



MEDICAL NEWS	1
LEGISLATIVE UPDATE	4
NHF NEWS	6
COMMUNITY NEWS	8

MEDICAL NEWS

May 28, 2004**Opperbas Holding Conducting Breakthrough Clinical Study**

Opperbas Holding BV announced last week that it is conducting a phase II clinical trial using its proprietary PEGylated liposomes to prolong the half-life and hemostatic efficacy of recombinant factor VIII, a technology developed for Opperbas by the Israeli-based Omri Laboratoires Ltd. research company. This technology has the potential to reduce the number of infusions needed by hemophilia A patients by 33%. This, consequently, could enable more patients to use prophylactic treatment. According to a company release, "In vivo experiments with several proteins indicate that the half-life and biological efficacy of liposome-formulated proteins are significantly prolonged."

Source: Opperbas Holding BV.

May 27, 2004**Baxter Warned about Advate Promotions**

Following a recent warning letter from the US Food and Drug Administration (FDA), last week, Baxter International removed select materials from distribution that were sent to physicians by its sales representatives. According to the Associated Press, "FDA was concerned that [Baxter] had not provided appropriate descriptions of side effects or sufficient supporting data for its statements." A Baxter press release pointed out that the letter "is not related to Advate's performance, clinical data or processing." In addition, Baxter spokeswoman Cindy Resman said that FDA sends out these types of letters to pharmaceutical companies fairly regularly.

Source: The Associated Press and Baxter International.

May 25, 2004**Study Finds Highest Response Rate to HCV Treatment in Black Patients**

A recent study showed that 26% of black Americans with chronic hepatitis C that were treated with a combination of Pegasys (peginterferon alfa-2a) and Copegus (ribavirin) achieved a sustained virological response (SVR). This is the highest response to treatment rate observed in a black population. Thirty-two percent of those who completed 48 weeks of treatment achieved a SVR. These results have led to a larger National

Institutes of Health study involving 400 patients in eight centers throughout the United States.

Source: *The Roche Group company release.*

May 24, 2004

Hepatitis C Adaptation to Disease Progression Reported

Scientists in England recently studied a high-resolution phylogenetic analysis of hepatitis C virus adaptation and its relationship to disease progression. The study can be found in the *Journal of Virology*, 2004;78(7):3447-3454.

Source: *Health & Medicine Week.*

May 19, 2004

World Blood Day Launched in South Africa

The World Health Organization (WHO) announced it will launch a worldwide campaign in Johannesburg, South Africa, in June to increase the number of volunteers who regularly donate safe blood. Many organizations are planning events in conjunction with WHO for World Blood Day on June 14.

Source: UN News Service.

May 19, 2004

Study Shows State AIDS Programs Face Funding Challenges

The recently released eighth annual National AIDS Drug Assistance Programs (ADAPs) Monitoring Report shows that many states are having to take cost-saving measures due to the rising costs of HIV drugs and the increasing number of people with HIV/AIDS who are living longer, despite a 9% increased in the ADAP budget between fiscal years 2002 and 2003. The problem lies in the fact that the need for HIV/AIDS medications exceeds available resources. The report was prepared by the Kaiser Family Foundation, the National Alliance of State and Territorial AIDS Directors and the AIDS Treatment Data Network.

The report shows that waiting lists and other cost containment measures affected client access in 13 states as of April 2004. Eleven states closed enrollment to new clients, many of whom were on waiting lists to receive medication. An additional ten ADAPs anticipated the need to implement additional cost containment measures before the end of the fiscal year (March 31, 2005). The study noted that because each state designs its own program, and there are differences in availability across the country and "what you get depends on where you live."

To qualify for ADAPs, patients must have no other funding source available to them. According to a Kaiser release, each year approximately 136,000 people receive services from ADAPs, representing about 30% of people estimated to be living with HIV/AIDS in the United States. All ADAP programs were created from funding set aside by the Ryan White Comprehensive AIDS Resources Emergency Act, and many also receive state revenue and other funding.

