

THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

Fall 2009

**Interview with
Ann Rogers:
Executive Director,
Delaware Valley
Chapter of NHF**

**Industry Leaders
Gather in
Washington, D.C.
for the Plasma
Protein Forum**

**Grifols: Inspired
by the Past;
Innovating for
the Future**

**Leading in
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In the interest of encouraging broad and open discussion of issues relating to plasma protein therapies, collection and fractionation, THE SOURCE magazine may contain statements of opinion on such issues.

These statements are those of the author and do not necessarily reflect the opinion of PPTA or its members.

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These companies have earned QSEAL certification. The PPTA QSEAL program is a promise to stakeholders worldwide, that the highest levels of care were invested into the production of these unique life-saving therapies.
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IN MY VIEW

DECISIONS MUST BE EVIDENCE-BASED, NOT DRIVEN BY POLITICS

JUST BEFORE SUMMER OFFICIALLY STARTED

I traveled to China. Before entering the country, I had to wait in the airplane until health officers had cleared the plane. A device (which looked like a small gun) was pointed at my head to check my body temperature, a procedure that was repeated twice at the airport while walking through big temperature scanners. In addition, I had to fill out a form that had questions asking whether I had been in contact with someone with the flu in the last seven days, plus all kinds of questions about symptoms that could possibly identify me as a potential swine flu carrier.

I welcome any initiative that demonstrates that a government cares about its citizens and takes measures to prevent the outbreak of an epidemic that could take someone's life. Actually, we have seen many measures announced recently in many countries in regard to an outbreak of Swine flu.

Let's look at the following statistics. It is unknown how many people with hemophilia live in China. A conservative estimate is 60,000. In a normal age distribution it would be fair to say that one-third of the population is between 0 and 20 years old. In China there are not many persons with hemophilia over the age of 50. In addition, it is estimated that about half of the patients do not reach the age of adulthood. Let me use these

numbers, even though I know that a number of 60,000 persons with hemophilia in China is low. It would also be fair to assume that there are more than 20,000 persons with hemophilia between the ages 0 and 20.

Using these figures, 50 percent will die before reaching the age of 21, which means that 10,000 people will die in 20 years or in other words, 500 per year. Is this acceptable?

Persons with hemophilia do not have to die at an early age. There are therapies available, but because of 25-year-old rules, imports of plasma-derived clotting factors are prohibited in China. When are we going to see the day that decisions are evidence-based and not driven by politics?

I know that this sounds simple, but sometimes it is simple. Today, persons with hemophilia have a normal life expectancy and do not have to fear that they might have severe joint problems if they can start early in their life with the lifesaving factor therapies. It is a shame that in some countries, patients are denied access and as a consequence become disabled or even lose their life.

I know that keeping a flu virus out of your territory is a good thing, but making lifesaving therapies accessible for citizens is just as important. ●



Jan M. Bult
PPTA President



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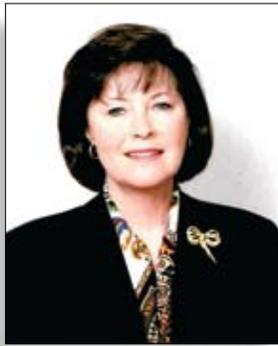
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ANN ROGERS

Executive Director, Delaware Valley Chapter (Pennsylvania) of the National Hemophilia Foundation

“THERE’S NEVER BEEN A BETTER TIME TO HAVE HEMOPHILIA IN THE U.S.,” says Ann Rogers.

With a personal and painful association with hemophilia, Rogers also is quick to stress that it is a very serious and complex blood disorder that is expensive to treat. As a mother who lost a son with hemophilia to HIV and who watches another manage a severe form of von Willebrand disease, she chooses to look at how far the community has come in the last two decades and to continually focus on the future.



Ann Rogers has been involved with the Delaware Valley Chapter of the National Hemophilia Foundation for 33 years.

What has been your history with hemophilia?

Since the beginning of factor concentrates in the early 1970s, we’ve seen an evolution in safety and efficacy of plasma protein therapies, which are life-sustaining, that is, we need them to control bleeding and we cannot live without them. We have watched the evolution of safe and effective therapies over 30 years of development and innovation from manufacturers around the world.

The HIV epidemic in the hemophilia community was a terrible time. We lost children, husbands and brothers due to contaminated factor concentrates. However, like many tragic events in history, there was a positive outcome from that tragic event—the development of safe, effective therapies. That positive outcome doesn’t balance our personal loss, but I do believe that

dark time in our history fueled the innovation more quickly. We will never forget what happened to us.

Today, we benefit as a national community of patients affected by bleeding disorders from that innovation. We now have therapies with smaller volumes that are safe and effective.

Today, children with bleeding disorders are healthy and many lead active, normal lifestyles and that is a major shift from previous generations. While the therapies are not a cure, they are like a cure, because for many patients with bleeding disorders they do a great job of controlling bleeding or preventing it altogether. Good medical management and the evolution of plasma protein therapies have changed the lives of people with hemophilia and von Willebrand disease in a dramatically positive way.



Independence Hall, shown in the background, is a U.S. national landmark located in Philadelphia, Pennsylvania.

The Delaware Valley is a term used to refer to the metropolitan area centered on the city of Philadelphia, Pennsylvania.

What do you see as the biggest concern of the hemophilia community today?

Today, our community still has big issues, but the issues are centered on how to maintain access to these life-sustaining therapies. The critical question is: How do we maintain access to care and medicine? My firm belief is that we achieve this through legislation at the state level by establishing hemophilia standards of care. We must legislate what we really need as patients with bleeding disorders. In Pennsylvania we are doing that with the introduction of HB 620 and SB 668, The Hemophilia Standards of Care Act. This proposed legislation, when passed by the General Assembly, will ensure that patients with hemophilia and von Willebrand Disease maintain:

- Access to all U.S. Food and Drug Administration (FDA)-approved plasma protein therapies for the treatment of hemophilia and related bleeding disorders.
- Access to the seven hemophilia programs in Pennsylvania.
- Access to the coagulation laboratories associated with the hemophilia programs in Pennsylvania. Outside laboratories contracted with insurance companies in Pennsylvania cannot perform specialized coagulation studies accurately or in a timely fashion. We need to have our laboratory studies done at the treatment centers.
- Access to options in pharmacy and home supportive services, including "full service pharmacies," 340B and mail order pharmacies. Patients' pharmacy needs can change over time. For example: An adult patient may be able to use a mail order pharmacy for his hemophilia needs, which reduces the cost for the insurance company. However, if that patient has shoulder surgery and can't self-infuse, he may need supportive care at home, including a homecare nurse, as ordered by his hemophilia

physician. We must make sure that insurers have different pharmacy options available as the needs of patients change. We have hundreds of examples of bleeding disorders patients in Pennsylvania who can't access the services they need at any given time.

In Pennsylvania, we don't believe that insurers are deliberately trying to hurt us. We believe they are trying to apply a disease management model that has worked well with other chronic conditions, such as asthma and diabetes, to patients with hemophilia. Disease management models have been very effective with some disease states, in reducing costs to insurers, while preserving the integrity of medical care. But it doesn't work well for patients with hemophilia. In fact, placing restrictions on the type of medicine (plasma protein therapy) a person can have, applying "fail first" requirements or preferred formularies, as well as restricting the amount of factor concentrates a patient can have on hand at home, has placed many people with hemophilia at risk.

How long have you been involved with the Delaware Valley Chapter?

My oldest son was born in 1976 with hemophilia and within a year we got involved in the Delaware Valley Chapter. My husband and I both have served on the board. In 1992 my son died of AIDS-related complications. I then took on the role as the Chapter Executive Director. Additionally, I have served in various leadership positions with the National Hemophilia Foundation (NHF), including being president of the Chapter Staff Organization of the NHF for six years. I have two other sons; the youngest has severe type three von Willebrand disease. ●

KYM KILBOURNE is PPTA's Assistant Director, North America Communications

"Today, our community still has big issues, but the issues are centered on how to maintain access to these life-sustaining therapies."

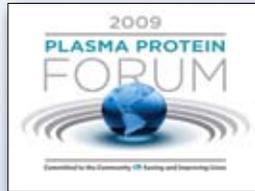


Far left:
A chapter family after successfully catching a fish.

Left: Children with bleeding disorders are healthy and many lead active, normal lifestyles. Seen here are children from the Delaware Valley Chapter community enjoying canoeing on the river.

