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MEDICAL NEWS

April 26, 2004**Risk Factor Identified for Severe Liver Fibrosis**

According to European researchers, severe liver fibrosis is frequently found in hepatitis C (HCV)-HIV coinfecting patients with elevated serum alanine aminotransferase (ALT) levels. The rate of complications due to end-stage liver disease is high in these patients, and anti-HCV therapy should be considered a priority, said the study, which was published in *Clinical Infectious Diseases*. The study was performed in 10 European health-care centers with 914 coinfecting patients from 1992 to 2002.

Source: Health & Medicine Week.

April 21, 2004**DNA Chip Offers Simultaneous Detection of HIV, HBV and HCV**

Researchers at the Chinese Academy of Science have discovered a qualitative DNA chip method that can detect HIV and the hepatitis B and C viruses. According to the investigators, blood samples containing the three viruses were tested using this DNA chip, and 1 pg of specific viral DNA fragments were detected on the chip wells after nested PCR. The study is published in *Biosensors and Bioelectronics* (2004;19(7):685-692).

Source: Genomics and Genetics Weekly.

April 21, 2004**Italian Hemophilia Foundation Established**

A new hemophilia foundation, Fondazione Paracelso, was recently established in Rome. The event was inaugurated by a visit from the president of the Italian senate and the Italian health minister. The Fondazione Paracelso joins the Italian Federation of Hemophilia Associations (FEDEMO) in serving Italy's 6,000 hemophilia patients.

Source: ANSA English Corporate Service.

April 19, 2004**New Therapeutic Vaccine Approach for HIV Infection**

Lipid Sciences presented preclinical data for its Viral Immunotherapy platform at the Keystone Symposium, HIV Vaccine Development: Progress and Prospects. The data illustrated that simian immunodeficiency virus (SIV, a surrogate for HIV) delipidated by Lipid Sciences' proprietary process resulted in a broad humoral and cell-mediated immune response at an extremely low antigen dose. The data supports the premise that delipidating HIV has the potential to be used as the basis for a novel approach to the development of an autologous therapeutic vaccine for HIV-infected patients. The delipidated retrovirus, SIVmac251, elicited a broader and more robust cellular and antibody response to a broad array of SIV antigens with very low boost concentrations of delipidated virus without the aid of an adjuvant.

Source: *Company release.*

April 19, 2004**Undetectable HCV Viremia and Normal Liver Enzyme Levels May Not Mark Cure**

According to researchers at the University of Ottawa, their observations challenge conventional wisdom that HCV polymerase chain reaction-negative patients with normal liver enzymes are "cured." The study is published in *AIDS* (2004;18(2):337-338).

Source: *Health and Medicine Week.*

April 16, 2004**Novel Hepatitis C Therapy Approach Presented**

New data for two proprietary antiviral hepatitis C (HCV) therapies being developed by Vertex Pharmaceuticals was presented at the annual meeting for the European Association for the Study of the Liver in Berlin. The company presented information on merimepodib, an oral therapy being developed in combination with the current therapeutic standard, pegylated interferon alpha (peg-IFN) and ribavirin, with the goal of enhancing antiviral efficacy and increasing the proportion of patients who achieve a sustained response to treatment. Vertex is also developing VX-950, an investigational HCV protease inhibitor. According to a Vertex senior vice president, the results presented at the meeting provide strong support for initiating the late-stage clinical development of merimepodib and the first clinical studies of VX-950.

Source: *Company release.*

March 30, 2004**Genetic Surgery in Womb Explored by British Scientists**

A British team in London has announced it is preparing to use gene transplants in the womb to cure an unborn child of hemophilia B. The team is the first to find success using a "tamed" human immunodeficiency virus (HIV) to implant corrective genes to cure fetal mice. The group is in discussions with the Gene Therapy Advisory Committee, which supervises human trials. The scientists also plan to consult doctors and patient groups about the ethical dimensions of the surgery, as many argue this method to correct diseases could be used to create "designer babies."

Source: *London Daily Telegraph.*

LEGISLATIVE UPDATE

April 30, 2004**Cover the Uninsured Week**

The nation's largest coordinated effort to promote affordable health coverage for all Americans, Cover the Uninsured Week, is kicking off a weeklong series of national and local activities May 10 to 16, 2004. The effort is cochaired by

former Presidents Gerald Ford and Jimmy Carter and counts its influential members among 800 national and local organizations. The nonpartisan campaign's goal is to make it a national priority to find solutions for the nearly 44 million uninsured Americans. The campaign's national spokesperson is Noah Wyle, of the popular television show "ER." To find out what can be done to help, visit www.CoverTheUninsuredWeek.org.

Source: Organization brochure.

