


**in this issue:**

MEDICAL NEWS 1

BLOOD SAFETY NEWS 3

NHF NEWS 4

## MEDICAL NEWS

**December 21, 2003**
**ABO Blood Type and Race Affects Measurement of von Willebrand Factor Activity**

According to research published in the *Journal of Thrombosis and Haemostasis*, three different principles can measure the activity of von Willebrand factor (VWF): ristocetin cofactor, collagen binding and activity ELISA. These diagnostic parameters were tested on 123 randomly selected females.

The researchers reported that race differences were seen in all tests except ristocetin cofactor, with Caucasians having lower levels of VWF than African Americans. Those with type O blood had significantly lower levels than non-O subjects. Blood type accounted for 19% of the total variance in VWF:Ag, and race accounted for 7%. These and other variances are partially to blame for the difficulty in defining diagnostic limits for VWD.

Source: *Blood Weekly*

**December 20, 2003**
**Despite St. Louis Plant Shutdown, No Interruption in ReFacto Production, Says Wyeth**

Wyeth announced plans to close its ReFacto facility in St. Louis in the hopes of cutting excess costs. According to a Wyeth release, "Current and projected demand for ReFacto, a recombinant hemophilia A treatment, can be satisfied with remaining manufacturing capability." ReFacto production will now wholly take place at a factory in Sweden.

Source: Wyeth press release and *St. Louis Post-Dispatch* (Missouri)

**December 18, 2003**
**Gene Transfer Therapy Evaluated for Market Introduction**

According to a MedMarket Diligence report, gene transfer therapy could be headed toward market approval and commercial introduction by the end of this decade. Due to advances in the utilization of viral and nonviral vectors to tar-

get single gene deficiencies, cancers, central nervous system conditions, tissue repair and other applications, there are a stunning number of companies and research institutions developing the science of gene transfer therapy.

The report, entitled "Gene Therapy: Worldwide Current Development Status and Market Potential," assesses the status of development in gene transfer therapy and the outlook for its commercial introduction. MedMarket Diligence is a publication that analyzes the market implications of new medical technology. Details on the report can be found at: [www.mediligence.com/rpt-a605.htm](http://www.mediligence.com/rpt-a605.htm)

Source: MedMarket Diligence

## December 16, 2003

### Two Organizations Collaborate to Improve Treatment for Patients with Inhibitors

Genmab A/S announced that it will collaborate with Dutch non-profit Sanquin Blood Supply Foundation to develop an improvement in the treatment for hemophilia patients with factor VIII (FVIII) who are not able to respond to standard therapy because of inhibitors. "Sanquin

has discovered a method of preventing these inhibitory antibodies from binding to factor VIII," said a Genmab press release. "This method involves using new antibodies to block the sites where the inhibitory antibodies bind and thus allow factor VIII to continue working." Researchers have covered locations on FVIII where inhibitors bind with antibody fragments to provide a blockade around the factor.

This research is only now being conducted on mice, and is many years away from beginning trials on humans. Genmab has agreed to provide support to Sanquin to validate this method and has the option of licensing the technology once developed. For more information visit: [www.genmab.com](http://www.genmab.com).

Source: *BioSpace News*

## December 2, 2003

### Business Is Booming for Genetic Counselors

Knowing the risks for certain genetic diseases can lead patients to eat healthier, exercise more and seek regular screenings for themselves and their family members. Genetic counseling is a rapidly growing field. Ten years ago, few diseases were known to be caused by specific genes, such as hemophilia, sickle-cell anemia and Huntington's disease. Today more than 1,000 genes have been linked to disease. For cancer alone, researchers have identified more than 50 disease-linked genes. The number of tests available to screen for genetic conditions will skyrocket in the future as researchers continue to decipher the 30,000 human genes in the genome. Women who are known hemophilia carriers and want to have children are sent to a genetic counselor if one is available at their hospital.

"But genetic tests don't offer black-and-white answers," says an article in the *Boston Globe*. "Rather, patients typically find out they have a partial chance of developing a disease—odds of 40%, 30%, sometimes lower." These odds, however, can help patients decide if they should take precautions, such as having a hysterectomy if they're shown to have a high chance of acquiring ovarian cancer.

Currently only the largest hospitals have genetic-counseling staffs, but because blood tests to be analyzed for genetic mutations are simple and relatively inexpensive, more hospitals and clinics will start to hire genetic counselors. Because the science is constantly expanding, doctors have found they have to regularly review medical journals or

attend seminars to keep informed.

For more information about genetic testing, visit:

[www.ornl.gov/sci/techresources/Human-Genome/medicine/genetest.shtml](http://www.ornl.gov/sci/techresources/Human-Genome/medicine/genetest.shtml)

Source: "Working the Odds: The Field of Genetic Counseling Is Booming As Doctors Learn More about The Hereditary Basis for Dreaded Disease, And Enlist Patients in the Battle Against Them." *Boston Globe*, December 2, 2003.

