



MEDICAL NEWS 1

BLOOD SAFETY
NEWS 2LEGISLATIVE
NEWS 4

NHF NEWS 7

MEDICAL NEWS

January 26, 2004**New Technology May Improve Protein Replacement Therapies**

New immune tolerance technology being developed by Bio Marin Pharmaceutical, Inc., may eventually make it possible to reduce or prevent the formation of factor VIII inhibitors in people with hemophilia. Treatment for those with factor VIII deficiency involves infusions of a recombinant protein. This new technology would improve the immune tolerance of patients with any chronic illnesses receiving protein-based biopharmaceuticals. Bio Marin has filed patent applications for its new technology, which was developed by research supported by a grant from the National Institutes of Health.

Source: Pharma Business Week

January 26, 2004**Intracranial Hemorrhage Study Encourages Prompt Treatment**

Researchers at the Universidade Federal de Sao Paulo in Brazil found that prompt factor treatment after every head trauma, even minor ones, could improve the prognosis of those with hemophilia. In the study, 401 cases of intracranial hemorrhages were documented. Each episode recorded the results of a CT scan, anatomic location, clinical presentation, relationship to trauma and clinical factors, including the presence of HIV or an inhibitor. The study found that those who received early replacement therapy fared the best.

More about the study can be found in: Haemophilia, 2003;9(5):573-577.

Source: Pain and Central Nervous System Week

January 26, 2004**Wyeth Withdraws FDA Application for Investigational Drug as it Prepares to Consolidate Factor Production in Sweden**

As a result of the closing of its St. Louis plant and its plan to consolidate factor production in Sweden, Wyeth announced it has withdrawn its marketing application to the Food and Drug Administration (FDA) for the investigational plasma, albumin and antibody-free recombinant factor VIII product,

according to a company release.

Once the transition to the Swedish facility is complete, Wyeth plans to update and resubmit its application to FDA, the release said. "Clinical trials for our investigational drug remain ongoing. Patients and clinicians involved in these clinical trials can be assured that sufficient clinical supply is available to complete the current studies."

February 31, 2004

Upcoming FDA Meeting to Address CJD and BSE Findings

The Food and Drug Administration (FDA) will hold the first quarterly meeting of the Transmissible Spongiform Encephalopathies (TSE) Advisory Committee on February 12 to 13, 2004, in Silver Spring, Maryland. The bleeding disorders community will be represented on the advisory committee by a consumer representative.

The committee will hear presentations on the presumptive transfusion-transmitted case of variant Creutzfeldt-Jakob Disease (vCJD) reported recently in the United Kingdom and receive updates on related experimental studies in animals on transmission of TSE agents by blood. Also, they will receive an update on the case of bovine spongiform encephalopa-

thy (BSE) recently recognized in the United States and will have a general discussion about potential models of risk-based approaches to sourcing of bovine materials used to make medical products.

The committee will have a preliminary discussion about FDA's current recommendations on measures to minimize risk from TSE agents in various types of medical products.

Established in 1997, the TSE Advisory Committee reviews and evaluates available scientific data concerning the safety of products which may be at risk for transmission of spongiform encephalopathies.

BLOOD SAFETY NEWS

January 23, 2004

UK Announces Plan to Compensate Hepatitis C Victims

British citizens who were infected with hepatitis C before the National Health Service (NHS) began testing blood for the virus in 1991 will be eligible for compensation by the United Kingdom government. Included are patients who have been cleared of the virus through treatment and those who caught the virus from someone infected by NHS blood products. Around 5,000 people in the UK with hemophilia were infected with hepatitis in the 1970s and 1980s before blood was screened.

Some who had lobbied for compensation were insulted at the amount of compensation, saying it "did not match the intense suffering of the victims." Patients are eligible for payments that range from £20,000 to £45,000 (\$34,000 to \$82,000 in US dollars). Currently, there is no similar compensation program for people affected by hepatitis C in the United States.

