: http://appropriations.house.gov/index.cfm?FuseAction=PressReleases.Detail&PressRelease_id=410.
Source: BNA Health Care Daily.

July 13, 2004

NIAID Expands West Nile Virus Treatment Trial
The National Institute for Allergy and Infectious Diseases (NIAID) has expanded its clinical trial of an experimental West Nile Virus (WNV) treatment to about

60 sites throughout the United States and Canada. The study is testing the safety and preliminary effectiveness of using a product containing WNV infection-fighting proteins, or antibodies, to treat people whose infection has reached or threatens to reach the brain. NIAID is trying to assess the safety of a blood plasma-derived substance containing WNV antibodies when given intravenously to patients with WNV infection. The trial can enroll up to 110 patients who have WNV-related encephalitis or are at risk of developing this severe neurological complication.

This NIH news release is available online at:
http://www.nih.gov/news/pr/jul2004/niaid-13.htm.

BLOOD SAFETY NEWS

July 22, 2004

Second Possible Transmission of vCJD Via Blood Reported in the UK
According to the United Kingdom's Department of Health, a second case of possible transmission of variant Creutzfeldt-Jakob Disease (vCJD), the human version of Mad Cow Disease, was reported when an autopsy of a patient who died of symptoms unrelated to the disease revealed the vCJD agent in their spleen. The patient had received a transfusion in 1999 from a donor who later developed vCJD. In December 2003, the UK announced a patient was reported to have died after receiving blood from an infected donor. That case was the first-known, person-to-person transmission of vCJD in the world. Following that case, the UK government banned anyone who had received a blood transfusion since January 1980. In response to the most recent case, the ban will be extended to donors who are unsure if they have previously received a blood transfusion and those donors who frequently donate blood where machine processing removed only certain blood components and the rest is returned to the donor (these are called apheresis donors).

"Although people may have concerns about the implications of this announcement, I would emphasize again that the exclusion criteria are being tightened because of a small but unquantifiable risk," said Health Secretary John Reid. "People should continue to have a blood transfusion when it is really necessary."

On July 28, 2004, the World Federation of Hemophilia released a statement regarding this case. "The risk of such occurrences appears to be predominately a risk for recipients of blood components collected in the UK," says the release. "Donor screening procedures and blood banking policies initiated in countries throughout the world appear to be appropriate to reduce the risk of such transmissions to a minimum." To read the release in its entirety, visit www.wfh.org/ShowDoc.asp?Rubrique=30&Document=374.

Sources: British Broadcasting Corporation and World Federation of Hemophilia.

July 16, 2004

FDA to Hold Workshop on Plasma Standards

From August 31 to September 1, 2004, the Food and Drug Administration's Office of Blood Research and Review will hold a workshop to aid in the development of standards for plasma that the agency says will address "regulatory concerns encountered over the years with preparation, shipment and use of plasma both for transfusion and in the manufacture of blood products such as factor VIII and IGIV." A major objective of the workshop is to gather information on the current industry practices for the manufacture of plasma.

The agenda is available at:
http://www.fda.gov/cber/meetings/plasma083104.htm.

July 9, 2004

USDA and HHS to Strengthen BSE Safeguards

Health and Human Services (HHS) Secretary Tommy Thompson and US Department of Agriculture (USDA)

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Secretary Ann Veneman announced on July 9, 2004, three actions being taken to further strengthen existing safeguards that protect consumers against the agent that causes bovine spongiform encephalopathy (BSE, also known as "mad cow disease").

The actions include:

o A joint USDA Food Safety and Inspection Service, USDA Animal and Plant Health Inspection Service and Food and Drug Administration (FDA) notice asking for public comment on additional preventive actions that are being considered concerning BSE.

o An interim final FDA rule that prohibits the use of certain cattle-derived materials in human food (including dietary supplements) and cosmetics. These high-risk cattle-derived materials include specified risk materials that are known to harbor concentrations of the infectious agent for BSE, such as the brain, skull, eyes and spinal cord of cattle 30 months of age or older, and a portion of the small intestine and tonsils from all cattle, regardless of their age. Prohibited high-risk bovine materials also include material from non-ambulatory, disabled cattle, the small intestine of all cattle, material from cattle not inspected and passed for human consumption and mechanically separated beef.

o A proposed FDA rule on recordkeeping requirements for the interim final rule relating to this ban.

FDA has reached a preliminary conclusion that it should propose to remove specified risk materials from all animal feed and is currently working on a proposal to accomplish this goal. Comments on these issues raised in the advanced notice of proposed rulemaking are due to FDA next month.

FDA News Release is Available at:
http://www.fda.gov/bbs/topics/news/2004/new01084.html

FDA Interim Final Rule: http://www.fda.gov/OHRMS/DOCKETS/98fr/04-15881.htm

FDA Proposed Rule: http://www.fda.gov/OHRMS/DOCKETS/98fr/04-15880.htm

FDA/USDA Advance Notice of Proposed Rulemaking:
http://www.fda.gov/OHRMS/DOCKETS/98fr/04-15882.htm

NHF NEWS

August 3, 2004

Get Out the Song Web Site to Debut

The *Get Out the Song!* Web site will be up and running in mid-August so participants can register to sing with the stars in either New York, Southern California, Atlanta, Orlando, Boise or Salt Lake City. The event will support millions of men, women and children with bleeding disorders. Venues and times will be announced for this, NHF's first nation-wide event, which will be held on March 5, 2005. Check in frequently for new news about performers, top fundraising teams, top fundraising singers, what's going to happen in each city and more. Form a team of friends or coworkers or register as an individual at www.hemophilia.org/song or www.getoutthesong.com. Call (866)-424-SONG for more information.

August 2, 2004

Online Registration Now Available for NHF's Annual Meeting

Register online for NHF's 56th Annual Meeting in Dallas. Visit hemophilia.org/annualmeeting to register and learn more about the Annual Meeting, which will be held November 4 to 6, 2004. This year's highlights include:

o Special educational programs and networking opportunities for par-

ents and new families, women with bleeding disorders and carriers, individuals with bleeding disorders and many others

o A fun-filled Youth & Adolescent Program with field trips, education and meeting and making life long friends

o Provider-developed preconference symposia on women with bleeding disorders and other CME and CEU -redited sessions for physicians, nurses, social workers and physical therapists

o 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 quickly so don't forget to make reservations early.

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).

Come join the fun and be in Dallas to celebrate 56 years of NHF's Annual Meetings!