April 28, 2004

Genetic Discrimination Bill Stalled In the House

A Congressional bill to curb discriminatory practices in the genetic testing of employees and health insurance applicants appears to have stalled in the US House of Representatives. Although a similar bill passed the Senate last fall by a 95-0 vote after concessions to the US Chamber of Commerce and a coalition of other business lobbies on remedies and penalties, a similar measure in the House is not moving despite nearly eight years of negotiations.

Genetic testing can determine an individual's susceptibility to an array of diseases. Understanding the genetic basis of certain diseases may lead to new treatments and better preventive care. As it stands, employers can refuse to hire, or could fire, those prone to develop a disease. Furthermore, an insurer could refuse coverage or charge higher premiums on the basis of genetic tendency. Such discrimination often deters people from undergoing genetic testing. This can potentially undercut innovative healthcare for individuals who are genetically susceptible to a disease.

The Senate bill, sponsored chiefly by Small Business Chair Olympia Snowe (R-ME) and Health, Education, Labor and Pensions Chair Judd Gregg (R-NH), with bipartisan backing from Majority Leader Bill Frist (R-TN) and Minority Leader Tom Daschle (D-SD), would bar employers and insurers from testing for genetic markers that suggest a worker, job applicant or health insurance buyer might be subject to a debilitating ailment that could affect job performance or require costly medical care in the future.

The US Chamber of Commerce expressed its opposition to such legislation in the House. The business coalition argued in an issue paper sent to House members that the legislation needs to acknowledge that if some workers with specified genetic markers posed a "significant risk to others, then employers should be able to make employment decisions based on such information."

The US Chamber of Commerce insists that any federal antidiscrimination law should take legal precedence over the 30 state laws that now exist to bar such practices; that the law should have an explicit expiration, or sunset, date so Congress could review its effectiveness and decide whether to renew it; and that results of genetic tests should be narrowed to include only immediate family members related by blood and not broad-based family history criteria.

The Genetic Information Nondiscrimination Act (S. 1053) was referred to the House of Representatives on October 15, 2003, but has not yet been delegated to a committee, thus adding delay to floor passage.

April 22, 2004

HHS Approves Multistate Purchasing Pool for Medicaid Programs

On Thursday, April 22, 2004, Health and Human Services (HHS) Secretary Tommy Thompson approved plans by five states to pool their collective purchasing power to gain deeper discounts on prescription medicines for their state programs. The multistate purchasing pool plans approved include Michigan, Vermont, New Hampshire, Alaska and Nevada. This is the first time in the history of the Medicaid program that states have worked together in this manner.

As part of HHS efforts to help states identify ways to reduce costs while improving quality, the Center for Medicare and Medicaid Services will provide guidance to states explaining the agency's requirements on forming new purchasing pools and joining existing purchasing pools.

Although the states are pooling their efforts in buying drugs, they all will maintain their own preferred drug lists and exercise clinical oversight of those lists to ensure adequate access to needed medicines for their beneficiaries. States will use a committee of clinicians and pharmacists to review beneficiaries' medical needs before considering drug manufacturers' discounts.

The five states have signed agreements with First Health Services Corp., a pharmacy benefits manager, to negotiate lower prices on their behalf. Together, the states estimate they will save about \$12 billion this year in Medicaid prescription drug costs.

April 8, 2004

Successful Congressional Briefing Highlighting NCBDD

On April 8, 2004, the External Partners Group (EPG) of the Centers for Disease Control and Prevention's National Center on Birth Defects and Developmental Disabilities (NCBDD) hosted a luncheon briefing for Congressional staff on Capitol Hill.

The NCBDDD is the section of CDC that houses the Hereditary Blood Disorders Team. A total of \$2,400 was donated by 24 member organizations of the NCBDDD-EPG, including NHF, to help support the cost of the briefing.

For more information on this Congressional briefing, go to <http://www.cdc.gov/ncbddd/partners>.

BLOOD SAFETY NEWS

April 9, 2004

CDC Finds Low Risk of Transfusion-Associated WNV Transmission

The Centers for Disease Control and Prevention (CDC) had reported that blood screening for West Nile Virus (WNV) has improved safety, but that a small risk of transfusion-associated transmission remains. In June 2003, blood collection agencies began using an investigational test to screen all blood donations for WNV and identify potentially infectious donations. About 6 million units of blood were tested from June to December 2003, and at least 818 donations were removed from the blood supply because they contained viruses. Six cases of transfusion-associated WNV transmission were reported, indicating low levels of the virus in blood components might escape detection.

CDC said the blood collection community and public health authorities will consider this data when developing screening strategies for 2004. The agency said the benefits of receiving needed blood transfusions outweigh the potential risk for WNV infection, although patients needing blood transfusions should be aware of the limited risk.