Sources: Press Association News and Birmingham Evening Mail

January 22, 2004

Japanese Transfusion Recipient Infected by HIV-Tainted Blood

Blood tainted with the HIV virus failed to be detected by the Japan Red Cross and was used in a transfusion. This is the first time that

anyone in Japan has been infected as a result of blood transfusion since 1999, when a new screening system was implemented. Only one patient received the tainted blood.

Source: "Japanese Transfusion Recipient Infected by HIV-tainted Blood." Blood Weekly.

January 20, 2004

A Powder to Halt Nose Bleeds Quickly

NosebleedQR, a topical powder that bonds with blood to halt a nosebleed immediately, has been introduced by Biolife. The Food and Drug Administration has been asked by Biolife to review evidence concerning the safety of this product for those with bleeding disorders.

Biolife claims that when a nosebleed begins and the powder is applied inside the nostrils, a kind of scab forms that serves as a "web" on top of the blood. Currently NosebleedQR is available over the counter at some drug stores for \$8 to \$10, and soon Wal-Mart and Albertson's stores will carry it as well.

A similar product, UrgentQR, is made of the same ingredients but is poured onto cuts. The National Hockey League, the National Football League and some university athletic departments have used UrgentQR to stop the bleeding from small wounds.

A small study conducted by Biolife in 2000 used the products on 45 patients in an emergency room environment. Forty-three patients' bleeding stopped within a minute, including seven patients who had used NosebleedQR. Thirteen of those patients' bleeding stopped within five seconds of the application.

Consumer Reports magazine called the products a "quick solution to an occasional messy problem."

Source: Wotapka, Dawn. "A Powder to Halt Nosebleeds Quickly." Newsday. January 20, 2004.

January 15, 2004

Survey Shows VWD Widely Undiagnosed

In August 2003, Harris Interactive conducted a survey for NHF that suggests that most women with von Willebrand disease (VWD) are not diagnosed or treated for it. Other research has suggested that up to 3% of women have VWD; not one of the 1,083 women in this nationwide survey had been diagnosed with this condition.

The Harris survey, which was conducted on behalf of NHF's *Project Red Flag: Real talk about women's bleeding disorders*, which is sponsored by Aventis Behring and the Centers for Disease Control and Prevention, found that many women report symptoms related to their periods, which is a possible indicator of a bleeding disorder. Ten percent of women in the sample reported that their periods last more than seven days, 33% described their menstrual flow as heavy and 48% have at one time bled through a tampon or napkin in an hour or less.

Drawing from these results, it's clear that there is a discrepancy between a woman's symptoms and a doctor's propensity to diagnose her. Just over half (54%) of the women surveyed reported that they or someone they knew

had sought treatment from a doctor for a heavy period, however, not one of these women was diagnosed with VWD. Most commonly, they were diagnosed as having fibroids (25%), endometriosis (21%), hormonal imbalance (17%), polyps (8%) and cancer (3%). In 17% of these women no diagnosis was made. The survey also found that only 9% of women had even heard of VWD, and if there is a family history of similar menstrual problems, a woman is likely to ignore the symptoms and resign herself to just "deal with it."

A similar problem exists within the medical profession. Many physicians are unfamiliar with VWD as well and think of heavy menstrual bleeding as a gynecologic rather than a hematologic problem. When many doctors think of bleeding disorders, they assume they only affect males. Should a doctor decide to perform tests for VWD, false negatives can occur, and menstrual problems are then attributed to hormonal changes.

Copies of the Harris survey can be obtained by contacting Anna DeSimone, education coordinator of Project Red Flag at (800) 424-2634, ext. 3705

LEGISLATIVE UPDATES

February 2, 2004**Fiscal Year 2005 Budget**

President Bush unveiled a \$2.4 trillion budget for fiscal year 2005 (FY 2005) on February 2, 2004. The budget includes \$571.6 billion on federal health programs and research funded through the Department of Health and Human Services (HHS). This is an increase of \$15.2 billion, or 2.7%, in budget authority over the fiscal year 2004 (FY 2004) budget request. The FY 2005 budget also proposes \$68.2 billion in discretionary spending for HHS, a decrease of \$1.1 billion, or 1.6%, from FY 2004.