### November 24, 2003

#### British Journal Recommends Immunization for Patients with Bleeding Disorders

The British journal *Haemophilia* outlined investigators' recommendations appropriate for patients with bleeding disorders, saying that the recommendations are different than those for people without bleeding disorders. The difference lies in the risk of hematoma formation at the vaccination site and the "unusual infective risks associate with the potential, and past, exposure to blood products," said the report. "Most vaccinations can be given subcutaneously and this should be the preferred route," said one of the report's collaborators. "All routine childhood vaccinations should be given at the appropriate time. All patients with bleeding disorders should be vaccinated against hepatitis A and B. HIV- positive patients should receive annual influenza vaccinations and should avoid the oral polio, oral typhoid, BCG and yellow fever vaccines."

NHF's Medical and Scientific Advisory Council (MASAC) recommends children with bleeding disorders should receive a hepatitis B vaccination at birth or at the time of diagnosis, and primary immune response should be documented. MASAC also recommends that all individuals two years or older who are hepatitis A virus seronegative should receive a hepatitis A vaccine.

The study can be found in *Haemophilia*, 2003;9(5)541-46.

Source: "Recommended Immunization of Patients with Bleeding Disorders Outlined." *Health & Medicine Week*, November 24, 2003.

MASAC Document #151:

<http://www.hemophilia.org/programs/masac/masac151.htm>

### BLOOD SAFETY NEWS

#### December 19, 2003

#### Possible Transmission of Variant Creutzfeldt-Jakob Disease in Transfusion Recipient

NHF has become aware of a recent report in the United Kingdom of possible blood transmission of variant Creutzfeldt-Jakob disease (vCJD). The information below was compiled from the British minister of health's statement to Parliament.

Earlier this week, British Minister of Health John Reid reported to Parliament a case of possible transmission of variant Creutzfeldt-Jakob disease in a transfusion recipient, the first report that the disease might be transmitted to people through blood. Blood experts have speculated vCJD might be spread through blood transfusions and in recent years have put in place precautions, such as donor deferral, should transmission ever be proven.

The transfusion reported this week occurred in 1996, before vCJD safeguards were applied to the blood supply in Britain. Nearly all vCJD cases worldwide have developed in the United Kingdom, which suffered an epidemic of CJD among its bovine population in the early 1990s. As a result of

the epidemic and the development of vCJD in more than 140 persons who consumed contaminated beef, all blood products for use in Britain today are now imported from the United States, where there have been no reported cases of human CJD.

The minister of health indicated, while it is possible that this case of vCJD was transmitted from donor to recipient by blood transfusion, it is also possible that both individuals separately acquired vCJD by eating infected meat or meat products. The minister further indicated that conclusive evidence may not be possible. "This is a single incident, so it is impossible to be sure which was the route of infection. However, the possibility of this being transfusion-related cannot be discounted," stated the minister.

In referring to persons who are dependent upon blood and blood products, the minister stated, "Many more patients of course, including [people with hemophilia], will have received plasma products before plasma was sourced from the USA. They will have received products derived from large pools of plasma donated from many thousands of people and thus heavily diluted. The UK-wide CJD Incidents Panel considers the risks for this group to be even lower than for those who received whole blood."

The potential for blood transmission of vCJD points to the continued need for vigilance by the governments and blood and plasma collectors and manufacturers. In the United States, the possibility of transmission of vCJD continues to be monitored by the US Food and Drug Administration (FDA) and the Centers for Disease Control and Prevention. In 1997 FDA established the Transmissible Spongiform Encephalopathies Advisory Committee, which evaluates evidence related to CJD and vCJD transmission. The bleeding disorders community is represented on the advisory committee by a consumer representative.

Source: British Minister of Health Statement to Parliament

#### NHF NEWS

### January 8, 2003

#### **MASAC Recommendation #155**

NHF's Medical and Scientific Advisory Council has released its recommendation #155, entitled "Guidelines for Emergency Department Management of Individuals with Hemophilia." Individuals with bleeding disorders who go to emergency departments for care may not receive appropriate, expeditious management. This recommendation provides guidelines regarding triage, assessment, diagnostic studies, indications for replacement therapy and treatment.

The full text of the recommendation can be accessed online at: [hemophilia.org/programs/masac/masac155.htm](http://hemophilia.org/programs/masac/masac155.htm) and [info@hemophilia.org](mailto:info@hemophilia.org) and or calling 800-42-HANDI

### January 7, 2003

#### **Date Set for NHF's National Sing-Along Fundraiser**

NHF is pleased to announce that after many conversations with chapters and others, a date has been set for the tentatively titled national fundraiser, A Song for You: A national sing-along to support people with bleeding disorders. The event, which is part of National Hemophilia Awareness Month, will take place on Saturday, March 5, 2005. Approximately 20 chapters have signed on to date. For more information, contact HANDI at 1-800-424-2634.