Source: *Kaiser Family Foundation release.*

May 17, 2004

Treatment Protocol Reduces Bleeding Risk for Tonsillectomies

Recently a team of otolaryngologist-head and neck surgeons and hematologists at the University of Virginia in Charlottesville studied the effect of administering desmopressin (DDAVP) to patients with von Willebrand disease (VWD) and those with factor VIII (FVIII) deficiency prior to and following tonsillectomies. The authors concluded that the use of DDAVP preoperatively was an effective pre and postoperative prophylactic regimen for mild to moderate type 1 VWD patients undergoing adenotonsillectomies. Postoperative bleeding was

shown to be reduced in FVIII-deficient patients in the study as well (though, only a small number of these patients were studied).

Source: *Health & Medicine Week*.

May 17, 2004

Children with Hemophilia Using Central Venous Catheters Found to Be at High-Risk for Infection

Swedish scientists recently found a high rate of infections associated with hemophilia patients with inhibitors using implantable central venous access devices. "The final decision to use a central line has to be a compromise between the medical goal, the patient's bleeding tendency, the social situation and the expected risk of complications at the particular hemophilia center," said the researchers. "Some of the complications may be reduced by adequate aseptic measures both during implantation and in the subsequent use and clear basic routines for surveillance of the systems and repeated education of the users." The study can be found in *Blood Reviews*, 2004;18(2):93-100.

Source: *Hematology Week*.

May 8, 2004

Oxford BioMedica Receives Funds of Factor VIII Treatment Trials

Oxford BioMedica, a biopharmaceutical company that specializes in gene therapy, received more than \$900,000 from the British government to conduct a factor VIII gene therapy treatment trial for hemophilia A. The company has developed LentiVector, a technology that uses the equine lentivirus to deliver therapeutic genes to target cells. Oxford BioMedica is the first company to benefit from money that the United Kingdom's Department of Health recently committed to gene therapy research.

Source: *The Pharmaceutical Journal*.

May 6, 2004

Heparin Reverses Preeclampsia in a Factor V Leiden Mutation Carrier

Researchers at the University of Helsinki Central Hospital in Finland reversed preeclampsia in a 29-year-old woman who was a heterozygotic carrier of the

factor V Leiden mutation by giving her low molecular weight heparin therapy (LMWH). The woman's blood pressure normalized, proteinuria diminished and her d-dimer values returned to normal. She carried the child to term and delivered a normal weight, healthy girl.

The study's authors noted that previously, only women with thrombophilia who had been preeclamptic in previous pregnancies had been given LMWH, and then, only prophylactically.

The authors suggested that inherited thrombophilia, spontaneous hyperreactio luteinalis and preeclampsia may be associated problems.

Source: *Blood Weekly*.

May 1, 2004

Study Looks at Sustained Suppression of HCV in Hemophilia Patients

In a recent study by the Hepatitis Study Group of the Association of Italian Hemophilia centers, 34 previously untreated adults with hemophilia and the hepatitis C virus (HCV) were treated with interferon alpha (IFN) and ribavirin and better achieved a greater sustained virologic response than with IFN monotherapy. Patients were given 5 million units of IFN three times a week for six months, followed by 3 mol/L for six additional months in combination with daily oral doses of 1 or 1.2 g of ribavirin.

According to the study, published in *Transfusion*, "Patients showing a negative RT-PCR result for serum HCV-RNA at the end of the 12-month treatment period and at the end of follow up (month 18) were considered to have had a sustained virologic response." Forty-four percent of the patients became HCV-RNA negative during the first six months of therapy and successfully continued the therapy for six additional months with no HCV-RNA detected. Fifty-three patients discontinued treatment after six months because they were nonresponders, 24% of which had normal ALT values but were persistently viremic. Three percent relapsed during the posttreatment follow-up. A reduction in dose was required in 20 patients because of fatigue, neutropenia and hypothyroidism. Overall, 41 percent of patients had a sustained virologic response.

The study noted, "We believe that rates of sustained virologic responses in hemophilic patients with chronic HCV should further increase using the combination of pegylated IFNs and ribavirin because the prolonged half life of these new formulations results in improved pharmacokinetics and pharmacodynamics with more profound and efficient suppression of virus activity than with standard IFNs."

Source: Transfusion. Volume 44, May 2004.