Some of the other highlights of the President's FY 2005 budget are \$1.5 billion for the Food and Drug Administration (FDA) to be directed towards improving FDA's surveillance of the nation's food supply and expanding efforts to prevent bovine spongiform encephalopathy (BSE), commonly known as Mad Cow Disease.

Other requests for increased funding favor the National Institutes of Health, global HIV/AIDS assistance, mandatory spending on Medicaid and a surveillance program designed to protect the nation against bioterrorism and strengthen the public health infrastructure. Mandatory spending on Medicare would also increase to help fund the implementation of a prescription drug discount card. (The card is an interim measure to help seniors and the disabled pay drug costs. The card will be phased out when the permanent prescription drug benefit that Congress created last year goes into effect on January 1, 2006.)

The budget request for the Centers for Disease Control and Prevention was decreased from its amount in 2004, as was funding for the Health Resources and Services Administration.

The press release outlining Secretary of Health and Human Services Tommy Thompson's remarks can be found at <http://www.hhs.gov/news/press/2004pres/20040203a.html>

January 23, 2004**Omnibus Appropriations Bill Helps CDC and NIH**

President Bush signed the Fiscal Year 2004 Omnibus Appropriations Bill into law on January 23, 2004, ending a nearly six-month delay in approval of the final funding bill by Congress. Federal government agencies for which a 2004 appropriations bill had not been enacted had been operating under a series of continuing resolutions since the beginning of the fiscal year on October 1, 2003. The omnibus bill contained seven appropriations bills, providing funding for the Departments of Agriculture, Commerce, Education, Health and Human Services, Housing and Urban Development, Justice, Labor, State, Transportation, Treasury and Veterans Affairs.

For the Department of Health and Human Services, the bill provides increased funding for the Centers for Disease Control and Prevention (CDC) of \$262 million for a total budget of \$4.78 billion and for the National Institutes of Health of \$1 billion for a total budget of nearly \$28 billion. More than \$380 million of the funds provided to CDC are for the prevention of emerging infectious disease threats, such as SARS, West Nile Virus and Monkeypox.

The final funding bill includes two items of interest to the bleeding disorders community. Language was included requesting a report from the Health Resources and Services Administration (HRSA) regarding the status of payments from the Ricky Ray Hemophilia Relief Trust Fund. The fund terminated by law on November 12, 2003. The conferees also included language urging the National Heart, Lung and Blood Institute (NHLBI) to work with medical associations and experts to develop treatment guidelines for von Willebrand disease.

Additional information on the FY 2004 Omnibus Appropriations Bill can be obtained from the House Appropriations Committee website at: <http://appropriations.house.gov/>

January 7, 2004

DIMA Implementation Affects Clotting Factor Reimbursement: Part B Drug Changes

The Medicare Prescription Drug, Improvement and Modernization Act of 2003 (DIMA), signed into law on December 8, 2003, contains two provisions affecting Medicare reimbursement of clotting factor in the home and hospital outpatient settings. This legislation establishes a Medicare prescription drug benefit for seniors beginning in 2006 as well as other program and payment adjustments. The Centers for

Medicare and Medicaid Services (CMS) has moved quickly to carry out the provisions of the bill taking earliest effect, including issuing two interim final rules on drug payment to reflect the changes made effective by the law on January 1, 2004.

Reimbursement for Home Delivery: DIMA Provisions

Under Part B of the Medicare program, DIMA calls for reductions in 2004 from 95% of average wholesale price (AWP) to 85% of AWP for most currently covered drugs. Clotting factor drugs are exempt from this reduction, but appear to be subject to the competitive bidding process called for in the law beginning in 2005. Under competitive bidding, entities will compete in geographic regions to be selected as the top bidders. (Every region will have at least two selected bidders.)