For more, see the *Morbidity and Mortality Weekly Report* at <http://www.cdc.gov/mmwr>.

NHF NEWS

May 3, 2004**National Hemophilia Foundation Fourth Annual Gala**

Leaders from chapters, volunteers, donors, medical providers and beyond will gather on June 12, 2004, for a gala to honor those who have made significant contributions to the community, while raising crucial funds in support of NHF programs.

The gala will honor seven NHF chapters that have amassed fifty years or more of service to the community. They are the Hemophilia Foundation of Southern California, the Delaware Valley Chapter, Hemophilia Foundation of Illinois, Hemophilia Foundation of Maryland, Inc., Mary M. Gooley Hemophilia Center, Northern Ohio Hemophilia Foundation, Inc., and the Western Pennsylvania Chapter of NHF. Funds raised from the evening will support NHF's ongoing educational initiatives, advocacy efforts and grant programs.

The gala will be held in the elegant surroundings of the Starlight Ballroom at the Waldorf=Astoria in New York City. Those interested in attending should visit the gala Web page at www.hemophilia.org for further information on tickets. For any inquires on the gala, please contact Rebecca Schoon, development manager, at 212-328-3722.

May 3, 2004**HANY Requests Proposals for Research Grants**

The Hemophilia Association of New York, Inc. (HANY), has launched a multi-grant, multiyear research support initiative for exploring inhibitors. The grant program has been made possible by the family of a HANY board member who pledged to raise \$1 million. After just five months, \$500,000 has been raised toward that goal.

The request for proposals runs in May's issues of *Blood* and *The Journal of Thrombosis and Haemostasis*, as well as through Health and Human Services regional coordinators. The deadline for submission of letters of intent is July 1, 2004. Authors of the accepted proposals will receive one or more two-year grants of up to \$250,000.

For more information, contact HANY, call 212-682-5510 or e-mail hany@best-web.net.

May 3, 2004**NHF Teams Up with ZLB Behring for the Second Annual Junior National Championship**

NHF and ZLB Behring (formerly Aventis Behring) have again joined forces to encourage kids with bleeding disorders to get active and stay fit. Together, the two organizations are offering the second annual Junior National Championship in baseball and golf. This championship consists of four regional events and a final competition.

The regional events are a chance for kids to get physically active and participate in friendly competition. During the morning of each regional event, all the kids attend a workshop where they learn how to exercise safely, get to know other kids with bleeding disorders and meet the Gettin' in the Game athletes—golfer Perry Parker and baseball player Corey Parker. The kids will also participate in baseball and golf clinics where, based on a point system for baseball and on general style and technique for golf, they can be selected by Corey and Perry for the afternoon's regional event. Other afternoon activities include more sports clinics for the kids who don't participate in the regional event and a session for parents to share their feedback on the program. Perry and Corey will select two regional event winners from each location—one for baseball and one for golf. The winners will receive a

free trip for themselves and up to two accompanying adults to compete in the final competition in Tustin, California, on October 2, 2004. At the final competition, Perry and Corey will select two grand-prize winners. These winners will receive prizes, including a trip for three to NHF's 56th Annual Meeting in Dallas from November 4 to November 6, 2004. The winners will be ZLB Behring's special guests and honored at the company's exhibit booth.

The Championship is open to children with bleeding disorders between the ages of seven and 18. Those who live near one of the cities where an event is taking place should look for an invitation in the mail or can register by phone at 1-888-508-6978.

2004 tournament dates and locations

- ▶ June 5 Houston, Texas
- ▶ July 10 Washington, DC
- ▶ August 14 Portland, Oregon
- ▶ September 11 Chicago, Illinois
- ▶ October 2 Tustin, California*

* Final championship event

Last year's winners include:

2003 NHF-Aventis Behring
Junior National Championship
Winners

Regional Golf Winners:

Corey Pierce, 17, Eugene, Oregon
Justin (JT) McClanahan, 17, Pine Island, Minnesota
Alex Craig, 14, Richardson, Texas

Christopher Smith, 17, Odessa, Florida
Chris Cale, 8, Newport Beach, California

Regional Baseball Winners:

Marcus Mangana, 10, Eugene, Oregon
Andy Klamm, 16, Thompson, North Dakota
Matt Peters, 12, The Colony, Texas
Ivan Sada, 17, River View, Florida
Robert DeLeon, 15, Spring Hill, Florida
Zach Dansker, 17, Newport Beach, California

Final Competition Winners:

Junior National Golf Champion:
Vohn Dum Dumaya, 11, Fairfield, California
Junior National Baseball Champion:
Ivan Sada, 17, River View, Florida