April 30, 2004

Drug Helps Prevent Stillbirth in Women with Thrombophilia

In a recent French study, 160 pregnant women with genetic risk factors for thrombophilia who had experienced one unexplained stillbirth at 10 weeks gestation or later, the anticoagulant enoxaparin was shown to prevent stillbirths. The women began taking 40mg enoxaparin by subcutaneous injection each day in the eighth week of pregnancy. Eighty-six percent of the women had a normal live birth, compared to only 29% of women given aspirin instead of enoxaparin. The enoxaparin babies also had higher birth weights than those born to women taking only aspirin.

A professor of biochemistry at the University of Vermont commented, "Although additional randomized clinical trials are needed before recommending enoxaparin therapy for the routine management of pregnant women with these specific thrombophilia disorders, the compelling results of this study and the relatively benign nature of the therapy support the potential of a wider application in treating individuals with recurrent fetal losses due to genetic thrombophilia."

Source: American Society of Hematology.

LEGISLATIVE UPDATE

May 17, 2004

NORD Holds 2004 Tribute Banquet

On May 17, 2004, the National Organization for Rare Disorders (NORD) held its annual Tribute Banquet in Washington, DC. NORD honored members of Congress, medical researchers, pharmaceutical firms and other individuals and corporations who have improved the lives of people affected by rare diseases. The following individuals and companies were honored:

NORD Health Leadership Awards:

US Senator Mike DeWine (R-OH) is a leading advocate for children on a broad spectrum of issues. He was honored, in particular, for his contributions toward increased research and improved medical treatments for pediatric disorders.

US Senator Christopher Dodd, (D-CT) has cosponsored, with Senator DeWine, legislation promoting the testing of pharmaceuticals in children to ensure that

pediatric dosage and side effects are well understood.

NORD Public Health Leadership Award:

Janet Woodcock, MD, acting deputy commissioner for operations, FDA, and director of the FDA Center for Drug Evaluation and Research

Corporate Awards:

BioMarin Pharmaceuticals, Inc.
Genzyme Corporation

Therapeutic Achievement Award:

Stephen Arnon, MD, California Department of Health Services

May 13, 2004

CDC's Futures Initiative Moves Forward

The Centers for Disease Control and Prevention's (CDC) director, Julie Gerberding, MD, announced the new goals and integrated operations of CDC on May 13, 2004. The Futures Initiative is an ongoing strategic development process that will allow the federal public health agency to have greater impact on the health of people around the world.

Since the announcement of the Futures Initiative in June 2003, CDC has worked with hundreds of employees, other agencies, organizations and the public to align its priorities. Dr. Gerberding announced two overarching health-protection goals:

" **Preparedness:** All people in all communities will be protected from infectious, environmental and terrorists threats.

" **Health Promotion and Prevention of Disease, Injury and Disability:** All people will achieve their optimal lifespan with the best possible quality of health in every stage of life. In addition, the agency is developing more targeted goals to assure an improved impact on health at every stage of life, including infants and toddlers, children, adolescents, adults and older adults.

The integrated organization coordinates the agency's existing operational units into four coordinating centers that will help the agency leverage its resources to be more nimble in responding to public health threats and emerging issues as well as chronic health conditions.

The National Center for Birth Defects and Developmental Disabilities will fall

under the operational jurisdiction of the Coordinating Center for Health Promotion, along with the National Center for Chronic Disease Prevention and Health Promotion (NCCDPHP). Donna Stroup, MD, former assistant director of NCCDPHP, will lead this coordinating center. Gerberding and executive leaders throughout CDC will be moving forward to implement these changes by October 1, 2004, the start of the next fiscal year.

For a complete list of the new coordinating centers and their directors, please visit <http://www.cdc.gov/od/oc/media/pressrel/r040513.htm>.

May 6, 2004

Senate Passes Opens Medicare Door to Families of Disabled Children

On May 6, 2004, the United States Senate voted unanimously to pass the Family Opportunity Act, which provides families of disabled children with the opportunity to purchase medical coverage under the Medicaid program. The act opens Medicaid services to families with incomes two-and-a-half or more times the poverty level. Under the bill, states can charge families a premium not exceeding 5% of their adjusted gross income and in return receive Medicaid services not covered by their private insurance.