BY KARA FLYNN

2009 Plasma Protein



PLASMA PROTEIN THERAPEUTICS INDUSTRY LEADERS GATHERED

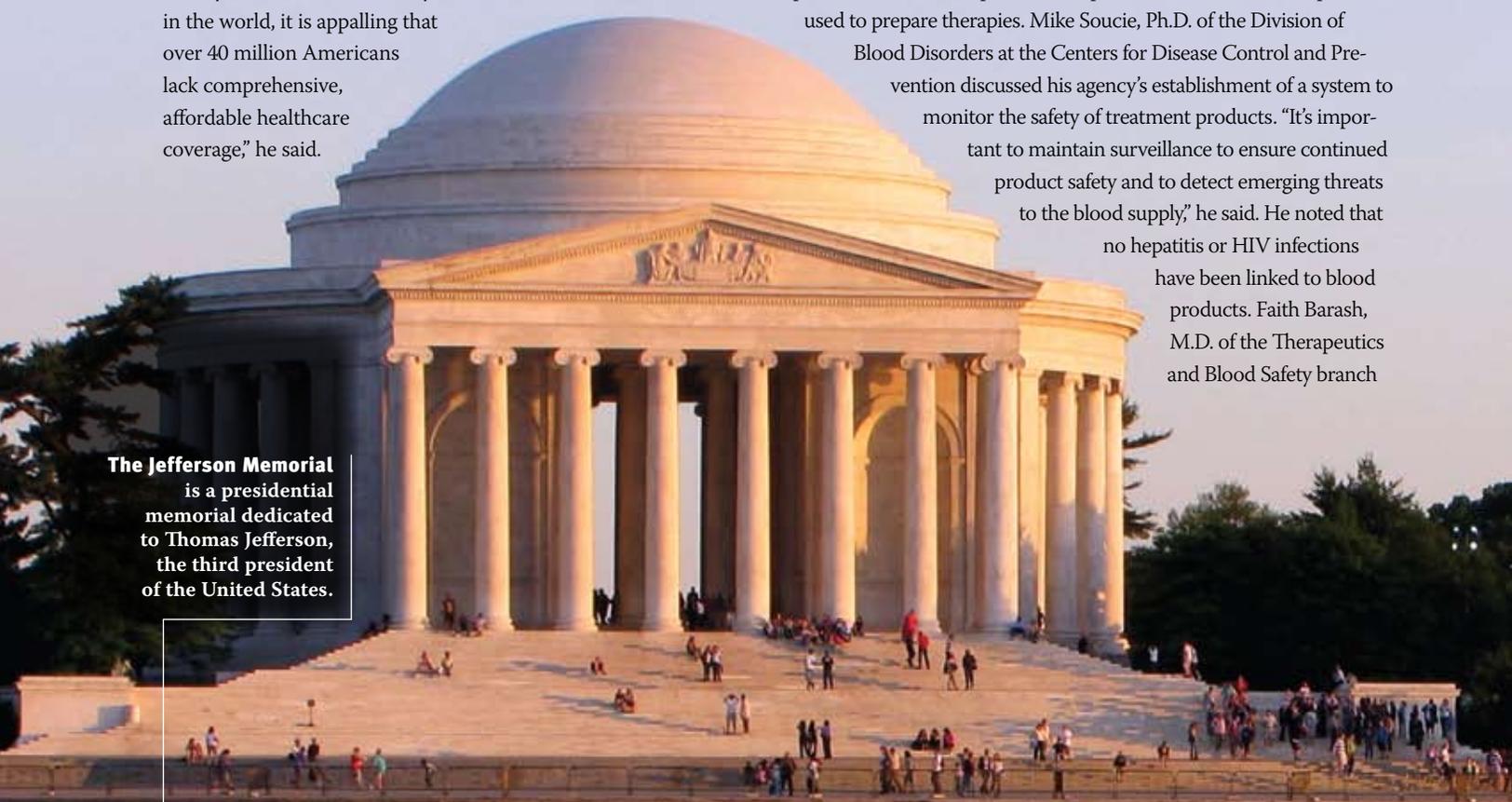
in Washington, D.C. for the annual Plasma Protein Forum held June 2-3. The event, which featured attendees from government agencies, patient advocacy groups, physicians, consumers and policymakers, drew more than 300 attendees who took part in the two-day meeting. Topics addressed at the conference included donor epidemiology; novel therapies in hemophilia; universal health care; strategies to secure the global drug supply chain and a discussion on significant contributions made by industry to help ensure the highest possible safety of finished therapies.

PPTA Global Board of Directors Chairman Larry Guiheen of Baxter BioScience opened the proceedings with a discussion on the state of the industry, focusing on industry priorities including ensuring access to critical therapies and a commitment to safety. In addition, Mr. Guiheen noted such challenges as the worldwide economic crisis and current focus on health care reform. "Communication of the essential nature of plasma biotherapies is critical," he said.

Presenting the key note address, U.S. Rep. Christopher Murphy (D-CT) discussed a topic on many participants' minds: health care reform in the 111th Congress. "In a nation that spends more money on healthcare than any other nation in the world, it is appalling that over 40 million Americans lack comprehensive, affordable healthcare coverage," he said.

Rep. Murphy, who serves on the House Energy and Commerce Committee's Health Subcommittee said it is imperative to cover all Americans, and lower the cost of health care. "In order to construct a cheaper system of universal care, I believe that the federal government needs to take a much stronger role in our health care system - to cover the presently uninsured." Rep. Murphy went on to say that addressing America's health care crisis is something we must face now. "Not only is it draining Americans of economic resources, but it is putting millions of families and children at medical risk."

Another Forum highlight came during a discussion of a multitude of safety initiatives that explored ongoing and planned activities to protect consumers of plasma therapies and the donors of the plasma used to prepare therapies. Mike Soucie, Ph.D. of the Division of Blood Disorders at the Centers for Disease Control and Prevention discussed his agency's establishment of a system to monitor the safety of treatment products. "It's important to maintain surveillance to ensure continued product safety and to detect emerging threats to the blood supply," he said. He noted that no hepatitis or HIV infections have been linked to blood products. Faith Barash, M.D. of the Therapeutics and Blood Safety branch



The Jefferson Memorial is a presidential memorial dedicated to Thomas Jefferson, the third president of the United States.

Forum BRINGS LEADERS TOGETHER IN WASHINGTON, D.C.

of the U.S. Food and Drug Administration also noted that surveillance is key in terms of “assuring the safety, effectiveness and availability of products that touch so many lives and are critical for public health.”

The first day concluded with a reception, where the Robert W. Reilly Award was presented to Bernard Horowitz, Ph.D. (see article on page 9). The award recognizes the lifetime achievements of an individual who has demonstrated unquestionable professional character, ethics and a commitment to the advancement of the plasma industry.

PPTA Source Board Chairman Gordon Naylor of CSL Behring, opened the second day of the Forum by welcoming participants and acknowledging that the industry continues to maintain strong growth. In addition, Mr. Naylor thanked plasma donors for giving the “gift of life” to people around the world with rare, chronic and genetic diseases.

A dialogue on “Novel Therapies in Hemophilia” with



PPTA Global Board of Directors Chairman Larry Guiheen of Baxter BioScience



David Knowlton, President and CEO of the New Jersey Health Care Quality Institute

President and CEO of the New Jersey Health Care Quality Institute, discussed the lessons learned in his state and how they are applicable to other states that are seeking to reform health care. Mr. Knowlton noted that New Jersey is home to more than 1.3 million uninsured, 240,000 of them children. “One out of every seven children in the state received no medical care as a result of being uninsured,” he said. In order to deal with this problematic situation, Knowlton said leaders in the state worked

Katherine High, M.D. addressed two approaches to hemophilia treatment that are currently in clinical trials. According to Dr. High, one is the use of adeno-associated virus (AAV) vector to deliver a gene for Factor IX into the liver for patients with hemophilia B. The other is a trial of an oral medication that may be able to raise circulating levels of clotting factor in patients who have hemophilia A or hemophilia B.

On the universal health care front, David Knowlton, President and CEO of the New Jersey Health Care Quality Institute, discussed the lessons learned in his state and how they are applicable to other states that are seeking to reform health care. Mr. Knowlton noted that New Jersey is home to more than 1.3 million uninsured, 240,000 of them children. “One out of every seven children in the state received no medical care as a result of being uninsured,” he said. In order to deal with this problematic situation, Knowlton said leaders in the state worked to enroll all who were eligible for state-sponsored coverage and manage available federal dollars for this purpose. In addition, he said the state made



Pictured left to right are PPTA President Jan M. Bult, U.S. Rep. Christopher Murphy (D-CT), Declan Murphy, Novens LLC, and Jay Greissing with PPTA.



sure that those who had health care coverage were able to keep it and provided an affordable and adequate coverage alternative for every man, woman and child in the state. He said New Jersey is working to create a model program that is state run with no employer mandate and includes a single self-insured plan. The target of this plan is not the poor, Knowlton said, but rather the middle class and he noted that discussions on this topic in the state are far from over. "We have no choice but to be successful," he said. "One billion dollars ago in terms of money lost to chronic disease in New Jersey was only nine days ago."

In a special presentation held in the afternoon, PPTA recognized consumers by honoring them for their participation in an educational video segment for public television. PPTA President Jan M. Bult honored the consumers for taking time out of their

busy schedules to work to educate the general public about continued access to plasma protein therapies (see sidebar below).

The afternoon featured a discussion on strategies to secure the global drug supply chain and a panel addressing issues related to industry safety measures (see story on page 10).

The 2009 Plasma Protein Forum's success was due in a large part to the terrific support provided by sponsors and exhibitors. Their contribution and participation helped make this year's Forum and Exhibit Hall a truly interactive event. PPTA extends its sincere thanks to the Plasma Protein Forum sponsors and exhibitors.

Save the date for next year's Plasma Protein Forum at the Hyatt Regency Reston in Reston, Virginia, June 15-16, 2009. ●

KARA FLYNN is PPTA's Director of Global Communications

PPTA Recognizes Consumers in Special Ceremony Held at Plasma Protein Forum

PARTICIPANTS IN A PPTA EDUCATIONAL television segment airing on public television channels across the U.S. were honored in a special consumer recognition ceremony during the Plasma Protein Forum in Washington, D.C. on June 3.

Honorees included British Davis, a high school student diagnosed with von Willebrand disease; Dakota McMullen, a 6th grade elementary school student who has hemophilia and John Boyle, an adult with primary immunodeficiency disease. In addition, PPTA recognized Ann Rogers, the executive director of the National Hemophilia Foundation's Delaware Valley Chapter (see page 4) who was instrumental in assisting the Association with the production of the television piece.

"By telling your inspirational stories, you have greatly assisted others in their understanding of these life threatening

disorders and diseases and the high-value and high-impact therapies used to treat them," said PPTA President Jan M. Bult. "We honor these outstanding individuals for the commitment they have shown to sharing with others the importance of plasma protein therapies in their day-to-day lives."

