THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

WINTER 2008

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"Demand Management" for IG in the UK

Albumin: History and Controversy

Hemophilia in the Philippines





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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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INSIDE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

- 2 IN MY VIEW Improving Patient Access Worldwide Requires Collaboration
- 4 "Demand Management" in The United Kingdom
- Hemophilia in the Philippines
 Project Share: Hope for the Forgotten
- PPTA Launches New Website www.DonatingPlasma.org
- 10 INTERVIEW Wolfgang Marguerre
- 12 Albumin
 History and Controversy
- PIDD Stakeholders Meet in the Netherlands
- 16 Barack Obama's Presidential Win Denotes Big Change in the U.S.
- Report on 2008 Source Business Forum
- 20 German Donor Dedicates 30 Years and 1,150 Donations to Saving and Improving Lives
- 22 Donor Epidemiology
 Top of Mind for Plasma Protein
 Therapeutics Industry
- 26 News
- 28 MEET THE PPTA STAFF Brenda Norman and Mary Straub
- 29 Events





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. BAXTER BIOSCIENCE BIOTEST AG GRIFOLS, S.A. TALECRIS BIOTHERAPEUTICS CSL BEHRING

IN MY VIEW

IMPROVING PATIENT ACCESS WORLDWIDE REQUIRES COLLABORATION



THERAPIES. In my job, I travel around the world and have the opportunity to meet many different people. Sometimes I am confronted by hard reality when I meet patients who have limited or no access to these life-saving therapies.

Recently, I attended a conference on primary immunodeficiency disease. In the same week, my wife traveled to the Philippines on a mission for hemophilia patients. Here are two stories from both of our experiences:

A physician in the Philippines must make a hard decision when there is a single dosage of clotting factor and two critically ill hemophilia patients requiring immediate care. The therapy is given to the motorcycle accident victim. The other patient, who was admitted with a shoulder bleed, later dies of a cerebral hemorrhage.

What can be said to comfort two parents in India who have already lost two sons because of primary immunodeficiency disease and are not sure whether they can afford the costs of therapy for their third son? The price of the therapy produced by a local manufacturer had gone up so much that it exceeds the costs for the therapy in the United States.



These stories are heartbreaking and difficult to rationalize. It can be wrenching when we realize that the fundamental problem in many countries is that the government is unable or unwilling to set up a system of proper reimbursement for therapies needed by patients with rare diseases. It is incomprehensible that some governments consider defense spending a higher priority than the national health budget and that in other countries we see that public money is used to further the wealth of a limited number of individuals.

There are also good examples of what a responsible government can achieve. In the time of the Soviet Union, the government dogma was that hemophilia did not exist in that country. We all knew better. Today, the government has made a commitment and the treatment levels for persons with hemophilia have risen to approximately three units per capita, which is much better than it was. In meetings, we see many more Russian patient groups then previously when there were so few.

The only way to improve access to critical therapies is to bring all parties together—patients, physicians, research experts, industry and government. Only when there is a willingness by all parties to cooperate and take responsibility, than there can be change. If that change is communicated widely—we can all live in a better world. ●



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he United Kingdom's much heralded Demand Management Plan (DMP), with its associated clinical guidelines and new immunoglobulin database, first came into effect in July 2007 to address concerns over sustained access by patients and physicians to a choice and variety of immunoglobulin therapies.

"DEMAND," MANAGEMENT" IN THE UNITED KINGDOM

BY CHARLES WALLER

FOLLOWING A REVIEW AND STUDY

of clinical practice in England by the firm

Deloitte Touche Tohmatsu this expensive plan was
introduced in response to perceived "product shortages."

Globally, PPTA members have responded to the rapidly
increasing demand for immunoglobulin in three ways:

1. Improved IgG production efficiency raising the recovery of immunoglobulin by 30%-plus. According to various sources, in five years this has increased the average recovery of im-

munoglobulin from a liter of plasma from about 3 grams to around 4 grams with a continuing upward trend in the average.

- 2. Increased plasma collections, moving worldwide collections up from around 10 million liters in 2004 to nearly 14 million in 2007 and the figure is still rising (see figure 1).
- **3.** Increased manufacturing capacity resulting in steadily increasing distribution of immunoglobulin in the United Kingdom (see figure 2).

The combined impact of these three measures has resulted in an all-time record in terms of immunoglobulin distribution, globally, regionally, internationally, and in the United Kingdom.

So, if in the past some physicians and patients in the United Kingdom found it difficult to have continuous access to the immunoglobulin of their choice, then today the situation should be greatly improved.

The impact of the DMP itself may be another story. There has to be a concern that the ominously titled "Demand Management Plan" ostensibly created with good intentions could result in patients and physicians again finding it a challenge to get their therapy of choice. Early reports from physicians suggest that most of them are unaware of the plan and the increased bureaucratic burdens it imposes on them. The Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) patient group is also receiving anecdotal reports that patients are finding it difficult to get state of the art treatment with immunoglobulins, "because of the DMP."

PPTA's United Kingdom group is working hard to gauge the actual impact on patient access and has started by carrying out some research to monitor the actual impact on physicians and pharmacists.

CHARLES WALLER is PPTA's Vice President of PPTA Europe

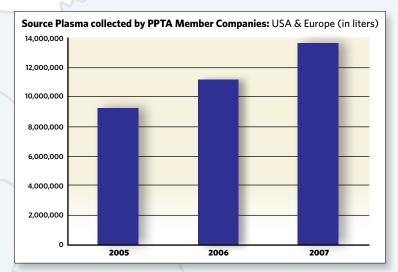


Figure 1. Annual Collection of plasma in PPTA plasma centers in Germany, Austria, the Czech Republic and the U.S. Source: PPTA

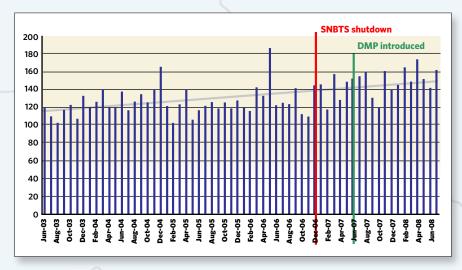


Figure 2. Monthly distribution of immunoglobulin in the UK by PPTA members. SNBTS: Scottish National Blood Transfusion Service. *Source: PPTA*

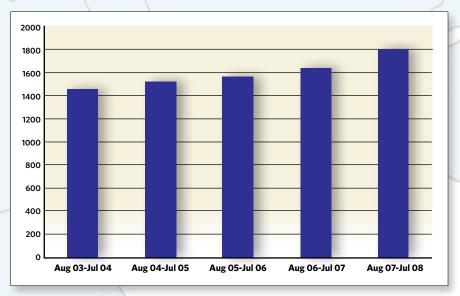


Figure 3. Annual distribution of immunoglobulin in the UK by PPTA members August 2003 – July 2008. Figure does not include UK national fractionator BPL, which is believed to supply over 45 percent of the UK market. *Source: PPTA*