May 1, 2004

NHF Goes "On the Road" to Tampa and Seattle

NHF's first "On the Road" meeting of 2004 took place on May 1 in Tampa, Florida, and was a huge success with over 180 attendees. People came from all over the state and were treated to many talented speakers and special events. Ben Shuldiner, who is one of the nation's youngest high school principals and has hemophilia, headlined a panel that included Lynn Obstein, a social worker from Rochester, and Chrissie Petersen from the Sibshops program that discussed family issues. The popular rap sessions were held during lunch, and the afternoon sessions began with two speakers, Todd Wills, MD, from the University of South Florida and Cindy Leisinger, MD, from Tulane University, who discussed blood-borne infections and hepatitis C and new treatments, respectively. An insurance panel sparked a lively discussion from the audience, and the day ended with physical therapist Angie Forsyth leading the group in some wonderful exercise techniques.

The Tampa "On the Road" was preceded by a one-day leadership conference, with the participation of representatives of NHF chapters and local associations, NHF Board members, regional coordinators of CDC regions and many others. A full report on the conference proceedings will be included in an "NHF Board Update" in the near future.

NHF's second "On the Road" program this year took place in Seattle on

Saturday, May 15, and was co-hosted by the Bleeding Disorders Foundation of Washington. The event featured sessions similar to those held in Tampa, including *Do the 5!*, prevention of blood borne infections, new hepatitis C treatments, local insurance reimbursement issues, and a family issues program featuring Ben Shuldiner.

The event preceded the Friday before by a national meeting focusing on women with bleeding disorders, which included peer education for trainers and much more.

For more information on the "Women with Bleeding Disorders" meeting, please or contact: Anna DeSimone, education coordinator of Project Red Flag at 212-328-3705.

April 30, 2004

Hollywood All-Stars to Raise Money for NHF

Even movie stars want to be baseball stars, and this year many of them are getting a chance to do just that and help out NHF as well. In St. Louis on May 15 and 16 and in Boston on June 5 and 6, the Hollywood All-Stars, a rotating team of several dozen stars of film, TV and music will come together to play baseball (well, softball actually) against pro sports teams. The stars will be facing the St. Louis Rams and the New England Patriot football teams.

The events are for the whole family, with proceeds being donated to NHF's local chapters. As an added enticement to sponsors of the event, there will also be golf outings, private parties with the celebrities and a sweepstakes contest for a weeklong stay in Hollywood. Additional games will be held in Austin, Texas, and New York during July and August.

For ticket information, visit www.metrotix.com, keyword: ALLSTARS.

If you or your company would like to be a sponsor of one or more of these events, please call Paul Kelly, NHF's special events coordinator, at (212) 328-3711.

April 29, 2004

Broadway Film to Benefit NHF

On Monday, June 7, 2004, NHF will benefit from the premiere of the award-winning film "Broadway: The Golden Age." This film is a collection of interviews, reminiscences and rare footage of some of the most beloved performers and famed performances from more than 100 of Broadway's greatest legends.

Made by filmmaker Rick McKay and released by Dada Films, "Broadway: The Golden Age" will be in theaters this July. Already the winner of awards at several prestigious film festivals, this film has won the hearts of fans and critics alike. According to Variety, "Rick McKay's exceptional new documentary is a veritable avalanche of the biggest names in the history of the American theatre...with the resulting film perhaps the screen's most authoritative encapsulation of Broadway history and an intimately resonant one."

The premiere will be followed by an exclusive VIP party at Sardi's, a restaurant that's importance to the 1940s and 1950s theatre community is recounted by many of the film's stars.

Among the luminaries expected are Chita Rivera, Arthur Laurents, Stephen Sondheim, Jerry Orbach, Barbara Cook, Kitty Carlisle Hart, Kaye Ballard, Celest Holm, Elizabeth Ashley, Lainie Kazan, Tommy Tune

and Marian Seldes.

For more information on the event, visit: www.broadwaythemovie.com.

April 8, 2004

Ryan White's 14-Year Legacy

April 8, 2004, marked the 14th anniversary of Ryan White's death at 18 years old. White became famous in the early 1980s when he contracted AIDS through a blood transfusion. His small-town Indiana middle school, knowing little about the disease, sequestered him in a classroom by himself to prevent its spread. Television cameras staked out the Whites' home and radio shows urged the school board to pull him from school.

Despite these protests, more than 1,500 mourners attended his funeral, and Indiana state flags flew at half-mast. Since 1991, the Ryan White Comprehensive AIDS Resources Emergency Act has provided more than \$6 billion in federal funds for AIDS services for those in need.

Source: Orlando Sentinel.