During the presentation, family members of the honorees were also recognized for helping their loved ones tell their personal stories on camera and educate millions of Americans about the rare and serious conditions treated with plasma protein therapies.

The educational public television segment was created by Boca Raton, FL-based Vision Media, a firm specializing in the production and distribution of educational segments on a number of issues ranging from the environment and current international affairs to health topics and medicine. The segment includes an introduction

from Hugh Downs, a well known U.S. television personality, who is the former host of the ABC News show 20/20 and NBC's Today Show.

PPTA worked with Vision Media on the five minute nationwide spot to provide important information to the viewing public with regard to the need for plasma protein therapeutics in treating rare, life-threatening, chronic and genetic conditions including bleeding disorders, immune system deficiencies, and alpha-1 antitrypsin deficiency. The segment will air throughout the remainder of 2009 hundreds of times in many of the top 200 designated market areas throughout the U.S.



Consumer recognition honorees and their family members at the Plasma Protein Forum with PPTA President Jan M. Bult.

BERNARD HOROWITZ HONORED WITH REILLY AWARD

BY KARA FLYNN

DR. BERNARD HOROWITZ, PH.D. was honored by PPTA with the prestigious Robert W. Reilly Award at the 2009 Plasma Protein Forum in Washington, D.C., recognizing his significant scientific contributions to the plasma protein therapeutics industry.

Dr. Horowitz was one of the inventors of the solvent detergent viral inactivation system, now an industry standard in the manufacture of plasma products, and he is one of the world's leading experts in viral inactivation and biological safety of human blood products. He holds more than 25 U.S. patents for these processes.

The Robert W. Reilly Leadership Award was initiated in 1998 to annually recognize an individual who best exemplifies the leadership qualities of Robert W. Reilly a leader of the plasma therapeutics industry association.

Dr. Horowitz studied biology at the University of Chicago and biochemistry at Cornell University Medical College and is a founding member of Horowitz Consultants, LLC, an organization that provides assistance in strategic planning, process development and the

evaluation of biological products. Most recently, he was a director of Omrix Therapeutics. Additionally, for more than 20 years, Dr. Horowitz headed numerous research and commercial development programs at the Kimball Research Institute and New York Blood Center in New York, and in 1995 he helped found V.I. Technologies, Inc., a for profit spin-out from the New York Blood Center.

He has served as a scientific consultant to the National Institutes of Health, the U.S. Food and Drug Administration, the National Hemophilia Foundation, the International Association of Biological Standardization, and the World Health Organization.

Dr. Horowitz has received several awards including the Morton Grove Rasmussen Prize from the American Association of Blood Banks and the 11th International Prix Henri Chaigneau from l'Association Francaise des Hemophiles. ●

KARA FLYNN is PPTA's Director of Global Communications

PREVIOUS HONOREES

2008

Peter Turner
CSL Behring

2007

Jean-Marie Vlassebrouck
BaxterBioScience

2006

David J. Gury
*formerly of
Nabi Biopharmaceuticals*

2005

Joseph Rosen
*Baxter BioScience/BioLife
Plasma Services*

2004

Dr. George Schreiber
Westat Inc.

2003

S. Tyrone Foster
*formerly of
Aventis Bio-Services*

2002

Richard Thomas
*formerly of
Bayer Corporation*

2001

Victor Grifols Lucas
*Chairman of the Board,
Grifols*

2000

Sammuel Penninger, Jr.
Serlogicals Corporation

1999

Jack Ryan
*formerly of
Bayer Corporation*

1998

John Bacich
*formerly of Baxter
Healthcare Corporation*



Bernard Horowitz, founding member of Horowitz Consultants, LLC accepted the Robert W. Reilly Award at this year's Plasma Protein Forum. Pictured left to right are PPTA President Jan M. Bult, Bernard Horowitz, and Larry Guiheen, chairman of the PPTA Global Board of Directors and President of Bio Pharmaceuticals for Baxter BioScience.

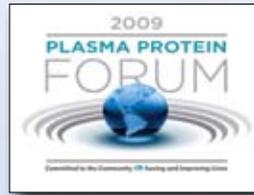
BY KARA FLYNN

“Decades of Safety

THE NATION’S CAPITAL SERVED AS A BACKDROP for a discussion on the considerable strides that have been made over the years towards providing the safest possible plasma protein therapies to patients who infuse or inject these lifesaving medicines. The panel discussion was moderated by PPTA's President Jan M. Bult during the Plasma Protein Forum and brought together industry, consumer organizations, physicians and regulators who have all made significant contributions to help ensure the safety of the finished therapy.

By organizing this panel, PPTA sought to explore the innovations that have been put in place over the years which continue to demonstrate industry's commitment to product quality and safety. Today's plasma protein therapies represent a compilation of state-of-the-art collection, viral removal and inactivation, and manufacturing protocols that have been fine tuned over the past two decades. Vigilance is the key common thread among the areas that form this multi-layered approach to safety.

Panel speakers during this two hour session included Craig Kessler, Chairman of the National Institutes of Health's Medical and Scientific Advisory Council (MASAC) and Prof. of Medicine and Pathology at Georgetown University Hospital in Washington, D.C.; Thomas R. Kreil, Ph.D., senior director of viral vaccines and global pathogen safety at Baxter BioScience; Bernard Horowitz,



Ph.D. of Horowitz Consulting, LLC.; Leonard A. Valentino, M.D., medical director at RUSH University and RUSH Children's Hospital in Chicago, Ill.; Jay Epstein, M.D., director of the Office of Blood Research & Review at the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA); Marcia Boyle, chair and founder of the Immune Deficiency Foundation; and Prof. Albert Farrugia, senior director of Global Access at PPTA.

Dr. Kessler opened the panel, discussing the formation of MASAC, which was initiated by the National Hemophilia Foundation to advance clinical care and promote hemophilia research in 1954. MASAC's recommendations serve as the basis for the standard of care in the U.S. related to hemophilia. Over the years, according to Dr. Kessler, MASAC has made a number of critical recom-



FDA's Dr. Jay Epstein gives a presentation on regulatory policy and evolution over the course of time.



Measures” HIGHLIGHTED DURING FORUM PANEL

mentations to the hemophilia community on issues of emergency care, infectious diseases and standards and criteria of hemophilia treatment. Council members are regarded as leading experts in the treatment and research related to care of bleeding disorders and the Council represents a unique partnership between government and the consumer community that has led to enhanced communications.

In a presentation titled “Regulatory Changes in the Last 20 Years: From Crisis to Confidence,” Dr. Epstein reviewed the crises of emerging infectious diseases from the HIV crisis in the 1980s, to some of the ongoing challenges that have occurred recently, including transfusion transmitted West Nile Virus, severe acute respiratory syndrome (SARS), and variant Creutzfeldt-Jakob Disease (vCJD). In addition, he discussed government’s response, which has included enhanced oversight and public input, and the collaborative CBER blood safety team and FDA/Department of Health and Human Services blood action plan. According to Dr. Epstein, improved outreach to stakeholders has also assisted greatly with regard to improving crisis management from regular liaison meetings with industry and consumer advocacy groups to more public scientific workshops for expert input. “Over the last two decades, FDA has become more proactive, more inclusive, more communicative and more vigilant,” he said. “These changes have improved the safety and availability of plasma products.”

According to Ms. Boyle, patients with primary immunodeficiency disease (PIDD) are concerned about the safety of plasma-derived products, however, the risk of infection is perceived to be low among patients and providers in surveys conducted by the Immune Deficiency Foundation. “The most clear and present danger to the health of patients with PIDD is availability of intravenous immunoglobulin (IVIG) products and obtaining treatment as a result of cost and coverage,” she said.

A highlight of the panel occurred when Dr. Horowitz, the recipient of PPTA’s Robert W. Reilly award (see article on page 9) talked about the calculations of residual viral risk and safety margins associated with plasma-derived products. Dr. Horowitz is one of the inventors of solvent detergent technology, which has had a significant impact over the years in terms of pathogen reduction.

“Marshalling of forces over a three decade period have resulted in plasma-derived products that have an outstanding pathogen safety record,” he said.

Dr. Kreil, chairman of PPTA’s Pathogen Safety Steering Committee (see article on the PSSC on page 16), pointed to some of the current safety procedures used in the manufacture of plasma-derived therapies that includes the so-called “safety tripod,” which comprises donor selection, testing of donations and viral reduction and removal process to ensure the manufacture of safe therapies.

Some of the conclusions reached during the panel discussion by participants included the following:

- Today’s plasma-derived therapies are amongst the safest biologics on the market.
- Communication among stakeholders including representatives of government, industry and consumer organizations is vital.
- Combined resources coupled with increased surveillance have led to outstanding pathogen safety records for plasma-derived products. ●

KARA FLYNN
is PPTA’s
Director of Global
Communications

The United States Capitol

PROF. CHRISTOPHER LUDLAM

President of EAHAD

CHRISTOPHER LUDLAM IS PROFESSOR OF HAEMATOLOGY AND COAGULATION MEDICINE

at the University of Edinburgh and Director of the Haemophilia and Thrombosis Centre at the Royal Infirmary of Edinburgh. Over the past 25 years, his major research activities have been dedicated to promoting the development and assessment of safe therapeutic products for treating hemophilia. Professor Ludlam has chaired various physician societies and advisory committees related to hemophilia treatment. Currently he is President of the European Association for Haemophilia and Allied Disorders (EAHAD).



Please tell us when and why the EAHAD was launched.