BY ROSE NOYES

rown fingers cradle the white sheet of paper. The young Filipino bends his back to better see his writing. In the still, hot room he breathes deeply, almost a sigh, then passes the paper to his neighbor who also writes and passes the paper on. Around the worn brown table serious young men in a colorful array of t-shirts eagerly wait to add their contact information—name, phone number, email address—to the growing list. I have asked everyone assembled in the large meeting room to write their information for me, not understanding that implicit with this request is the unspoken understanding that I can somehow help the people around the table. Not a word is spoken by the one signing his name, the act of putting name to paper is not a chore but an act of hope. No one chafes at the heat...not even the mothers, sisters and little brothers...but legs and arms are frequently moved, easing painful joints. Everyone listens intently to the Catholic priest with the almost Irish lilt to his speech. He points to a large used fruit dehydrator on a table along one wall. Father Donald Kill, Father Don as he is known to all, explains the virtues of his people with hemophilia (PWH) selling dried fruit to students in schools as a way to make money. At his side sits Laurie Kelley, head of LA Communications and the founder of Project Share and Save One Life. Her son was diagnosed with hemophilia as an infant 21 years ago. She has flown the 8,000+ miles from Boston to the Philippines to see firsthand the results of her company's efforts to distribute donated clotting factor to hemophilia patients in the Philippine Islands. I sit next to Laurie and Father Don. We each have a mission: Laurie will experience firsthand the impact of Project Share's factor donations. Father Don as trip organizer will be our travel guide plus bodyguard. After living in the islands for almost 32 years he is fluent



Manny S., a patient with hemophilia, listens intently during a discussion with Father Donald Kill and Laurie Kelley.

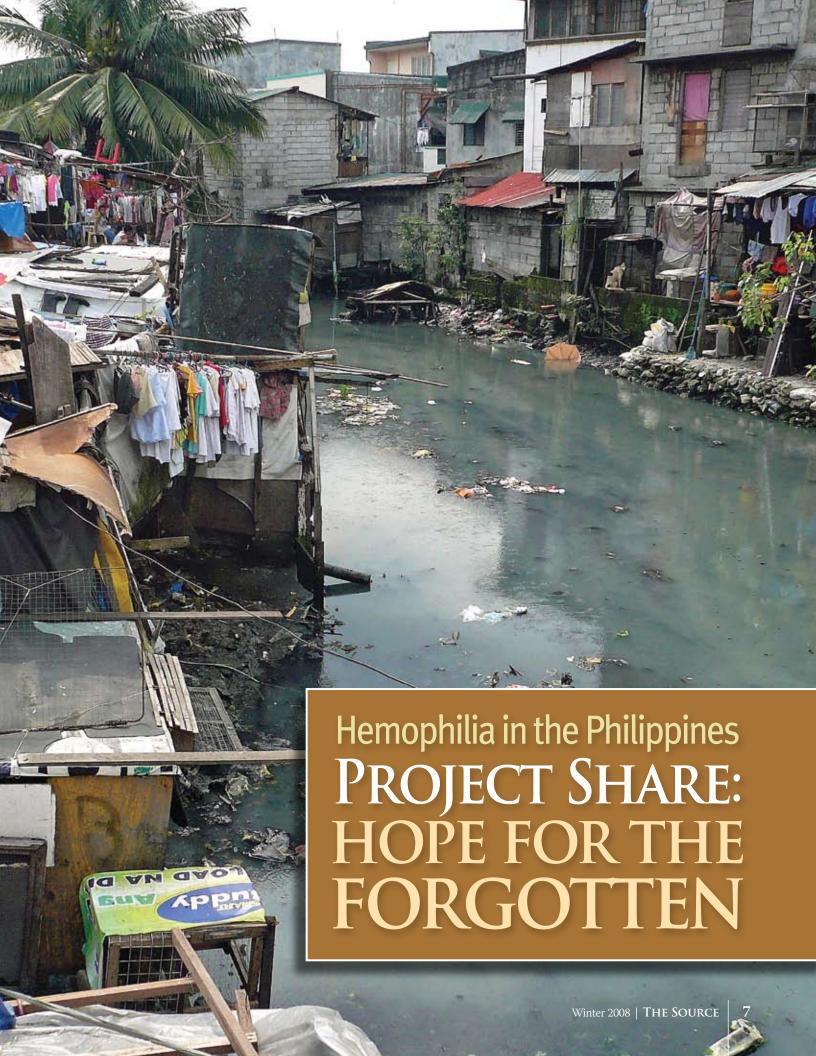
Right: Living quarters in Manila, The Philippines.

in Cebuano, or Visayan, a southern dialect. And I will document the trip. We ask questions, "Do you go to the hospital when you have a bleed?" No, there is no medicine to treat us, and we must pay for everything except the bed and the doctor's care, even the syringes and needles. If the clinic has fresh frozen plasma (FFP) or cryoprecipitate, we must pay for that as well. And that could mean one-half to one month's wages or more. "How much did it cost to travel here today?" About 300-500 pesos, or a day's pay. "How many of you have spoken to your elected officials and asked for help?" (Blank stares.) No one has, we have been told the government has more important health priorities. Also, if we lobby too vigorously, if we protest, we are afraid of the consequences. "How much will it cost to rent a storefront for an

Internet café?" *Maybe 1,500 pesos a month.* (In the Philippines where workplace prejudice is common for hemophiliacs, any alternative work scheme is worth a try, and many here are computer savvy and dream of using their talent to make money.) But always from everyone is the plea, "*Do you have any factor (concentrate) for us today? Can you help us get any?*"

I hold the sheet of paper with the names of the 13 young men. At the end of the meeting, one of them, Manny S., comes to me and asks if I know of an American sponsor who would help him sell his artwork and thus help support his family. He tells me he also makes lampshades from newspapers. His haunting eyes implored me to give him an affirmation, but all I can say is I will see what I can do. My heart feels like lead in my chest. When he turns away, I glance through the other names. Ruel C. wrote, "Im family man person. Pls help my 5 children for school- Thanks, Ruel [sic]". Yesterday, the first meeting with patients,







There were hardly any orderly rows of buildings, but haphazard tumbles of crazy, quilt structures with some so small, from the air they looked like dog's houses.

Yet, I could see in the city areas of commerce and finance with tall, impressive skyscrapers.



Top of page: A child walks in a typical street on the outskirts of Manila.

Below: Father Donald Kill, a Catholic priest, and Laurie Kelley, of LA Communications, talk with hemophilia patients. was just as difficult. I listened to Angelo Cuevas, a soft spoken young man, tell his story of suffering from repeated joint bleeds. I restrain myself from contorting in sympathy while Angelo, his voice sometimes breaking, described the howls of anguish that scared his neighbors while he pounded the wall with his fists because the pain drove him mad. Listening to the hemophilia stories left me feeling absolutely helpless. I knew that even though I was not a part of Project Share, my presence here automatically included me as a person *from the United States who has come to give us hope.* As each person held my gaze with his or her own, asking for relief I could not give, I felt miserable and depressed.

When I flew into the Ninoy Aquino International Airport, several days earlier, I drank in the lush green of the coastal plain and the patchwork of rice paddies surrounding Manila. I could see the mountains in the distance covered with neverending blankets of trees. But the human habitation disturbed me. Flying over the outskirts of the city, there were hardly any orderly rows of buildings, but haphazard tumbles of crazy, quilt structures with some so small, from the air they looked like dog's houses. Yet, I could see in the city areas of commerce and finance with tall, impressive skyscrapers. What were the living conditions of most people?