Upon being awarded the bid, providers in that region can choose either to purchase drugs from one of the winning entities at the bid price or purchase drugs independently and receive reimbursement at average sales price (ASP) plus 6%. In addition to this payment amount, providers of clotting factor would receive an additional administration or dispensing fee to be determined by the secretary of Health and Human Services later this year.

Throughout much of the drafting of the DIMA legislation, it was believed that clotting factor would be exempt from the competitive bidding process. NHF is seeking clarification of this provision prior to its implementation.

January 7, 2004

CMS 2004 Interim Rule

On January 7, 2004, CMS issued the interim final rule on 2004 implementation of the payment reductions. A listing of Part B payment for clotting factor drugs is provided below. While clotting factor is exempt from the 10% reduction in 2004, NHF believes CMS made an error in the calculation of reimbursement for code J7195, Factor IX, Recombinant, and will comment on this to CMS by the March 8, 2004, deadline.

DIMA Implementation Affects Clotting Factor Reimbursement:

For hospital outpatient reimbursement under the final 2004 rule issued by CMS on November 7, 2003, NHF had successfully maintained 2004 hospital outpatient prospective payment system (HOPPS) rates for clotting factor products at the 2003 levels. In the rule, CMS stated that adoption of the payment freeze was due to the poor quality of the hospital cost data available for these products and the potential negative impact of further reductions

in reimbursement. This payment freeze avoided use of a "dampening" effect to limit decreases in drug payment that otherwise would have occurred if reimbursement were based solely on hospital cost data. The 2004 proposed rule had offered a 10% limit on reductions in payment for blood and blood products in the hospital outpatient setting.

One exception in the final 2004 rule was J7197, anti-thrombin, which suddenly lost its separate ambulatory payment classification (APC) status and was bundled for payment with other administration codes. Anti-thrombin is used for patients with clotting disorders.

2004 Part B Reimbursement

(All prices in international units, except as noted.)

Product Code	2004 Rates
J7190 Factor VIII, plasma	\$ 0.87
J7191 Factor VIII, porcine	\$ 2.04
J7192 Factor VIII, recombinant	\$ 1.26
J7193 Factor IX, non-recombinant	\$ 1.12
J7194 Factor IX, complex	\$ 0.37
J7195 Factor IX, recombinant	\$ 1.12
J7197 Antithrombin III	\$ 1.25
J7198 Anti-inhibitor	\$ 1.43
Q0187 Factor VIIa, recombinant, per 1.2 mg	\$1,681.50
Q2022 Von Willebrand factor	\$ 0.95

Additional information on the DIMA legislation can be accessed at <http://waysandmeans.house.gov/Special.asp?section=43>. The January 7, 2004, CMS rule can be accessed at <http://www.cms.gov/physicians/pfs/>.

DIMA Provisions

Passage and enactment of DIMA halted implementation of the November 7, 2003, final HOPPS rule to allow for implementation of the new DIMA HOPPS drug reimbursement provisions. Section 621(a)(1) of DIMA mandates the classification of separately paid hospital outpatient radiopharmaceuticals, drugs and biologicals into three payment categories, with payment based on specified percentages of reference average wholesale price (AWP) for each category. The three categories are sole source, innovator multiple source and non-innovator multiple source. Reimbursement for these three categories is stipulated to be no less than 88% and no more than 95% of AWP for sole

source drugs, 68% of AWP for innovator multiple source drugs and 46% of AWP for non-innovator multiple source drugs.

CMS 2004 Interim Rule

On January 6, 2004, CMS issued an interim final rule to implement the hospital outpatient reimbursement changes called for in DIMA. In putting forth this rule, NHF believes CMS has made errors in the interpretation of the payment categories that could result in less than optimal care for Medicare beneficiaries with hemophilia and other bleeding disorders. Several clotting factor products are identified as multiple source resulting in reimbursement for these drugs at below hospital acquisition cost.