In 2005, a group of 45 hemophilia physicians from 15 European countries were brought together to consider some of the future difficulties that might arise in the provision of optimal hemophilia care. It was agreed that the three most important issues were the current lack of trainee physicians in haemostasis and thrombosis, the side effects of treatment with clotting factor concentrates, and the likely future pressure on funding for hemophilia care. The physicians

met on six occasions as the Interdisciplinary Working Group (IDWG) and were supported by an educational grant from Baxter Bioscience. The group produced the European Principles of Haemophilia Care (*Haemophilia* (2008) 14, 361-374) and the European Curriculum on Haemostasis and Thrombosis (*Haemophilia* 2009 Jan; 15 (1):337-44) to try to address and help develop hemophilia services and promote recruitment of doctors. The IDWG also developed a proposal for a European-wide surveillance system for adverse events. This proposal received EU and pharmaceutical funding, which has led to the establishment of the European Haemophilia Safety Surveillance Scheme (EUHASS, www.euhass.org) and that started collecting data on October 1, 2008.

Once the physicians had completed the arrangements for the above projects and because the meetings had been useful and productive, they wished to continue to meet. This led to a general invitation to a meeting in Vienna in June 2007 to consider establishing an Association. The meeting was very well attended and there was much enthusiasm to form a European group to

continue and promote further collaboration across the continent. Therefore, an agreement was put together to form the European Association for Haemophilia and Allied Disorders.

Who is the membership of EAHAD?

The Association was keen to have an inclusive membership to represent all who help provide services for hemophilia. Thus membership applications are encouraged from all such individuals including physicians, nurses, physiotherapists, clinical scientists and others. Ordinary members are those who work in the hospital services and pay a modest subscription rate (which includes a subscription to the journal *Haemophilia*) and a vote at the annual general meeting. We welcome members from industry (in the above groups of professionals) as Associate Members; they are excused from paying the subscription rate and do not have a vote at the meeting. Applications for membership are encouraged and can be made via the web site (www.eahad.org).

What are EAHAD's main objectives?

There are three principal aims of the Association. These are to promote high quality clinical care for individuals with hemophilia and similar disorders, clinical research (particularly collaborative multi-center clinical studies), and to provide education for all interested in hemophilia and associated inheritable bleeding disorders.

What do you believe are EAHAD's main achievements so far?

Since June 2007, when the association was founded, there have been two very successful annual scientific meetings in Amsterdam and Munich, with the latter attracting 400 delegates. These meetings have attracted clinicians, nurses and clinical scientists from across the world – at the last

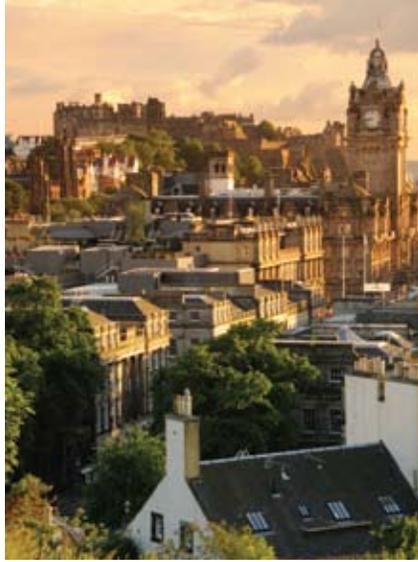
European Association for Haemophilia and Allied Disorders

EAHAD

EAHAD promotes high-quality clinical care, clinical research and provides education for all interested in hemophilia and associated inherited bleeding disorders.

On January 27, 2009, clinicians, nurses, patients and Members of the European Parliament gathered for the Launch of the European Principles of Haemophilia care in the European Parliament.

meeting 37 countries were represented. The programs have included recent advances in clinical and laboratory aspects of hemophilia, but in the future it is likely that there will be a broader range of conditions considered, particularly in relation to the less common heritable bleeding conditions. We have collaborated very closely with the roll out of the European Haemophilia Safety Surveillance (EUHASS) program across Europe. We have set up Working Parties to bring investigators together to undertake collaborative research. The Association has adopted the European Principles of Haemophilia Care and the European Curriculum on Haemostasis and Thrombosis and is actively promoting these. We have worked in partnership with the patients' group, the European Haemophilia Consortium (EHC), to promote the Principles of Care particularly in Eastern Europe.



Edinburgh, Scotland, the site of the 2010 EAHAD meeting

Please tell us about the recent launch of the European Principles of Haemophilia Care.

Along with the European Haemophilia Consortium (EHC), we had a most useful day at the European Parliament explaining to members of the European Parliament (MEPs) about hemophilia and how modern treatment can radically improve the lives of those with congenital bleeding disorders. The meeting was based around the Principles of Care and its 10 recommendations for national hemophilia services. We explained why it is important to have coordinated national arrangements. The meeting was well attended and supported by many MEPs who were ready to add their signatures to the Principles of Care document to indicate their support for its recommendations. It is anticipated that MEPs will take the document back to their countries' departments of health to help disseminate the recommendations and their implementation.

Please tell us what you believe are other recent important European developments on hemophilia care.

Europe has a very good reputation for being a leader of hemophilia developments probably because hemophilia centers work within national frameworks and have coordinated arrangements for patients and their coagulation laboratories. Much of the very good work has arisen out of across the continent collaboration between enthusiasts. Projects include the Pednet partnership of pediatric centers that has investigated inhibitor development, the type 1 von Willebrand disease International Prophylaxis Study, the European Study of Clinical, Health Economic and Quality of Life Outcomes in Haemophilia Treatment (ESCHQoL), which has assessed quality of life in relation to clotting factor usage, the Acquired Haemophilia Registry and the Malmo Brother Pairs Study.

How do you see the future for EAHAD?

EAHAD's most conspicuous activity will be its annual scientific meeting in February of each year. The meeting will aim to be the principal focus for hemophilia activities within Europe. Although it would be possible to fill many days of the meeting with presentations, we are determined to keep it strictly to two days so that it is less intrusive of individuals' work commitments. The gathering will be an opportunity for both experts and those entering the field to learn of recent developments and to discuss and promote collaborations for future clinical

studies. We are keen to encourage attendance by trainees and future meetings will have more activities to appeal to those entering the field. Reports from European collaborative projects, such as EUHASS and the Rare Bleeding Disorders Register, will be prominent in the program. That this annual meeting has attracted such a large attendance, within a couple of years of the establishment of the Association, demonstrates that it is highly valued by hemophilia professionals.

To help promote collaboration, Working Parties are being established to address topics that are best addressed by bringing together interested clinicians and clinical scientists. By attracting interest from hemophilia centers it will be possible to mount studies on large numbers of patients which would otherwise be impossible because hemophilia is a relatively rare condition. Europe has a good track record in such collaborations already as described earlier. Modest funding will be made available to the Working Parties to help with initiation of the projects. One of our first Working Parties will be assessing factor VIII structure – function relationships and clinical status in patients in whom there is a marked discrepancy between 1 and 2 stage factor assay results. This project may have far reaching implications for measurement of factor VIII levels in patients treated with new modified, longer half-life, factor VIII molecules.

Promotion of hemophilia services at a governmental level can often be most effectively achieved if all stakeholders are represented in presentations to elicit additional support and development of services. To this end we shall continue to work closely with the EHC to improve services, especially in Eastern Europe.

When and where will the next EAHAD meeting be held?

EAHAD's next Annual Scientific Meeting will be held at the Edinburgh International Conference Centre on February 4-5, 2010. The meeting is open to all with an interest in hemophilia and related bleeding disorders. Program, registration and accommodation details are available at www.eahad.org. We look forward to seeing you in Edinburgh and welcoming you to Scotland's capital! ●

JOHAN PRÉVOT is PPTA's Director, Health Policy Europe



INTERNATIONAL PLASMA PROTEIN CONGRESS

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SAVE THE DATE

LEADING IN RESEARCH: LEADING IN SAVING LIVES

PPTA's Pathogen Safety Steering Committee (PSSC)

BY ILKA VON HOEGEN, PH.D.

THE PATHOGEN SAFETY STEERING COMMITTEE (PSSC) is one of the earliest and most experienced committees with a predominant presence within the PPTA organization. Many of its committee members have contributed to the committee's success since the early days of the Association's existence. The highly experienced, well-seasoned PSSC members take special pride in the proactive approach they take at increasing the margin of safety of the therapies they manufacture, most importantly the development and introduction of voluntary standards for Nucleic Acid Testing (NAT) testing for HIV, hepatitis B (HBV) and hepatitis C (HCV), which preceded the implementation of the first mandatory NAT testing for HCV in the European Union in 1999. Furthermore, PPTA member companies introduced a 60-day inventory hold for plasma donations to minimize the risk of window period donations entering a plasma manufacturing pool. It also allows discarding the plasma prior to manufacturing if post-donation information becomes available that would disqualify the donation.

The overall goal of the PSSC is to provide data for patients, healthcare providers, and regulatory authorities to demonstrate that plasma-derived medicinal products are manufactured with the greatest safety margin.