The compound housing Father Don's religious order, the Columbans, surrounds a white multi-story stucco building built around a large courtyard of banana and palm trees on a one way street. The building, in fact, every building in Manila, is stained dingy gray from the carbon soot belched by the aging Jeepneys (colorful indigenous minibuses) and other vehicles with no exhaust emissions controls. People hold handkerchiefs or hospital masks to their faces as they negotiate the cracked, heaped up pavement. On this street, as in almost every street in the city, power lines and telephone cables hang like medusa bundles of black spaghetti from tilted wooden poles. Little girls of five years rush out to our taxi to panhandle. They sometimes have small leis made of sampaguita (jasmine) blossoms. Pedestrians are in danger of "undisciplined" car drivers; jeepneys maneuvering; and pedal or engine driven tricycles for hire maneuvering in and out of traffic. The Philippines is classified as a "medium income country," yet the grinding poverty of its citizens is a direct negation of that appellation. It is the start of the cooler, rainy season and it is still difficult to leave our air-conditioned rooms. Climate control is too expensive for the majority of Filipinos; I can't begin to imagine the oppressive heat in full summer.

It is so easy to see dirt and squalor, and yet there is also beauty everywhere. The sky is filled with spires of pearly white clouds and the blue is every shade from cobalt to cerulean. There is a quiet dignity about the Philippine people. Their faith and their ability to care for each other sustains them when there is nothing else to keep them alive. They suffer quietly and try to hide their misery from public eyes. They are a very devout people and have conveniently located churches and basilicas for worship on every block, it seems. Even the megalithic Mall of Asia, the largest shopping center in Asia, has small store size (airconditioned!) chapels, where shoppers can listen to mass seven days a week.

Laurie and our intrepid linebacker of a companion, Father Don, already understand the many layers of complexity surrounding hemophilia in this part of the world. In a country where the governing elite regularly deflect charges of corruption and malfeasance there is scant room in the health budget for hemophilia. Patient groups try to provide help, but cannot reach those in the far-flung islands, over 7,000 in the Philippine archipelago. When a poor mother listens to her son's crying in the night from a swollen knee filled with blood...when the parents of a young girl undiagnosed with von Willebrand's desperately try to find our why her menstrual cycle does not end – *who will help?*

Rose Noyes is a guest writer for The Source.

Editor's Note: This article will be continued in the next issue of The Source.

PPTA LAUNCHES NEW WEBSITE WWW.DONATINGPLASMA.ORG

BY KARA FLYNN

quick perusal of the results related to the internet search term "plasma" often yields a great deal of information on plasma televisions, rather than the starting material for a wide range of life-saving medicines. While the donation of whole blood is well regarded and a much-needed activity and there is a great deal of information available on the world wide web, Internet users have often found a lack of information on the urgent need for plasma donations.

Recognizing a need to provide information to potential and current plasma donors, as well as underscoring the importance of plasma donation, which is critical in helping to save countless lives, PPTA launched www.DonatingPlasma.org. This new educational website features information on plasma donation, how it helps patients with chronic, life-threatening diseases, and will help users easily find their local IQPP-certified source plasma donation center.

PPTA designed this informative site in an effort to heighten public awareness of the importance of donating plasma. Donating Plasma.org is the only industry-wide website that seeks to explain and clarify the plasma donation process.

The site contains up-to-the minute information about plasma donation, including requirements for donor eligibility and donor health, frequently asked questions, and the time and commitment required to donate plasma. In addition, the site serves as a resource for those interested in donating plasma and who want to learn more about the patients who are helped everyday by someone who gives plasma.

DonatingPlasma.org offers information regarding the diseases and disorders treated with plasma protein

therapies, differences in blood and plasma collection, video segments offering real-life interviews with patients, plasma donors and physicians, and links to patient group organizations and other online resources.

To view the new website, please go to www.DonatingPlasma.org. lacktriangle

Kara Flynn is PPTA's Director of Global Communications.

EDWARD GRAJEDA / ISTOCK, WWW.DONATINGPLASMA.ORG, BONOTOM STUDIO



WOLFGANG MARGUERRE

By Jan M. Bult

you get, it is something you take."
When Wolfgang Marguerre started to

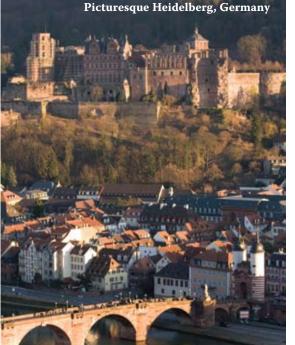
esponsibility is

learn how to play the violin at the age of six years, he could

never have imagined that 50 years later he would start recording CDs with classical music that he gives to many associates of his successful company, Octapharma. He especially enjoys playing his violin made by Lauretius Guadagini.

Twenty five years ago in his home in Paris, Wolfgang Marguerre and Robert Taub started a company that has become the largest privately held company in the plasma protein industry. The total number of personnel at the start was 2.5 and now it has grown to 2,400 employees spread over 27 offices in the world.

Having been active in this industry for many years, Wolfgang Marguerre strongly believes in the importance of a level playing field for all play-



ers that includes the private as well as the not-for-profit sector. He does not consider the not-for-profit manufacturers as easy partners. Both sectors provide patients with the needed plasma protein therapies, but in many countries, the public sector sector enjoys benefits that are

> not available to private sector manufacturers. This ranges from access to plasma as starting material to tax exemptions.

Wolfgang Marguerre grew up in Heidelberg, Germany, a city to which he returned a few years ago after having been away for 30 years. Heidelberg is famous for its historical castle, which actually can be seen from his home. As a student, he was lucky enough to own his own car. He proudly remembers that first car, his legendary red Messerschmidt. Today, this kind of car is a well-desired collector's item.

Octapharma is a healthy and strongly growing company with, according to Wolfgang Marguerre, the right balance between research, globalization, quality and production. For many years,



the company has obtained the required plasma for fractionation from ABC (America's Blood Centers) collection centers, a partner that he very much respects. Since more plasma is required to meet the growing needs of Octapharma, the decision was made to have their own plasma collection centers in addition to the existing suppliers. Currently, according to Mr. Marguerre, Octapharma owns approximately 40 collection centers, but it will not take very long before Octapharma doubles this number. At this point, Wolfgang Marguerre expresses his view that for

many years countries like the United States and Germany have been able to supply a lot of plasma, but in today's world there is a need to expand the collection of plasma to other countries. That expansion requires legislation and regulations to allow this collection. There could be a role for an Association like PPTA to assist in creating that option.

Wolfgang Marguerre believes in PPTA as an organization that provides a platform for open discussion and an exchange of opinions on relevant industry matters. For a strong organization, it is important that the focus of attention is spread among the different regions in the world. Octapharma has been a member since the year 2000, and Wolfgang Marguerre has been on the Board of Directors ever since.

Wolfgang Marguerre is full of energy and the word retirement is not a part of his vocabulary. He is very proud of his three children, who all are working in the Company:

One son Tobias, has responsibility for Scandinavia, Eastern

Europe and the plant in Stockholm. His other son Frederic is responsible for Canada, the United Kingdom, Australia and most of the Middle East. His daughter Christina recently gave birth to his first grandchild, making Wolfgang a proud grandfather. Christina is working as a Product Manager in the Hemophilia department.

is proud of the achievements of his company and that shows.

JAN M. BULT is PPTA's President.