Many families with disabled children

have had to face the choice between giving up custody of their child or keeping the family in poverty in order to obtain rehabilitation and other specialized services that Medicaid offers. This bill allows families to step out of poverty while maintaining crucial healthcare services.

The House equivalent of the act was referred to the Committee on Energy and Commerce in April and remained in the Subcommittee on Health as of April 24, 2003.

NHF NEWS

June 6, 2004

NHF Embarks On Major Research Venture with NHLBI

In an unprecedented arrangement NHF and the National Heart, Lung, and Blood Institute (NHLBI) have agreed jointly to sponsor a collaborative research program that will support studies leading to improved treatments for hemophilia, von Willebrand disease and other hereditary bleeding disorders, with the ultimate goal of finding a cure. 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.

The objectives of the new program are to stimulate basic research to improve therapy and enhance understanding of immune response and safety issues related to novel therapeutics, gene transfer or cell-based therapies for bleeding disorders. A one-time request for applications (RFA) entitled "Improved Therapy for Hemophilia" will be released by NHLBI and published in the NIH Guide for Grants and Contracts during June 2004. Applications received by the January 2005 deadline will be assigned a priority score and receive a written critique by an appropriate peer review group convened by NHLBI. The National Heart, Lung, and Blood Advisory Council and NHF will conduct a secondary review of the applications. NHLBI will initially choose which applications it would like to fund and pass the remainder of the top-scoring projects to NHF for consideration. Awards pursuant to the RFA are contingent upon the availability of funds and the receipt of a sufficient number of meritorious applications. The anticipated award date is September 30, 2005.

This initiative is the result of considerable imagination and labor on the part of a number of key NHF volunteers, including Gina Shreve, PhD, former NHF president; Kenneth Mann, PhD, chair of the NHF Research Working Group; W. Keith Hoots, MD, chair of NHF's Medical and Scientific Advisory Council; Glenn F. Pierce, PhD, MD, former NHF president; and Stephen Bender, chair of the NHF Resource Development Committee.

May 29, 2004

NHF Reacts to Avigen Announcement

On May 27, 2004, biotechnology company Avigen announced that it will discontinue its development of a gene transfer therapy for hemophilia. On May 29, NHF released a statement saying that despite Avigen's announcement, NHF remains optimistic about the potential for gene transfer and other investigational therapies for hemophilia and will continue to support such research. "We are confident that breakthroughs in recent years, and those to come, will lead us to a cure for bleeding disorders," said NHF President Richard Metz, MD. "This is a time to redouble our efforts to support promising research so that breakthroughs for hemophilia and all genetic diseases will happen sooner, not later," Metz said.

Avigen's decision to end its hemophilia drug trials was announced in a company press release. The statement quoted Glenn Pierce, PhD, MD, Avigen's vice president of research and clinical development and former NHF president, who

explained that "Due to certain scientific, regulatory and clinical hurdles we believe are specific to hemophilia, discontinuing the Coagulin-B trial makes the best strategic sense at this point." Pierce went on to say that Avigen and its partner, Bayer HealthCare, have "made significant progress in establishing proof of principle that hemophilia gene therapy could work in the clinic." Click here to read NHF's formal press release:

May 22, 2004

MASAC Lauds NHF Board Reorganization

On May 22, 2004, NHF's Medical and Scientific Advisory Council (MASAC) approved a resolution regarding the NHF Board reorganization currently underway. The resolution expressed a "vote of confidence" in the Board's transition team and an appreciation for the prospect of improved communication between the Board and MASAC. MASAC also recognized NHF's office staff for their commitment to the bleeding disorders community.

West Nile Virus Update

June 8, 2004

Mosquito Season Accompanied by Fear of West Nile Virus

As summer draws near, fear of West Nile Virus (WNV) becomes heightened. During the last week of May, both Arizona and New Mexico reported the country's first human cases of the mosquito-borne illness, and officials in Southern California have found 157 birds that had been killed by the disease. Evidence of the disease was also found in a horse in Virginia and in a pool of mosquitoes in Indiana. A health official in Arizona noted that this is early in the year to be seeing patients sick with WNV, which means this season will be longer.