One of the most significant programs that the PSSC led was the development and implementation of a voluntary standard for Parvovirus B19 in 2000. This effort is an example of where the plasma industry, through the coordination of the PSSC, took the lead in advance of international regulatory authorities. When the standard was developed, the PSSC concluded, based on scientific data that a cut-off level for Parvovirus B19 DNA of 10^5 IU/ml in the plasma production pool was the right cut-off to ensure safe therapies without unnecessary loss of plasma. The standard was established because PPTA member companies demonstrated that robust virus removal steps in their manufacturing processes had the capability to remove a virus load to a much greater magnitude than the 10^5 IU/ml level with minimal plasma waste. Since its introduction, PPTA member companies have generated an abundance of

data demonstrating the value of this voluntary standard. In the past years, regulatory authorities followed the PPTA initiative and requested an even stricter cut-off limit of 10^4 IU/ml.¹

PSSC Works Collaboratively With Regulatory Authorities

When in 2004 West Nile Virus (WNV) started to spread across the U.S. raising concern about the safety of plasma protein therapies, PPTA responded immediately to this challenge by compiling existing data and also proactively generating new data demonstrating that plasma protein therapies are safe with respect to WNV. The newly generated data for WNV inactivation confirmed the predictions of the model virus concept. In addition, the PSSC worked with the U.S. Food and Drug Administration (FDA) to ensure clear communication on this issue. As a consequence, U.S. regulatory authorities implemented specific safety measures for labile blood products but refrained from introducing comparable measures for plasma protein therapies in acknowledgement of the scientific evidence PSSC provided.

When it first appeared, variant Creutzfeldt Jakob Disease





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(vCJD) had not been transmitted through transfusions. Nevertheless, PSSC members collaboratively generated data on the clearance of prion proteins by the manufacturing process and facility cleaning procedures either through individual companies or as a collaborative PPTA effort². When it appeared that vCJD may be transmitted by blood transfusion, concerns were raised

¹*Guidance for Industry Nucleic Acid Testing (NAT) to Reduce the Possible Risk of Human Parvovirus B19 Transmission by Plasma-Derived Products - <http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm>*

²*Zerlauth, G NAT testing for source plasma. Presented to the 18. Meeting of the Advisory Committee for Blood Safety and Availability, (ACBSA), "Prioritizing Decisions in Transfusion Medicine: Transfusion Transmissible Diseases"; US Department of Health and Human Services, Washington DC, USA, January 23-24, 2003
Transcript: <http://www.hhs.gov/ohs/bloodsafety/pastmeetings/transcripts/20030123.html>*

³*Dichtelmüller et al. 2009 Robustness of Solvent / Detergent Treatment of Plasma Derivatives: A Data Collection from PPTA Member Companies. Transfusion: in press*

⁴*Collaborative Study of PPTA Member Companies on Virus Removal by the Steps of the Cold Ethanol Fractionation Process, manuscript in preparation*

that it could be also transmitted by plasma protein therapies. With the recent discovery of a hemophilia patient in the United Kingdom whose autopsy demonstrated presence of abnormal prion proteins in the spleen, the concern for transmission of vCJD was heightened, particularly for the hemophilia community. To address this concern, PSSC presented data at a FDA Transmissible Spongiform Encephalopathic (TSE) advisory committee in June 2009 that reiterated the efforts PPTA member companies are taking to reduce the risk of the vCJD threat to our patients. These efforts included additional controls on the plasma donor population, increased diligence on the sourcing of the production of raw materials to reduce the opportunity for the introduction of prions in the production systems, and finally the PSSC provided their most up-to-date scientific evidence demonstrating effective removal of prions in the production processes.

Over the past 15 years, PSSC and the industry have provided leadership to the regulatory landscape by introducing voluntary standards as a collaborative initiative, as well as proactively working with regulatory authorities and patient organizations to ensure the safety of the products. The revised European Medicines Agency (EMA) Guideline on Plasma-derived medicinal products 269/95 which is currently under discussion reflects the joint effort of regulatory authorities and industry to develop robust regulatory guidance.

In conclusion, a number of pathogen associated challenges have been encountered, such as WNV, Severe Acute Respiratory Syndrome (SARS) or vCJD. Thanks to the excellent work

COMMITTEE



Christoph Kempf



Juan I. Jorguera



Douglas Lee

of the PSSC in collaboration with regulatory agencies and other stakeholders, none of these challenges have substantiated themselves to put patients at risk.

The Role of PSSC

PSSC's plays several roles within the PPTA including 1) providing the experts that generate scientific data for regulatory policy making, 2) representing PPTA within the scientific community, 3) participating in communications to stakeholders regarding the safety of plasma protein therapies and 4) responding to immediate challenges imposed by existing or newly emerging pathogens. Particularly with respect to the latter, the focus of the PSSC over the years has changed from reactive initiatives driven by immediate public concerns towards that of a more strategic organization focused on the overall public health of our patient and donor communities. This change in direction is illustrated in efforts of the committee by providing data either in the form of peer reviewed publications^{3,4}, or by presentations at scientific congresses.

Today, PSSC's mandate is to provide state-of-the-art scientific evidence to assure the patient and donor community and provide a basis for regulatory agencies to develop sound and reasonable policies. Even without immediate threats through known and emerging pathogens, PPTA's member companies remain committed to research and development. ●

ILKA VON HOEGEN, PH.D., is PPTA Europe's Senior Director of Quality and Safety



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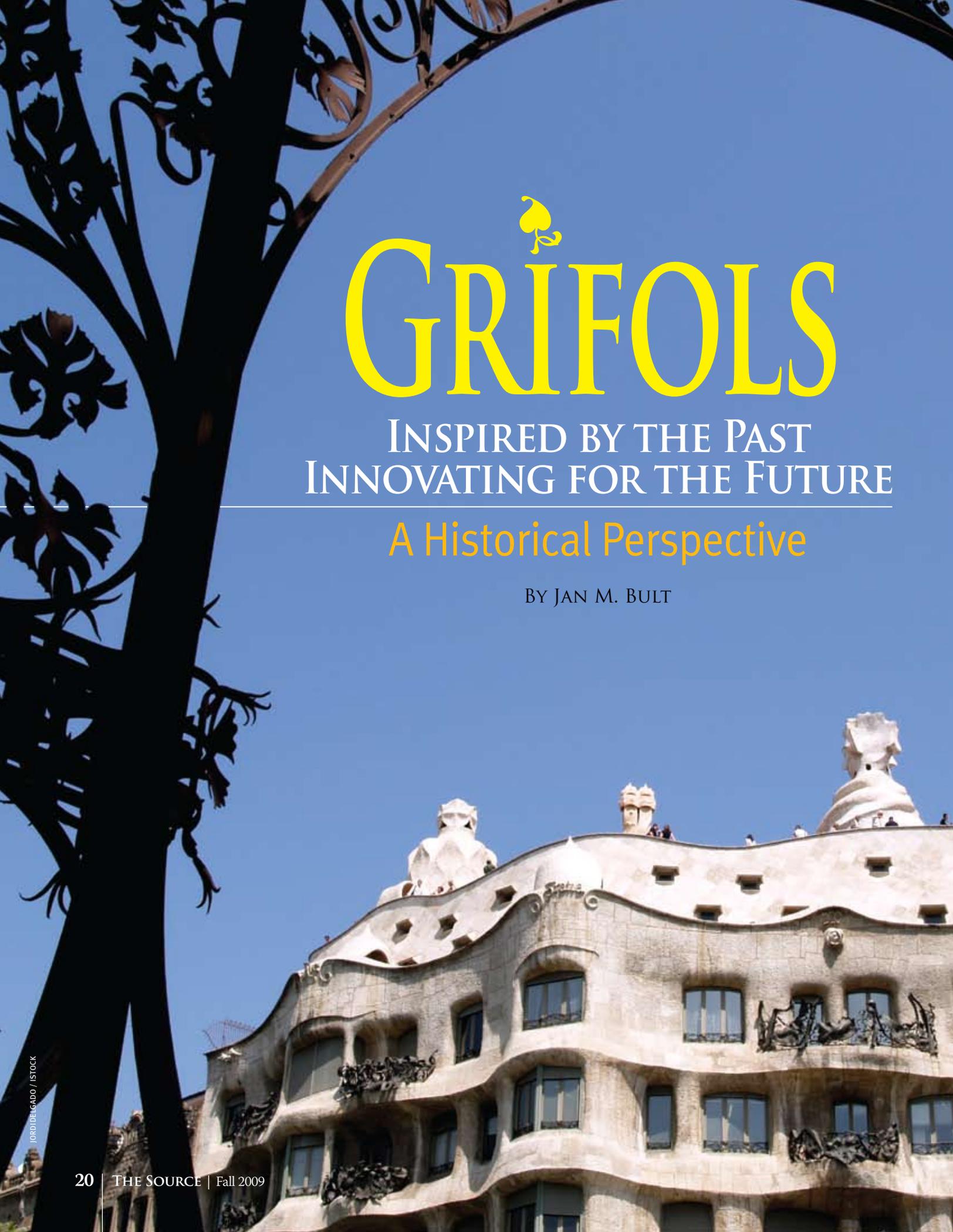
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GRIFOLS

INSPIRED BY THE PAST
INNOVATING FOR THE FUTURE

A Historical Perspective

BY JAN M. BULT



GRIFOLS IS THE FIFTH LARGEST COMPANY in the plasma protein fractionation industry with headquarters in Barcelona, Spain. The name comes from the family who founded the company. It all began in 1857 with Jose Antonio Grifols Morera, a medical doctor who practiced homeopathy first in Vilanova and later in Barcelona. He was so popular with patients that they were waiting on the stairs for their turn. His work was succeeded by Jose Antonio Grifols, the grandfather of the current President of Grifols. He followed in his father's footsteps and studied medicine and also practiced homeopathy. The most important decision he took that affected the future of the company was to go to Germany to study hematology for two years. Very early he knew the importance of microbes and became very meticulous about hygiene. He would never touch anything that was touched by someone else. His hand washing routine was very intense. He would not touch a coin and in a restaurant he always used a napkin to clean silverware before he used it. Bread was never touched with his hands and he drank beer with a straw. In 1940 he started the company that later became GRIFOLS.