Wolfgang Marguerre



BY PROFESSOR ALBERT FARRUGIA

ALBUMIN

HISTORY AND CONTROVERSY

The Winds of War

n May 8, 1941, Captain Douglas B. Kendrick of the U.S. Army reported on the use of human albumin in traumatic shock. His words have relevance as we continue to observe the benefits of this unique plasma therapy:

"...This patient was 20 years of age and was admitted to the hospital 16 hours after injury. He had a bilateral compound continued fracture of the tibia and fibula. He had fractures of five ribs with associated pleural damage, pneumothorax and subcutaneous emphysema. At the time of admission, his blood pressure was 76/30. Two bottles of albumin, consisting of approximately 25 grams, were injected over 30 minutes. The blood pressure after injection was 106/70... his blood pressure remained above 130...he has had no evidence of circulatory failure since the albumin was administered... this patient appeared quite groggy and irrational when I first saw him, but 12 hours later he was very clear mentally and appeared to be feeling better."

Evidence Ba During its first for the plasm dramatic ob tioned. There collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic ob tioned. The collaboration mortality consome regular led to pluming the plasm dramatic o

Another early and dramatic demonstration of the benefits of albumin in trauma was reported on January 5, 1942 by Dr. (later Brig. Gen.) Isidor S. Ravdin, who had just returned from Hawaii and described the administration of albumin to seven very severely

burned patients injured 10 days earlier at Pearl Harbor.

"All seven patients were given albumin, and all showed prompt clinical improvement, including one whose state was so critical that the administration of albumin to him was debatable. There was no question as to his response: He was unconscious in the morning when he was given 250 grams of albumin. In the afternoon, he was talking, but was disoriented. The following morning, he was given the same amount of albumin. Twenty-four hours later, the edema had disappeared and he was taking food by mouth."

It is important to note that these patients, besides being critically ill from their burns, were also severely edematous (swollen) from receiving large amounts of salt solutions in the early part of their treatment, before they were given albumin. This led to problems in accessing their veins to administer the albumin and other intravenous therapies needed to keep them alive. I will come back to this point later.

Evidence Base for Albumin

During its first half century of use, albumin was a stable basis of the plasma protein industry and its benefits, through dramatic observations such as those cited above, were unquestioned. Then, in 1998, a controversial review from the Cochrane collaboration claimed that albumin caused increased patient mortality compared to crystalloid (salt) solutions. This caused some regulatory authorities to issue cautionary statements, and led to plummeting albumin consumption in many countries.

This caused major concerns for the plasma protein industry, but, more importantly, did it affect patient care? Quite soon after the Cochrane review was published, other analyses by eminent investigators suggested that the review's conclusions were challengeable. Then, in 2004, a very well-designed clinical trial performed in Australia compared albumin with salt solution in the treatment of intensive care patients. It found that the Cochrane review had been incorrect in

its conclusions. Mortality in patients given albumin was no higher than in those given saline. This seemed to remove any doubts about the safety of albumin, and consumption rose once again.

In the meantime, new and exciting indications were explored, and albumin was found to be effective in a wide range of indications other than its basic indication of replacing plasma volume in situations of acute loss, such as injury and burns. On the surface, this would seem quite obvious; if albumin is the most common protein in human plasma and clearly provides plasma with its capacity to

maintain blood volume, then what could be more natural and expected that it should have a number of important roles, such as binding and transporting important metabolites and clearing toxic substances?

However, it is the case that albumin, like many medicines that have been established in medical practice for many years, has not been subjected to the same level of investigation that current drugs have to undergo before they are approved for the market. It is difficult to carry out clinical trials in areas of established practice, and such trials are very expensive. With

a relatively established and "unglamorous" drug like albumin, the demands of modern day Evidence-based medicine" (EBM) are very onerous and not always possible to satisfy. This has led to continuing pressure to demonstrate the safety and efficacy of albumin relative to other therapies.

In this regard, the PPTA is concerned that Chapter Five—Albumin—in the recently published "Guidelines for blood components and plasma derivatives" of the eminent German

Medical Association (Bundesärztekammer) throws doubts on the safety and efficacy of albumin, which are unreflective of modern knowledge of this important therapy. In terms of optimal patient care PPTA finds it disturbing that Chapter Five imposes a selective interpretation of EBM, together with its advocacy of other therapies, such as starches, which are associated with increasingly acknowledged adverse events. In future months, PPTA hopes to contribute to the debate on albumin. We hope that the results of this process will enhance general understanding on albumin, which could be under-

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mined by an excessive adherence to principles that are irrelevant to plasma protein therapies. In particular, patient safety, which could be compromised by usage of dangerous alternative therapies, needs to be foremost in our minds. I will address these issues in more detail in future issues of the *Source* magazine.

Professor Albert Farrugia is PPTA's Senior Director, Global Access



PIDD STAKEHOLDERS MEET IN THE NETHERLANDS

By Johan Prévot

's-Hertogenbosch, a historical city and capital of the "Noord-Brabant"

province in the southern region of the Netherlands served as the host city for the XIIIth meeting of the European Society for Immunodeficiencies (ESID), organized jointly with the Xth meeting of the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and the VIIIth meeting of the International Nursing Group for Immunodeficiencies (INGID) on October 16-19, 2008. These groups work to facilitate an exchange of ideas and information between medical professionals and patients with primary immunodeficiency disease (PIDD).

The IPOPI meeting opened with a joint welcome address from several noted speakers including Bianca Pizzera, Chair of IPOPI; Geert-Jan van Moorsel, Chair of the Dutch PIDD organization; Jan M. Bult, PPTA's President; Theo Evers, the International Plasma Fractionation Association's (IPFA) Executive Director; and Steven Baxter and Sven Fandrup of IPOPI. The meeting featured a range of informative and educational presentations. Joy Rosario of IPOPI chaired a session focusing on PIDs in India in which Rubby Chawla talked about the experiences of her association, the Indian Patients' Society for Primary Immunodeficiencies (IPSPI). Germany was then featured in the spotlight in a session chaired by Sven Fandrup featuring a presentation from Andrea Pytlik of the Deutsche Selbsthilfe Angeborene Immundefekte (DSAI). Martine Pergent of IPOPI and Steven Baxter discussed the practical aspects necessary to develop successful national patient groups, while Vicky Modell of the Jeffrey Modell Foundation, David Watters of IPOPI and Sven Fandrup went on to brief delegates on how to best prepare and conduct

fundraising activities. Dr. Teresa Espanol, an IPOPI board member, was in charge of the daily session aimed at summarizing the highlights from the day's ESID conference and also facilitated a question and answer session about phagocyte deficiencies.

After a welcome reception at the end of day one, IPOPI held its General Assembly during which its new Board of Directors was elected as well as its new chairperson. Immediately afterwards, it was announced that José Drabwell was elected as the new Chairperson of IPOPI. Mrs. Drabwell, who previously served as Vice-Chair and Treasurer of the organization, succeeds Bianca Pizzera.

On day two of the meeting, delegates were treated to several first-class sessions, including presentations by Prof. Helen Chapel and Prof. Jordan Orange on proposals on guidelines for the appropriate use of immunoglobulins in the United Kingdom and United States, by Dr. Teresa Espanol on vaccination problems for PIDD patients as well as by Prof. Albert Farrugia, Senior Director, Global Access, PPTA, on how to work with national regulators and health authorities.