In March 2004, the Centers for Disease Control and Prevention (CDC) quelled many fears when it announced that up to 1,033 blood donors carrying a West Nile infection were identified through a screening process for the virus from late June through November 2003. The screening test has helped health officials map the spread of the disease. A CDC official told attendees, "The entire blood community went from the identification of a problem in the blood supply to the development and implementation of a solution nationwide within a year."

CDC recently launched a mapping program for the 2004 WNV season that tracks cases in the US by geographic region using US Geological Survey technology. The Web site shows the number and location of cases reported to state health departments of human, avian, mosquito sentinel animal and veterinary

WNV. The site can be accessed at: westnilemaps.usgs.gov/index.html.

On a research note, recently in Great Britain, an experimental vaccine against WNV was the first to successfully produce antibodies in 15 human subjects in a small preliminary trial. If future tests show this vaccine to be safe and effective, it could be available to the public in 2007.

For additional information about WNV from the CDC go to: www.cdc.gov/ncidod/dvbid/westnile/.

Sources: America's Blood Center's Newsletter and ProMED Mail.

COMMUNITY NEWS

June 2, 2004

Katherine High Named President of American Society of Gene Therapy

Katherine High, MD, a hematology researcher at The Children's Hospital of Philadelphia, was recently named president of the American Society of Gene Therapy (ASGT) at the Society's annual meeting in Minneapolis. High is well-known for her studies of the molecular biology of hemophilia. Over the past decade, she has investigated a gene transfer approach to treating hemophilia B by delivering the gene to produce clotting factor to a patient with a deficiency.

In addition to her position at Children's Hospital, High is a Howard

Hughes Medical Institute Investigator, one of 12 physician-scientists recently selected for major accomplishments in patient-oriented research. She also is the William H. Bennett Professor of Pediatrics at the University of Pennsylvania School of Medicine. She will serve as president of ASGT until June 2005.

ASGT is the largest medical professional organization representing researchers and scientists dedicated to discovering new gene therapies.

Source: The Children's Hospital of Philadelphia.

May 25, 2004

Hemophilia Federation of India's Executive Director Dies

Ashok Verma, the executive director of the Hemophilia Federation of India (HFI) died on May 25, 2004, from complications of liver cancer. In a release by HFI, it was said that Verma "has left a legacy of dedication and service that will always keep him alive in our midst." He is survived by his wife, Benedicte, and a daughter.

Source: Hemophilia Federation (India).

May 23, 2004

Newman Opens Sixth Hole in the Wall Gang Camp

Actor Paul Newman celebrated the opening of his sixth Hole in the Wall Gang camp for chronically ill children

on May 23, 2004. Painted Turtle Camp is a 173-acre facility in California that will be able to take in 1,000 kids each summer. Newman and several other actors provided the early stages of funding for the camp. Now it stands on its own with funding from individuals, corporations and foundations. The camp's first session will host children who have had liver transplants. Other sessions are for children with hemophilia, skin diseases, sickle-cell disease and kidney disease. For more information, visit www.thepaintedturtle.org.

Source: The Los Angeles Daily News.

May 21, 2004

Memorial Stone Unveiled in UK

A stone that will commemorate the more than 1,200 hemophilia patients in the United Kingdom who contacted HIV through tainted blood in the 1980s was unveiled in the UK on May 21, 2004. It bears the inscription, "This grove of 1,200 trees was planned to celebrate the lives of people with haemophilia who were infected with HIV." It is the only memorial of its kind in the UK.

Source: UK Newsquest Regional Press.

April 12, 2004

Groundbreaking Hemophilia Researcher Dies

Ilsey Ingram, a researcher known for making substantial advances in the clinical care and self-treatment programs for people with hemophilia, died on April 12, 2004. Ingram was the director of the Supraregional Haemophilia Reference Centre and the Supraregional Centre for the Diagnosis of Bleeding Disorders at the St. Thomas Hospital in London. In 1979, the Haemophilia Society gave him the MacFarlane Award. He later became vice president of the Haemophilia Society and earned the title of professor of experimental haematology at London University. After his retirement, Ingram lobbied for the compensation of those who had received AIDS-contaminated blood products. He is survived by his wife, Patricia, and a son and two daughters.

Source: Daily Telegraph.