His son, Victor Grifols Lucas, an inspired technology visionary, continued to build on the establishment of the Grifols company. He was born in 1919 and with his wife, Nuria, he has five children – Victor, Quique, Nuria, Alberto and Raimon. Three of them are active in the company, one is working as a lawyer and is providing legal counsel and another one is practicing medicine. Many people know Victor Grifols Roura, the current President and CEO of the company. Almost 25 years ago in 1985 he took over from his father as the Managing Director of Grifols, following the family tradition.

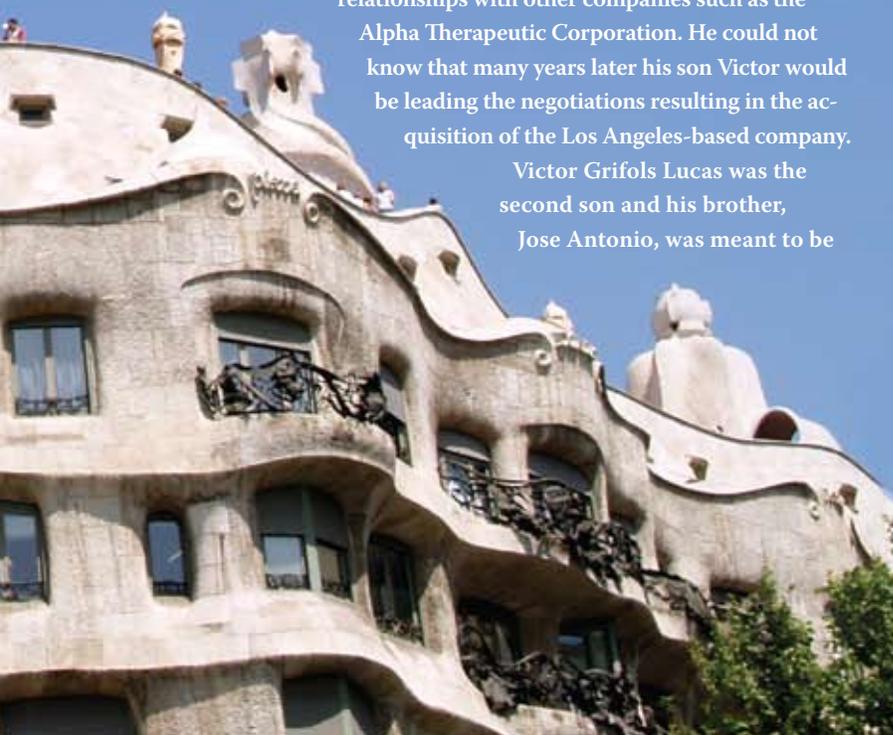
Victor Grifols Lucas, who is 90 years old, had a vision to further develop the company. He forged relationships with other companies such as the Alpha Therapeutic Corporation. He could not know that many years later his son Victor would be leading the negotiations resulting in the acquisition of the Los Angeles-based company.

Victor Grifols Lucas was the second son and his brother, Jose Antonio, was meant to be

the natural successor of his father. Jose Antonio was a physician with an enormous interest in blood and plasma. He was the first scientist to present his work on plasmapheresis as a real and reliable technique to obtain plasma in Lisbon and also the first in publishing them in 1952. He felt that there had



Above: Victor Grifols Lucas
Left: La Pedrera (Casa Mila) by Antoni Gaudi





Pictured left to right: Victor Grifols, Victor Grifols Lucas and Alberto Grifols.

to be a way to re-infuse the red cells to the donor to avoid waste of red cells when collecting plasma. He foresaw that plasmapheresis would be the technology that would be used for the collection of plasma. He also went to Boston to visit Dr. Edwin J. Cohn, the developer of the fractionation technology that is still used as the basis for the manufacturing of so many plasma protein therapies. But then a tragedy happened. Jose Antonio went to a medical congress in Rome, came back ill and thought he had a flu. His father looked at his blood under the microscope and found the devastating evidence of leukemia that then took his life two weeks later.

Victor Grifols Lucas experienced a great deal of changes when the company was still growing. He is considered by many as a hard worker, and as a matter of fact, he is still working developing new ideas and providing technical advice to the company. This exceptional man experienced the Spanish Civil War, the Second World War, economic pressures prior to the re-installment of democracy in Spain and was able to stay focused and grow

a company that developed and continues to have an excellent reputation. As an example of his pragmatism and ingenuity, after the Civil War there was not a lot of material available, so he designed a lot of technical equipment by himself. "You had to do it yourself", he said.

His father and brother went to Lausanne, Switzerland to learn more about microbiology and freeze drying. Many problems were faced in the beginning. There was no vacuum pump available and gas was used with a petrol flame to evaporate the water. One can only imagine the difficulties. There was no choice other than trying to be inventive because under the Franco regime, it was difficult to obtain money.

Grifols has a museum in Barcelona that highlights many of the historical developments of the company. Currently, a second museum is in development that will be opened in Los Angeles, California. On an evening in May 2009, I had the opportunity to meet several of the Grifols leadership in their museum and learned a lot about the history of the company.

Walking around in the Grifols museum is very impressive. There are multiple examples of equipment developed by Victor Grifols Lucas. While listening to this man when he tells the fascinating stories that go with it, you realize that a lot of excellent work has been done in the 1940s and 1950s that formed the basis for an industry that saves so many lives.

As mentioned earlier, his son Victor Grifols Roura is currently leading a successful organization. He is extremely proud of his father and shows that. Many in the company welcome the continuing advice that they receive from him. His advice is respected, helpful and leads to continuous improvement.

Both father and son are unanimous when it comes to safety. Safety starts with the donor and it underscores the importance of collection centers and the good work that is done by all involved in the collection of plasma.

GRIFOLS is more than a name. ●

JAN M. BULT is PPTA's President



The Grifols leadership team with Jan M. Bult, PPTA President, at the Grifols headquarters in Barcelona, Spain.

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KIM KOEHLINGER REMEMBERS meeting a woman a few years ago in intensive care who was so paralyzed by the effects of Guillain-Barré-Syndrome (GBS) that she thought she would never recover. Koehlinger, who battled a similar devastating illness 17 years ago and is a living example that she would one day recover, offered words of encouragement and understanding at a critical time, but in this case, they fell on deaf ears. When he met the woman again about a year later as she walked, under her own power, into a meeting, she told Koehlinger that she had heard him, but didn't believe him. "I thought I'd be that way forever," she said.

As a fighter who has recovered from Chronic Inflammatory Demyelinating Polyneuropathy (CIDP), a rare and debilitating disease of the peripheral nerves that renders its victims almost completely paralyzed, Koehlinger knows all too well how frightening the disease is and how easily the feelings of hopelessness can take over.

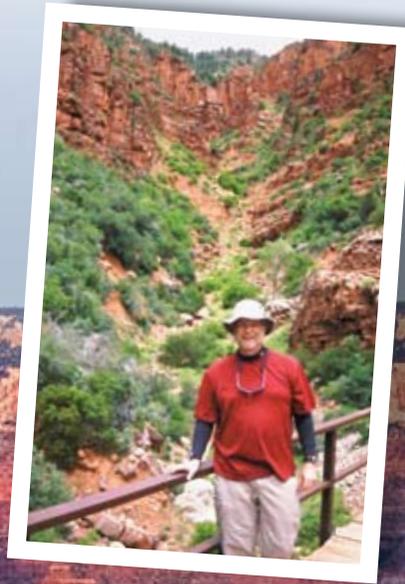
Piecing together the puzzle

The first sign that presaged Koehlinger's illness was a tingling in his left eyelid. He recalls it vividly as a strange sensation that began as he attended a Valentine's Day luncheon in February of 1992—like something was caught in his eye. A short

time later, he noticed upper back pain he initially attributed to over exertion at the gym, but it continued to worsen and progressed to Bell's palsy, the generic term used to describe paralysis and an inability to control facial nerves, on the left side of his face. "I used my fingers to blink my eyelids and eventually had to

apply paper tape to hold them shut at night so I could sleep," Koehlinger said.

From then on, Koehlinger continued to deteriorate. The pain radiated across his back and extended to his elbows and knees. His neurologist continued to hunt for an answer. Doctors performed numerous tests, but simply hadn't gathered enough clues

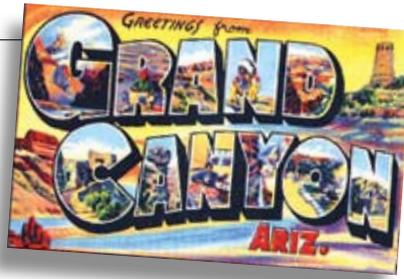


EVANS ARTS PHOTOGRAPHY / ISTOCK

FROM PARALYSIS TO TREKKING

ONE OF THE WORLD'S GREAT WONDERS

Recovered for years from CIDP, an Indiana native shares his journey...



to solve the mystery. The night before he entered the hospital, Koehlinger, a Fort Wayne, Indiana native and avid fan of the local minor league hockey team, the Komets, noticed that he had trouble walking up the steps at the game. And for the first time, when a goal was scored and the crowd cheered, he needed to plug his ears, because the noise was so intense. (Koehlinger learned later that muscles in his middle ear had become paralyzed.) Koehlinger had an appointment with his neurologist the next day for a nerve conduction study and discussed his latest symptoms. “A light bulb went on,” Koehlinger said. “The doctor said, ‘I know what you have.’”

By the end of February, Koehlinger was hospitalized. This was his initial attack, and the first diagnosis was GBS.