Marcia Boyle of the Immune Deficiency Foundation (IDF) and Fred Modell of the Jeffrey Modell Foundation (JMF) were the first speakers to take the stage on day three with presentations on the importance of collection and the dissemination of national and international statistics on PIDDs. Mrs. Boyle updated participants on the outcomes of the various successful surveys undertaken by IDF in the United States and the way IDF uses them in their lobbying activities. Mr. Modell talked about the experience of the JMF in conducting surveys and producing strong statistical reports on various topics such as the impact of awareness and education programs on the diagnosis of patients and the economic impact of undiagnosed patients with primary immunodeficiencies. Roberta Pena, of the Asociación Civil de Ayuda al Paciente con Inmunodeficiencia Primaria (AAPIDP), provided delegates with an overview of the benefits of a collaboration between national patient organizations and IPOPI. Other sessions included "How can national member organizations work together on a regular basis," during which the Swedish and the Danish patient

organizations talked about the advantages of the "twinning" of their respective organizations as well as on accessing information for PIDD patients. In addition, a meeting was held between the IPOPI Board of Directors and industry representatives, during which IPOPI reviewed their most recent achievements including the reinstatement of immunoglobulin on the World Health Organisation (WHO) List of Essential Medicines, the European Union PID Consensus Conference and the recently published Pharmaceuticals Policy and Law publication on Immunodeficiencies.

The closing day of the IPOPI meeting mainly featured highlights of the many

outstanding sessions held during the main ESID conference. The ESID meeting kicked off with an Educational Day during which leading experts provided presentations on recent clinical developments in the field of primary immunodeficiencies. One of the presentations was given by Prof. Alain Fischer from France on "trends in primary immunodeficiencies." In his presentation, Prof. Fischer highlighted the importance of PIDDs, which should be part of every medical training as they have become over the past 40 years a new chapter of medicine, teaching us how our immune system deals with infectious agents, how it avoids reactivity to self and

how it may fight cancer. The remainder of the ESID conference program boasted a range of state-of-the-art clinical sessions on topics such as T-cell development, migration and regulation of the immune response, stem cell regeneration, new



Above: Jose Drabwell, left, new Chair of IPOPI and Bianca Pizzera, right, Outgoing Chair. Below, from left to right: A few members of the IPOPI team: Sven Fandrup, Martine Pergent, Joy Rosario, Marcia Boyle, Jose Drabwell; Dr Teresa Espanol, Roberta A. de Pena and Stefen Baxter.



insights in B-cell developments, longterm management in PIDD, extended frontiers in immunodeficiencies, as well as various workshops on gene therapy, diagnosis of PIDD, the ESID registry and a series of satellite symposia.

The next ESID, IPOPI and INGID joint meeting will take place on October 7-10, 2010 in Istanbul, Turkey. ●

JOHAN PREVOT is PPTA's Assistant Director of Public Affairs, Europe



BARACK OBAMA'S PRESIDENTIAL WIN Denotes Big Change for the U.S.

BY JULIE A. BIRKOFER

ovember 4 marked an impressive victory for
Barack Obama, whose campaign focused on "change."
While he begins the process of settling into the White House, members of Congress on Capitol Hill are anticipating how those changes will be translated into legislation. The first order of business of the newly elected administration and the 111th Congress will likely be to shore-up the floundering economy.

Health care is a sweeping topic that includes universal healthcare, covering the 40 million-plus uninsured and creating savings in Medicare, which is one of the budget busting "entitlement programs" we heard the candidates discussing. President-elect Obama will begin to prioritize his legislative agenda for the 111th Congress in order to begin effecting the "change" on which he campaigned.

For plasma protein therapies, the view narrows and becomes much more specific. For example, two issues, follow-on biologics and comparative effectiveness, may be issues that the 111th Congress would consider; likely not until their second session, beginning in 2010. The chances of these issues coming to closure next year are slim at best. Let's take a look at follow-on biologics and comparative effectiveness and seek to understand the issue and potential impact on this industry.

The success of the Obama administration's agenda will require the President-elect

to work with the new Congress, which should prove to be relatively easy after the Democrats secured solid gains in both the U.S. House of Representatives and the U.S. Senate. Moderate and conservative Democrats, especially those that are members of the Blue Dog Coalition, a fiscally conservative Democratic group, in the House, as well as procedural tactics in the Senate, could play a vital role to ensuring that President-elect Obama's legislative agenda is carried out in a fiscally responsible manner and includes reasonable policies that will have bipartisan support.

Also impacting President-elect's success will be his selection of Secretary of Health and Human Services (HHS). Top contenders include: former Senate Majority Leader Tom Daschle (D-SD), along with former Oregon Governor Dr. John Kitzhaber, and Dr. Howard Dean, the former governor of Vermont, who recently served as Chairman of the Democratic National Committee, as individuals who are under consideration for the position

of Secretary of the Department of Health and Human Services.

Mending the economy, which became the most critical issue in the closing weeks of the election, will likely be the first order of business for the new administration. Health care costs are an enormous part of the economic concerns for many Americans. President-elect Obama is committed to ensuring every American, especially every child, has affordable, quality health care coverage. Health care decisions will become increasingly data driven. The use of comparative clinical effectiveness data to reduce health care costs, as well as the creation of an abbreviated Food and Drug Administration (FDA) approval pathway for follow-on biologics, are both possibilities. Both comparative effectiveness and follow-on biologics could have a significant impact on patient access to plasma protein therapies.

Follow-on biologics remains unresolved as the 110th Congress winds to a

The success of the Obama administration's agenda will require the President-elect to work with the new Congress, which should prove to be relatively easy after the Democrats secured solid gains in both the U.S. House of Representatives and the U.S. Senate.

close despite the introduction of multiple bills in both the House and Senate. For example, the United States House of Representatives Committee on Energy and Commerce had before it three pieces of legislation that committee members had introduced, but took no action on any of them because of the schism between committee members on many key provisions.

While PPTA has been able to generally support pieces of legislation on followon biologics that acknowledge the unique nature of plasma protein therapies, the Association has several concerns, such as the effects on patient access to care and inclusion of language that would require the FDA to make a determination of interchangeability at the time of

approval of the follow-on

biological between that product and the innovator product. Because brands of immune globulin, blood clotting factors, and alpha-1 antitrypsin are not interchangeable, such a mandate could lead to access difficulties for consumers that require a specific therapy as part of their treatment plan should the FDA take such action. Indeed, PPTA will continue to inform congressional staff that such a determination would be impossible for plasma protein therapies.

Another health policy issue that could potentially adversely affect access to plasma protein therapies is comparative clinical effectiveness. Health policy experts contend that if Congress establishes an entity to conduct comparative clinical effectiveness research; patients, providers, and payers will finally be afforded the opportunity to truly make evidence-based treatment decisions for diseases and

conditions. Proponents envision a public-private entity insulated from the political pressures

of an Administration and congressional interference that would provide analysis evaluating the relative value of drugs, devices, diagnostic and surgical procedures, diagnostic tests, and medical services. Although well intended, the establishment of such an entity could lead to a situation in which the data it produces could ultimately be used for restrictive coverage and reimbursement decisions.