A listing of hospital outpatient reimbursement under the interim final rule is provided below. These rates became effective on January 1, 2004, but, at this time, are subject to retroactive change pending further CMS review of comments received by March 8, 2004. CMS plans to publish a refined rule on April 1, 2004.

NHF is preparing its comments on the interim final rule at this time and will seek to make these comments available prior to the March 8, 2004, deadline to assist other interested parties in submitting their own comments. NHF has also requested an opportunity to testify before CMS' Ambulatory Payment Classification Advisory Committee later this month. This advisory committee makes recommendations to CMS on the HOPPS system.

2004 Hopps Reimbursement (All prices in international units, except as noted.)

Product Code	Per 1/6/04 Rule	Per 11/7/03 Rule
J7190 Factor VIII, plasma	\$ 0.51	\$ 0.42
J7191 Factor VIII, porcine	\$ 1.52	\$ 1.89
J7192 Factor VIII, recombinant	\$ 1.01	\$ 0.61
J7193 Factor IX, non-recombinant	\$ 0.51	\$ 0.51
J7194 Factor IX, complex	\$ 0.51	\$ 0.18
J7195 Factor IX, recombinant	\$ 1.01	\$ 1.04
J7197 Antithrombin III	Bundled	Bundled
J7198 Anti-inhibitor	\$ 1.01	\$ 0.69
Q0187 Factor VIIa, recombinant, per 1.2mg	\$ 1,083.93	\$1,495.30
Q2022 Von Willebrand factor	\$ 1.01	\$ 0.46

Additional information on the DIMA legislation can be accessed at <http://waysandmeans.house.gov/Special.asp?section=43>.

The January 6, 2004 CMS rule can be accessed at <http://www.cms.gov/regulations/hopps/2004ifc/>.

NHF NEWS

January 30, 2004

Tampa and Seattle to Be Sites of 2004's On the Road Conferences

NHF and the Centers for Disease Control and Prevention (CDC), along with volunteers from NHF's National Prevention Program (NPP) have announced the dates, locations and new formats for the On the Road events for 2004.

The first day of each of the two On the Road weekends this spring will feature a national meeting, each with its own special focus.

The first day of the April 's conference in Tampa, Florida, will be devoted to an NHF-chapter/association national caucus. The May event in Seattle will begin with a national conference on women's bleeding disorders sponsored by NHF and CDC.

The second day at each of the two venues will be devoted to prevention education. These sessions will be co-hosted by NHF, CDC and the local chapters/associations in collaboration with area HTCs. Individuals from within as well as from outside each local area will be invited to participate.

Mark your calendars and stay tuned for program information and invitations.

On the Road, Tampa, Florida

April 30 to May 1

Friday: NHF Chapter/Association National Caucus
Saturday: Prevention Education Day
Co-Hosts: Florida Chapter of NHF and Hemophilia Foundation of Greater Florida, Inc.

On the Road, Seattle, Washington

May 14 to 15
Friday: Women's Bleeding Disorders Conference/Workshop
Saturday: Prevention Education Day
Co-Host: Bleeding Disorders Foundation of Washington

Project Red Flag Discussion Group for Women on Project Red Flag Web Site

NHF is pleased to introduce a new feature on the Project Red Flag Web site—a discussion group for women. The discussion group has been designed especially for women, girls and parents /guardians of female children affected by bleeding disorders. This is the place to ask questions and connect and share with people who have common interests and concerns. This discussion group is part of NHF's new Web site, which will feature discussion groups on many topics.

Project Red Flag: Real Talk About Women's Bleeding Disorders is NHF's public awareness campaign. This program is made possible by support from the Centers for Disease Control and Prevention and Aventis Behring.

For more information or technical assistance, please contact Anna DeSimone, NHF Project Red Flag Coordinator at (212) 328-3700 ext. 3705 or Adesimone@hemophilia.org.