Restricted treatment options limit recovery

Once in the hospital the trial and error of treatment began. At the time, intravenous immune globulin (IVIG) was experimental for GBS, and insurance refused to cover it, so instead the hospital started with plasma exchange. However, Koehlinger continued to decline, and despite numerous plasma exchanges, there was little positive progress. “When I went to rehab on March 10, I was com-

pletely paralyzed,” Koehlinger said. “I could only move my head from side to side.”

He remembers having to rely on friends to help feed him during much of his nearly seven week hospital stay. While Koehlinger did walk out of the hospital nearly two months later, he had no idea that it would only be a short remission.

IVIG therapy a turning point

About two weeks after his hospital discharge, Koehlinger started to relapse. When he tried a few rounds of plasma exchange again, he simply got worse. At this point, his doctor finally stated that they needed to try IVIG therapy. Koehlinger started with IVIG every day for five days, and, “the results were dramatic,” he said. The deterioration stopped and he found himself stable. “For every day that I went downhill, it would take weeks or months to get back to that point, so the results were remarkable,” Koehlinger said.

Koehlinger received IVIG as an outpatient at the hospital throughout the balance of 1992 when periodic relapses occurred. At that point, his diagnosis officially changed from GBS to CIDP given the numerous relapses he experienced. He continued only to receive IVIG when he was symptomatic, rather than as a preventive course of treatment. This protocol went on until 1996

The Grand Canyon
is a steep-sided gorge
carved by the Colorado River
in the United States
in the state of Arizona.



Having battled CIDP years ago, Koehlinger has taken several hiking trips to the famous site in the years since recovering. Kim Koehlinger (third from the left in photo below) with family and friends during a recent hiking trip to the Grand Canyon.



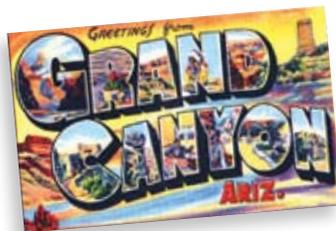
when Koehlinger had a serious relapse, and after nearly four years of trial and error treatment, Koehlinger and his doctors decide to change their approach and start a regular course of IVIG every two to three weeks, whether he was symptomatic or not, along with a course of prednisone and an immunosuppressant. Even while recovering, Koehlinger acknowledged how difficult it was for him, “The fear is so intense,” he said. “The first thing you feel or even think you feel—because your mind plays tricks on you—I would call the doctor.”

Koehlinger stayed with the new regimen for roughly 18 months, eventually weaning off of the prednisone and immunosuppressant, and sticking just with the IVIG. Koehlinger recalls that at some point in the years to follow, he changed to back-to-back IVIG treatments for two days, changing the frequency to about every six weeks.

During this time, with no relapses, Koehlinger says his mindset started to improve. He admits, “The whole thing made me crazy, and my depression was pretty severe during that time.” He adds that as a patient with such a severe condition, he turned inward and the illness, any illness like this, impacts everyone in your life.

Recovery leads to “grand” hike

Koehlinger kept up his IVIG treatments until September 2002, when after having the time extended to nearly two months, he stopped feeling the triggers. He credits IVIG treatments with making each relapse less severe until they finally subsided. At last, for Koehlinger, it was time to start living again. In January 2003 with a group of friends, he hiked to the bottom and back to the top of the Grand Canyon. He described standing at the top and looking out at the Canyon after coming through such a traumatic health crisis as nothing short of amazing.



He’s been back for the challenge three times since then.

Koehlinger says that overcoming his CIDP is the most difficult thing in life he’s had to face. He stresses that you can’t give up and that a positive mental attitude is vitally important. He recalls how quickly things changed for him back in 1992—from managing his own business and planning a ski trip with family, to the inability to blink to nearly complete paralysis in a matter of weeks.

Staying connected

Since recovering, Koehlinger has been involved with the GBS/CIDP Foundation International where he has served as president and currently is on the board of directors. Koehlinger continues to visit patients, typically in rehabilitation institutions to offer encouragement to patients and families whenever possible. “To give someone hope,” he says. “To explain that it is not permanent when people wonder, ‘am I going to be like this for the rest of my life.’”

Koehlinger adds that he’s had to turn the experience into something positive for his own well being. “I look at this as, ‘if I can’t do something to help someone to get through this, then this rotten experience was a waste of my time.’ ●

KYM KILBOURNE is PPTA’s Assistant Director, North America Communications.

NEW ORLEANS

Charting the Course



 PPTA

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RARE PLASMA RELATED DISORDERS

RARE DISEASES ARE RIGHTLY SEEN AS A GROWING EUROPEAN HEALTH PRIORITY. Both the European Commission and several European Union (EU) Council Presidencies have held rare diseases as a priority area in health care policy. Many rare diseases cannot be diagnosed or treated. Rare plasma disorders can be diagnosed and successfully treated, but unacceptable diagnosis rates for some plasma disorders means that treatment can only begin late, or not at all.

We believe that the European Commission proposals are a positive step that must be supported by EU Member States. Rare diseases include over 200 plasma protein disorders such as Hemophilia, Primary Immunodeficiencies, Guillain-Barré syndrome, C1 inhibitor deficiencies and Alpha-1 antitrypsin deficiency many of which are treated with therapies derived from human plasma. The rarity of some of these disorders often means that information on treatment options, symptoms, diagnosis and access to treatment is not optimal and that unnecessary associated health care costs are incurred due to a higher rate of hospitalization, increased number of missed days of school and work and increased infection rates.



EU Member States represented – 8

Belgium, Cyprus, Germany, Ireland, Netherlands, Romania, Slovakia, United Kingdom

Political groups represented – 4

- ALDE** (Alliance of Liberals and Democrats for Europe)
- EPP-ED** (European People's Party and European Democrats)
- GUE/NGL** (European United Left–Nordic Green Left)
- PES** (Party of European Socialists)

European Parliament committees represented – 13

- | | |
|-----------------------------------------|-----------------------------------|
| Environment | Human Rights Sub. Committee |
| Public Health and Food Safety | Regional Development |
| Internal Market and Consumer Protection | Fisheries |
| Economic and Monetary Affairs | Agriculture and Rural Development |
| Employment and Social Affairs | Culture and Education |
| Industry | Civil Liberties |
| Research and Energy | Justice and Home Affairs |
| Budgets | Foreign Affairs |

We assert and emphasize that the following actions should be supported by the European Parliament, European Commission and European Council:

1 Each EU Member State should have a dedicated rare diseases action plan, using tools such as professionally run national patient registries and networks of reference centers for diagnosis and treatment, as proposed by the European Commission in November 2008.

2 European patients have the right to access the treatment that they need. In the case of life-threatening plasma protein disorders, this requires the widest possible access for patients to plasma protein therapies and the implementation of appropriate treatment levels of care, especially in EU Member States where access to treatment is restricted and/or not optimal.

3 The need for an adequate supply of safe and high-quality human plasma for further manufacturing into therapeutic products.

4 It is proposed to create a European Parliament Interest Group on Rare Plasma Related Disorders that would meet regularly to ensure that the unique challenges facing these patient groups are appropriately taken into account in relevant EU legislation and actions. Face-to-face consultation between EU decisionmakers and patients' groups facilitates a greater understanding of the challenges faced by patients and where the EU can contribute and add value. This is reflected in the recently created liaison mechanisms between plasma

related patient organizations and the European Commission.

5 In future communications, legislation and discussions in the European Union, the intrinsic differences between the collection of whole blood and the collection of plasma and the medicinal products derived from it should be considered carefully. The logical separation of whole blood and plasma will lead to a better understanding of the unique nature of the therapies for rare plasma related disorders, and ultimately better care and treatment for patients.

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INSIDE PPTA

PPTA MEMBER NEWS
FROM AROUND THE GLOBE



istics by either exempting them or requiring U.S. Food and Drug Administration (FDA) guidance; and opposing the expansion of the 340B drug pricing program that increasingly makes plasma protein therapies subject to unsustainable deep federal discounts. PPTA staff also has been reaching out to reporters covering health policy in order to further promote the Association's position on language that affects patient access and plasma protein therapies. As a result, a story appeared featuring the Association's policy position and white paper on follow-on biologics in FDA Week, and Association documents that support PPTA's position were posted on Inside Health Policy.com. The bills have been moving through Congress at breakneck pace;

SOLVENT DETERGENT TREATMENT DATA

› Solvent/Detergent (S/D) treatment is an established virus inactivation technology that has been applied in the manufacture of medicinal products derived from human plasma for more than 20 years. PPTA's Pathogen Safety Steering Committee (PSSC) has collected data on the inactivation of enveloped viruses by S/D treatment, in total the results from 308 studies were compiled. The data demonstrate the robustness of virus inactivation by S/D treatment for a broad spectrum of enveloped test viruses and process parameters. The manuscript has been accepted for publication in the September issue of the scientific journal *Transfusion*. It is also noteworthy that European and U.S. regulators have indicated they will reduce the respective regulatory requirements for robustness studies, should such a database should become publicly available.

HEALTH CARE REFORM IN THE U.S.

› Throughout the summer leading up to the August Congressional recess, PPTA staff and the members of the Federal Affairs Steering Committee (FASC) have been monitoring the evolution of House and Senate health care reform legislation, and specifically advocating on behalf of key provisions that affect access to plasma protein therapies. Those key provisions include comparative effectiveness research that puts patients first rather than cost; biosimilar legislation that recognizes plasma protein therapies' unique character-

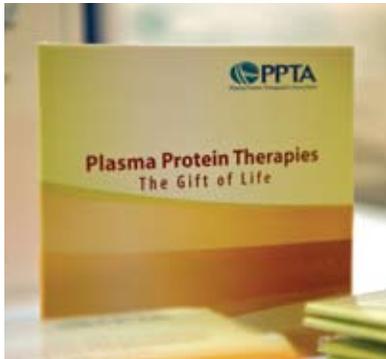
istics by either exempting them or requiring U.S. Food and Drug Administration (FDA) guidance; and opposing the expansion of the 340B drug pricing program that increasingly makes plasma protein therapies subject to unsustainable deep federal discounts. PPTA staff also has been reaching out to reporters covering health policy in order to further promote the Association's position on language that affects patient access and plasma protein therapies. As a result, a story appeared featuring the Association's policy position and white paper on follow-on biologics in FDA Week, and Association documents that support PPTA's position were posted on Inside Health Policy.com. The bills have been moving through Congress at breakneck pace; for specific information about where each key issue stands, contact PPTA Federal Affairs staff or your company's FASC member.