Never before has the Association been in such a position of strength. That strength is drawn from many things, such as working with sophisticated consumer groups and advocating for patient access to plasma protein therapies. Our messages regarding the unique nature of plasma protein therapies and the imperative of specialized reimbursement for these fragile populations have never been stronger. Working together with consumer advocates and the talented staff of our member companies, PPTA is ready for the challenges and opportunities that the 111th Congress and new administration will present to our industry.

For more in depth coverage of the elections, please visit the full article posted in the Issues/U.S./Federal section of the PPTA website at www.pptaglobal.org.

Julie A. Birkofer is the PPTA Vice President, North America

REPORT ON THE

2008 SOURCE BUSINESS FORUM



By Kara Flynn

ith temperatures dropping to an unseasonably cool 40 degrees Fahrenheit outside, attendees of the 2008 PPTA Source Business Forum were more than inclined to give their full attention to informative PPTA presentations and updates and an expert panel on European Medicines Agency (EMEA) epidemiology guidelines. The event took place in conjunction with the AABB Expo, in Montreal, Canada, on Sunday, October 5, 2008.

The event opened with welcoming remarks from CSL Behring's Gordon Naylor, Chair of the PPTA Source Board of Directors. Mr. Naylor discussed the robust nature of the source plasma industry, with collections of plasma up significantly in 2008. In her report, Ms. Ileana Carlisle, Biotest Pharmaceuticals Corporation and PPTA Source Board Treasurer, noted that there will be no charges to the dues or program fee structure for 2008. In addition, PPTA Source is operating on its targeted budget, and she gave a recap of the anticipated projects of 2009. John McVey, Baxter Healthcare and Chair of the Source Regulatory Policy Steering Committee, provided an update to members on regulatory issues. Among the areas of interest for Source members includes differentiating plasma for manufacturing use from blood components for transfusion; the uniform donor history questionnaire; the hepatitis C (HCV) donor seroconversion study; and examining alternative methods for the measurement of total protein and hematocrit/hemoglobin (Hgb/Hct).

Sybille Beck, Manager of PPTA Source Europe and Communications, provided an update on European Source activities. In her presentation, Ms. Beck discussed European source plasma collection; a recent SIPLA study of the safety of long-term intensive plasmapheresis in donors in Germany; a proposal on unique collection center identification; and recent expansion in the European Union. Kara Flynn, PPTA's Director, Global Communications, discussed the Association's Source Industry Image and Credibility Campaign. This project, integrated with global communications projects, is aimed at building awareness of source plasma donation and its therapeutic uses; fostering positive media outcomes; methods of improving local community relations; and consumer group outreach. Ms. Flynn discussed a number of the projects that have been completed during the Campaign's initiation —including the recent launch of www. DonatingPlasma.org, a website designed to educate potential and current source plasma donors (see page 9.)

Following Ms. Flynn, the Business Forum panel discussants took to the stage to provide information on a topic that has been of great interest to the Source industry—epidemiology guidelines. This panel session provided an analysis of current issues regarding implemen-

tation and implications of the epidemiology policies, the industry approaches and further considerations. To assist PPTA members in addressing some of the issues they might face, PPTA brought together experts from within the plasma industry to discuss these issues during the Source Business Forum. The panel consisted of four members: Mr. Roger Brinser—Biolife Plasma Services, Mr. Sam Lovick—CSL Limited, Mr. Tommaso Paoli—Kedrion Biopharmaceuticals and Mr. Gote Carlebjork—Octapharma AB.

With the EMEA policy of submission of new epidemiological data to obtain information on the infection risk in donor populations, PPTA's Plasma Master File Task Force has been working to elaborate on an appropriate reporting structure for viral marker rates for all plasma fractionators to minimize the risk of different interpretations. Panelists during "Current Perspectives on the EMEA Epidemiology Guideline," discussed two simultaneous approaches for consideration: a risk-based approach based on end product evaluation and an approach based on donor population epidemiology.

The PPTA Source Business Forum was exceptionally well attended this year. Many members also provided valuable feedback and contributed a number of noteworthy ideas for discussion next year. The PPTA staff thanks the membership for their continued support and looks forward to seeing everyone at next year's Source Business Forum in New Orleans, Louisiana on October 25, 2009.

KARA FLYNN is PPTA's director of Global Communications



GERMAN DONOR DEDICATES 30 YEARS and 1,150 Donations to Saving

n November 2007, German marathoner Heinz Behrmann celebrated his 1,150th plasma donation at the ZLB Plasma Center in Kiel and reached yet another remarkable milestone this year—30 years of donating. Mr. Behrmann shared his extraordinary story with us for this edition of The Source.

I first learned of plasma donation in 1978, when I was 25, from family friend Christa Johannsen, who worked as a nurse at the plasma center in Bremen. My wife and Christa knew each other from the many years they spent together working together as nurses at the University Children's Clinic also located in Bremen.

The first time I donated, I was so nervous that I did not feel really well during the donation—and spent some time with my legs up in the air. Nonetheless, I went back and things went well.

The initial incentive to donate was the fee, which I believe was 20 DM (10 euro) at the time. Then again, I was still a young man back in the day. Increasingly over time, the health and fitness aspects of donating came into play when I started training for long-distance running in 1986 at age 33. Blood pressure and pulse readings taken at the plasma center, along with blood tests to monitor my levels of iron and red blood cells,

served as key indicators, enabling me to get myself into shape and to swiftly recognize any signs of overtraining. My training goal was to run a marathon, and I achieved my personal best in November 1991 with a time of two hours 50 minutes when I was 41. That was my 187th marathon.

Over the years, people often asked me how much compensation I received with nearly 30 years of dedication as a donor, and what I have done with all of the money. If only I would have saved that up, right? The truth of it is I never saved a penny of it; I spent it all as part of regular expenses. On one occasion, however, I remember we did end up saving up the fees and treating ourselves to a lovely weekend trip to Berlin to mark my 500th donation celebration. On that special trip, I ran the Berlin marathon and, for the first time, I ran under the Brandenburg Gate.



and Improving Lives

During my first few years as a regular donor, a lot of friends and colleagues asked whether donation was healthy, suggesting that in the long run it would surely produce side effects that would be detrimental to my health. These days, when I tell people that neither the doctors nor I have observed or experienced any side effects of any description, the subject is quickly dropped. I even took two breaks during my donation career, to allow the center to verify that I remained in good health and free from any risk behaviors that would make my plasma unsafe for use in producing therapies.

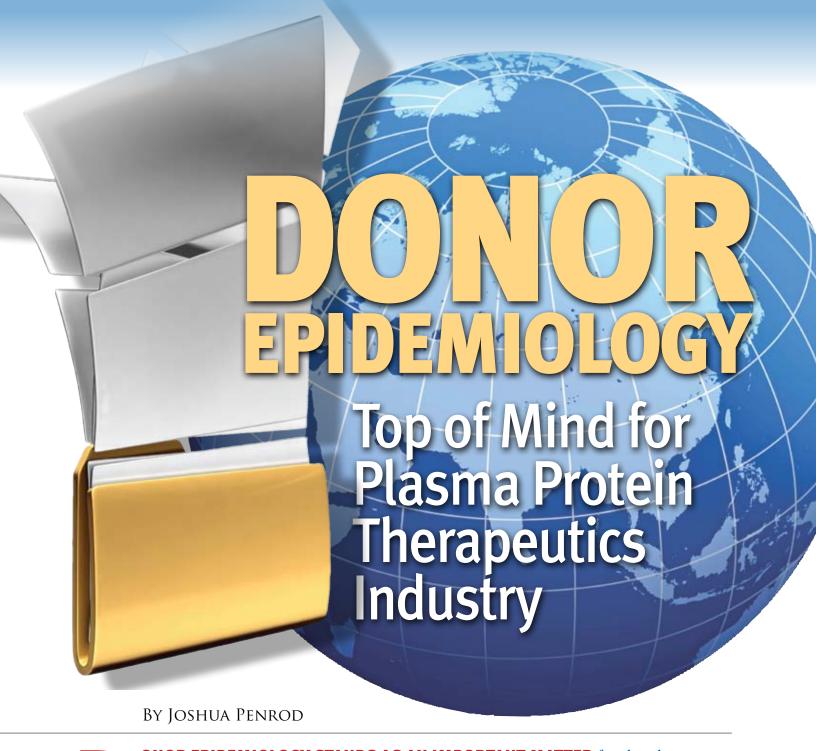
For many years now, I have kept plasma donation flyers on my desk and I have a great deal of contact with members of the public. People often comment on the certificate I was awarded in 2003 to recognize my 1,000th donation, which is framed and hanging up in my office.

On the occasion of special celebrations, which have recently included my 1,000th and 1,111th donations, I too have taken the opportunity to say thank you by leaving a large glass jar filled with toffees in the waiting room for visiting donors.

EDITOR'S NOTE: The Source thanks ZLB Plasma in Kiel.



Heinz Behrmann and center manager Gesine Lamp, who was two years old when Mr. Behrmann started donating plasma.



onor EPIDEMIOLOGY STANDS AS AN IMPORTANT MATTER for the plasma collection and fractionation industry. It has been brought back to the forefront by the institution of the European Medicines Agency (EMEA) Epidemiology Guideline EMEA/CPMP/BWP/125/04 and ongoing industry efforts to address this complex, nuanced and global issue. In recent months, the PPTA Epidemiology Task Force (EPITF) has discussed and analyzed several different approaches, which can provide a solution and a system that can accommodate the many diverse issues in donor epidemiology and the policies regarding it. In addition, the 2008 Business Forum in Montreal featured a panel discussion of epidemiology by industry experts, which served as a vehicle for the membership's better understanding of current undertakings. This article will summarize the current work of the EPITF, the Business Forum discussion and briefly outline some general next steps to be taken.

EPITF Activities

The PPTA Viral Marker Standard (VMS) has been in place since 1999. This Standard, an integral component of the International Quality Plasma Program (IQPP), uses plasma center collections and average industry rates for HIV, Hepatitis C, and Hepatitis B to create limits within the IQPP certification program for centers to use as a guide for determining epidemiology within the center. One of the more important features of the VMS is its use of donations as a denominator for the calculated rates, along with the distinction of applicant and qualified donations. The limits themselves are controlled by the industry average and are adjustable for population trends.

The VMS, therefore, is a form of statistical and quality control over some of the steps in the complicated plasma manufacturing process. It has been used with great success for nearly a decade and has helped the industry to ensure collection from a low-risk donor population. It has also helped instill stakeholder confidence in the industry's processes and high-quality products.

In contrast, the EMEA Epidemiology Guideline uses a different system for calculation of viral marker rates. Its measures of prevalence and incidence are based on the number of donors, rather than donations as under the PPTA VMS system. Similarly, instead of applicant and qualified categories, the Guideline uses First-Time and Repeat donors. With these definitions, a Repeat donor is a donor that has previously donated at any time, in contrast to the qualified category in the IQPP, which has a six-month period in which a donor remains qualified.

In 2006 PPTA created a new template and system for data collection by the member companies beginning in 2007. The new web-based system allowed for simultaneous collection of data based both on the IQPP and EMEA definitions and comparisons of rates. At this point, with only a single year's worth of data to review, no trending or tracking has been undertaken, although with the completion of the 2008 data set, more insight will be attained.

With all of the data category requirements, the EMEA Guideline requires that the holder of a plasma master file (PMF) be able to report on and interpret a number of different elements—for both source and recovered plasma. First, the PMF holder is responsible for observing and analyzing individual plasma center epidemiology; second, the PMF holder is expected to facilitate tracking and trending of these data. Lastly, the PMF holder is to establish a range of limits for acceptability of epidemiological data. This final point has been the area of the most recent discussions for the industry.

The establishment of an industry-wide system for use in reporting and interpreting epidemiology data has therefore occupied the interest of many of the industry's leading experts. The discussions and consensus-building approach has been complex, and its importance is magnified due to the many different technical challenges, regulatory scrutiny, and policy implications. The two main lines of thinking include the residual risk (as used currently in the VMS) and end-product (currently in development, and which captures the complete manufacturing and testing paradigm used by the industry) approaches. The

EPITF and others are currently examining and these approaches in an effort to create an updated system that addresses all of the industry's and regulatory authorities' concerns.

Business Forum

Because of the member companies' interests in the epidemiology issues, and the ongoing dialogues with regulatory authorities and within the industry, the PPTA Source Business Forum held a panel discussion focusing on epidemiology (see article on the Business Forum on page 19). Specifically, the panel addressed the EMEA Guideline, the EPITE's efforts, and the Guideline's impact in the EU and the U.S. The four distinguished panelists noted some of the more pressing concerns entwined in the debate:

- The importance of having global epidemiological limits established to ensure collection from low-risk donor populatons to avoid different interpretations by national regulatory authorities on the meaning and impact of epidemiology.
- The inclusion of a holistic approach and a context that recognizes safety and quality of plasma and finished product at each and every step in the chain.
- To ensure that any industry-wide system will be consistent with the policy concerns and requirements of various regulatory authorities.

Outcomes and Next Steps

The EPITF will be preparing an industry position for consideration. The EPITF outcomes will be the basis for the future decision-making and industry policies, such as the VMS, and the industry position for purposes of interacting with regulatory authorities. Several of the most important industry touchstones for incorporation in the future direction of the management of the epidemiology issue, both from a regulatory and industry operations perspective are:

- Industry's vigilance and quality and safety of plasma and finished products.
- The need for healthy, committed donors.
- The ability for the industry to maintain a global, holistic system that incorporates the industry's best thinking and best efforts.
- The proper approach and methods taken in—and feasibility of—constructing a singular industry solution.
- The importance for the industry in having an approach that equitably treats all starting materials.

The EPITF has not yet reached a conclusion for its recommendation. A preliminary meeting with regulatory authorities is currently being planned to exchange ideas and current thinking regarding the industry position and regulatory input. Over the next several months, the EPITF and other related industry working groups will move toward a consensus establishing the industry's position with regard to the complex epidemiology issue.

JOSHUA PENROD is PPTA's Vice President, Source.