IMAGE AND CREDIBILITY

› For many years, the source plasma collection industry was cautious about its profile as in the past negative media coverage regarding plasma collection practices was a regular occurrence. Over the past two years, however, PPTA has worked with members of the Source Board of Directors and Source Image Task Force and other industry groups to create a multifaceted Source Industry Image and Credibility Campaign designed to profile the source plasma collection industry to the general public.

To this end, PPTA worked to create a number of tools designed to create greater awareness of the need for plasma donations and life-saving therapies. The projects implemented since Fall 2007 have included the following:

- **DonatingPlasma.org**, a website dedicated to plasma donation, providing credible information about the industry and information to current and potential donors.
- During a 10-week campaign, PPTA ran ads on **Facebook**, a free-access social networking website that enabled the Association to drive potential plasma donors to donatingplasma.org.
- A CD-ROM, "**Effectively Communicating with the Media**" was produced out of a need for additional information to be provided to plasma collection center managers and employ-



ees providing tips on handling media calls more effectively.

- A **PPTA DVD “The Gift of Life,”** was provided to plasma collection center managers to help educate donors and employees about the industry.

- PPTA worked with the National Association of Farm Broadcasters (NAFB) to create a **rural radio campaign** that would run in several locations where PPTA members have centers in less populated areas of the U.S.
- A **community-based presentation** was created that could be used by members to discuss the positive impact that plasma collection centers have on the community at large.
- **Media talking points** were created to address media inquiries and provide appropriate industry messages regarding plasma donation and the volume of donations over the last several years with members.

DONATINGPLASMA.ORG

› Since the launch of donatingplasma.org in September 2008, traffic by browsers has steadily increased, with more than 14,000 users viewing the site last month. One of the most consistently popular areas on the website is the “Find A Donor Center” feature that allows people who are interested in donating plasma to find a center by search-

ing by zipcode. Although this feature has been very useful for potential donors, PPTA staff have worked to enhance this feature on the website. With this in mind, improved mapping software was launched over the summer that allows browsers to search by state and city, providing additional information at a glance. In addition, the inclusion of Google Maps software on the website allows potentially interested donors to pinpoint the location of a plasma collection center within minutes. PPTA believes these new and improved features of donatingplasma.org will provide browsers with the additional information they need in order to quickly determine the distance of a collection center in their vicinity. ●



GLOSSARY OF TERMS

| | | | |
|---------------|-----------------------------------------------------------|--------------|-----------------------------------------------------------------------|
| AAV | Adeno-Associated Virus | MASAC | National Institutes of Health Medical and Scientific Advisory Council |
| CBER | Center for Biologics Evaluation and Research | MEPs | Members of European Parliament |
| CIDP | Chronic Inflammatory Demyelinating Polyneuropathy | NAT | Nucleic Acid Testing |
| EAHAD | European Association for haemophilia and Allied Disorders | NHF | National Hemophilia Foundation |
| EAPPI | European Association of the Plasma Products Industry | PIDD | Primary Immunodeficiency Disease |
| EHC | European Haemophilia Consortium | PSSC | Pathogen Safety Steering Committee |
| EMEA | European Medicines Agency | S/D | Solvent Detergent |
| EUHASS | European Haemophilia Safety Surveillance System | TSE | Transmissible Spongiform Encephalopathy |
| GBS | Guillain-Barré Syndrome | vCJD | variant Creutzfeldt Jakob Disease |
| IDWG | Interdisciplinary Working Group | WNV | West Nile Virus |
| IVIG | Intravenous Immune Globulin | | |

STAFF



ILKA VON HOEGEN, PH.D.

MY NAME IS ILKA VON HOEGEN.

I am PPTA's Senior Director, Quality and Safety. I joined PPTA in January 1998. Over the years I worked primarily with the Pathogen Safety Steering Committee (PSSC) to respond to challenges imposed by viruses and transmissible spongiform encephalopathies (TSEs) or prion diseases. With the Regulatory Affairs Steering Committee (RASC) we address issues to improve the regulatory environment and facilitate patient access to plasma protein therapies. In addition, I work with a number of task forces on upcoming flashpoints and I am the liaison with the German Arbeitsgemeinschaft Plasmaproteine herstellender Unternehmen (AGP). In addition, I also coordinate European epidemiology issues

Tell us about your background.

I was born in Stuttgart, Germany and moved to Cologne when I was nine. I studied biology at the University of Cologne. Under the kind supervision of my first clumsy steps in the science world by Prof. Ruth Ehring, I completed my thesis on the E. coli galactose operon at the Genetics Institute. During my doctoral studies at the Max Planck Institute for Züchtungsforschung in the group of Prof. Joachim Schröder in Cologne, I worked on Agrobacterium tumefaciens induced plant tumors and finished my degree at the University of Freiburg. I continued my odyssey through the scientific world and moved to the German Cancer Center in Heidelberg where I studied the Interleukin 1 receptor in the department of Prof. Peter Krammer, followed by a post doctoral fellowship at Stanford University in the U.S. with Prof. Jane R. Parnes. After three very happy years and 22 days, my husband and I decided to leave California and to return to Germany. I found a position at E. Merck in Darmstadt to develop antibody-targeted tumor therapies. From there I moved to Belgium and spent 14 months in the regulatory department of Smith Kline Beecham,

when a friendly colleague pointed out an advertisement for a position at the European Association of the Plasma Products Industry (EAPPI), which ultimately became PPTA. As a hobby I like golf, although I have never arrived at a good handicap. My husband of 23 years always asks: why don't you learn it, when you like it so much?

What is your proudest professional achievement?

The acceptance of the PSSC's manuscript on the robustness of Solvent Detergent (S/D) treatment for publication has been personally gratifying for me. This was a particularly cumbersome process, and I am very pleased with the positive outcome. U.S. and European Union regulators have indicated that they would lower their requirements for robustness studies for S/D treatment, when a peer reviewed data base is available. This would be an example of how science can provide regulatory relief.

What is most rewarding about working in this industry?

When I started with PPTA in January 1998 the industry was not very

well perceived by many stakeholders, I believe that the PSSC and all the other working groups and task forces with which I have had the pleasure to work with over all these years, significantly contributed to increase the credibility of PPTA's member companies with regulators, clinicians and most importantly patients. Today we are seen as reliable partners in the joint effort to ensure the safety and availability of these often lifesaving therapies. ●



2009

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|------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| September 11–13 | European Haemophilia Consortium Annual General Meeting Vilnius, Lithuania |
| September 15–18 | 42nd Annual Meeting of the German Society for Transfusion Medicine and Immunohaematology (DGTI) Rostock, Germany |
| September 24–25 | Sixth Global Forum on the Safety and Supply of Treatment Products for Bleeding Disorders Montreal, Canada |
| October 8–11 | 15th Australian and New Zealand Haemophilia Conference Brisbane, Australia |
| October 11–14 | 22nd Annual Congress European Society of Intensive Care Medicine Vienna, Austria |
| October 15–17 | LASID 2009 - First Meeting of the Latin American Society for Primary Immunodeficiencies Cartagena de Indias, Colombia |
| October 18–21 | HAA 2009 - Annual Scientific Meeting of the Haematology Society of Australia and New Zealand, the Australian and New Zealand Society of Blood Transfusion and the Australasian Society of Thrombosis and Haemostasis Adelaide, Australia |
| October 24–27 | AABB Annual Meeting New Orleans, USA |
| October 25 | PPTA Source Business Forum New Orleans, USA PPTA members only |
| October 26–27 | 10th Workshop EPPOSI on Partnering for Rare Diseases Therapy Development Brussels, Belgium |
| October 29–31 | 61st Annual Meeting of the National Hemophilia Foundation (NFH) San Francisco, CA |

2010

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|-----------------------|----------------------------------------------------------------------------------------------------|
| March 9–12 | International Symposium on Intensive Care and Emergency Medicine Brussels, Belgium |
| March 13–14 | 2nd Pan-European Conference on Haemoglobinopathies Berlin, Germany |
| March 16–17 | International Plasma Protein Congress 2010 Berlin, Germany |
| April 10–17 | 62th AAN - Annual Meeting of the American Academy of Neurology Toronto, Canada |
| May 22–25 | 56th Annual Meeting of the Scientific and Standardization Committee of the ISTH Cairo, Egypt |
| June 10–13 | 15th Congress of the European Hematology Association Barcelona, Spain |
| June 15–16 | Plasma Protein Forum Reston, VA, USA |
| June 26–July 1 | XXXIst International Congress of the ISBT Berlin, Germany |
| July 10–14 | Hemophilia World Congress Buenos Aires, Argentina |
| October 7–10 | XIVth Meeting of the European Society for Immunodeficiencies Istanbul, Turkey |
| October 9–12 | AABB Annual Meeting Baltimore, MD, USA |
| October 10 | Source Business Forum Baltimore, MD, USA PPTA members only |

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- Cut costs by reducing frequency of integrity testing

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