3&4 MARCH 2009 - PARIS, FRANCE INTERNATIONAL PLASMA PROTEIN CONGRESS

WHO SHOULD ATTEND

- collectors of plasma
- patient communities
- pharmaceutical regulators
- physicians

- health ministries
- legislators
- industry professionals: business developments, marketing, medical, production, public policy, purchasing, quality, regulatory affairs, reimbursement, research and development, sales

WHY ATTEND

The Congress builds in the success of the 2008 event and offers:

- the opportunity to learn about and discuss new and key developments affecting your field
- presentations from industry experts and professonals to expand your knowledge of the latest plasma protein industry issues
- networking with all stakeholders to improve your contacts

SUPPORTING ORGANIZATIONS













CONFIRMED SPEAKERS

Larry Guiheen, PPTA Global Chairman Alain Fischer, AP-HP Necker Hospital, Paris Sam Lovick, CSL Behring Graham Sher, Canadian Blood Service

Rainer Seitz, Paul-Ehrlich-Institut

Martin Terberger, European Commission David Watters, IPOPI

Ségolène Aymé, Orphanet - ECTF on Rare Diseases Timothy Evans, The Royal Brompton Hospital, London

Fred Modell, Jeffrey Modell Foundation

Chris Ludlam, The European Association for Haemophilia and Allied Disorders

Brian O'Mahony, Irish Haemophilia Society Jürgen Wallner, University Vienna

Patrick Robert, The Marketing Research Bureau

Peter Hellstern, University Hospital Ludwigshafen

Kirsten Seidel, ZLB Plasma Services

Jürgen Wohlfahrt-Laymann, Plasma Service Europe GmbH

Marcell Heim, University Hospital Magdeburg

Larry Warren, Alpha One

Mark Skinner, World Federation of Hemophilia

Jose Drabwell, IPOPI

Jan Bult, PPTA President

SUPPORTING MEDIA



SPONSORS





AGENDA

Tuesday 3 March 2009

Session 1: Keynote Session

- State of the Industry
- Role of the reference centers in the treatment of PID
- The Precautionary Principle and Plasma Proteins: A New Approach
- Canadian thoughts on self-sufficiency

Session 2: Regulatory Affairs - what's up in Europe?

- Paediatric Regulatory Framework in the EU: Relevance to plasma protein therapies
- Revision of 269/95 what has changed, TBC
- New Variations Regulations
- Counterfeit medicines Think locally, act globally, TBC

Session 3: Rare diseases - a European Health Priority?

- European Commission actions in the field of rare diseases, European Commission
- The Rare Diseases Community Priorities, Eurordis
- Rare Diseases: The European Parliament's viewpoint, European Parliament
- Plasma related patient organizations
- Overview of Member States Public Health Policies for Rare Diseases

Session 4: Developments in the clinical use of plasma proteins

- New perspectives on the therapeutic use of albumin
- Underdiagnosis: the real cost of PID
- The role of EAHAD by C. Ludlam, The European Association for Haemophilia and Allied Disorders
- Neurological use of Ig review, TBC

Hilfenhaus Award Cocktail Reception

Wednesday 4 March 2009

Session 5: Meeting clinical needs - various perspectives on how much plasma do we need

- Patient perspective
- Immunoglobulin: the "driving" product for plasma demand, TBC
- European Commission's perspective, TBC
- Ethicist's perspective
- Plasma demand in 2015

Session 6: Getting the plasma we need

- Regulatory environment of plasma collection in Germany
- Demographic developments, Robert-Koch-Institut
- Sparing the donor's red cells during frequent plasmapheresis
- Opening new plasma centers
- TBA

Session 7: Optimizing access to plasma protein therapies

- Challenges facing Alpha One Trypsin patients
- Optimizing access to coagulation factors: patient perspective
- Immunoglobulin: the importance of range and choice of products, TBC
- Optimizing access to PID treatment: patient perspective
- The implication of a level playing field

ORGANISED BY



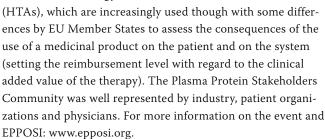
NEWS

PLASMA PROTEIN NEWS FROM AROUND THE GLOBE

EUROPE

> PPTA staff attended the Workshop on Partnering for Rare Diseases Therapy Development organized by the European Platform for Patients' Organizations, Science and Indus-

try (EPPOSI) at the French National Assembly in Paris. The two-day event was organized in the context of the official program of France's Presidency of the European Union. The meeting program included national sessions as well as European sessions on topics ranging from the importance of early diagnosis and patient registries to access to care and reimbursement policies. The meeting also featured a discussion on Health Technology Assessments



> PPTA staff and the Chairman of the **EU Health Policy Steering Committee** met with representatives of the Substances of Human Origin (SOHO) team of the European Commission's Directorate General for Health **and Consumers** in Brussels, Belgium. The goal of the meeting was to discuss the need to differentiate between plasma and blood (from collection practices to end product) in two upcoming European Commission dossiers: the first one will be produced as a result of the recently published Call for Tender on Testing Methods and Testing Laboratories by the end of 2009 and the second one will be the next Commission Report on the "Promotion of Voluntary Blood and Tissue and Cells Donations" due to be released in June 2010. Overall the outcomes of this productive meeting were positive: PPTA will be consulted during the preparation of both reports and the Commission agreed to feature separate sections on plasma collection and products in both reports.

▶ The European Commission published a proposal on crossborder healthcare. The stated overall objective of the proposal is, "to ensure that there is a clear framework for cross-border

healthcare within the EU, in order to provide sufficient clarity about rights to be reimbursed for healthcare provided in other Member States; for those rights to be realized in practice; and to ensure that the necessary requirements for high-quality, safe and efficient healthcare are also ensured for cross-border care."



Place de la Concorde and The French National Assembly in Paris

Responding to a Parliamentary Question from MEP Thomas

Ulmer (EPP-ED/Germany) on the need to differentiate between blood and plasma (products and collection practices), the European Commission published its answer. The Commission acknowledged the importance of patient access to treatments produced from blood components and that therefore different systems for blood and blood components collection may co-exist in the EU. The Commission further indicated that it, "will pay due regard to the different blood components collection procedures when considering the interpretation and implementation of the principle of voluntary unpaid blood donations in the European context."

USA

The annual
PPTA/U.S. Food and
Drug Administration (FDA) Liaison
meeting was held on
September 26 and



was well-attended by both organizations. Topics of interest to both parties were discussed and included: current priorities of FDA and PPTA; clarification of PPTA comments to the Proposed Rule: Requirements for Human Blood and Blood, which had been submitted to FDA on August 4, 2008; FDA performance on pre-license inspections; FDA implementa-

tion of FDAAA, specifically progress on identification of the Standards for the Standard Numerical Identifier; and FDA's recent data request for viral marker data. FDA acknowledged that it was thankful for data submitted by PPTA and member companies and has concluded that there is no "uptick" in seroconversions in qualified donors.

▶ Senate Finance Committee leaders announced they had reached agreement with the Democratic and Republican leadership on a comprehensive energy and tax package that includes funding for mental health parity legislation (H.R. 1424, S. 558). House and

Senate negotiators reached agreement on policy provisions for compromise mental health parity legislation last June, but have not been able to find ways to fund the legislation. The House bill includes a Medicaid rebate increase from 15.1 percent to 20.1 percent to help offset the mental health parity

tors have indicated that a Medicaid rebate increase is undesirable and not likely to be used to pay for the legislation. PPTA continues to reach out to Senate leaders in opposing a Medicaid rebate increase to pay for the mental health parity legislation.

legislation; however, Senate negotia-

▶ PPTA staff attended the **Western Medicaid Pharmacy Administrators Annual Conference** in San Antonio, Texas. The "cost of clotting factor" and state-based initiatives to contain costs, while ensuring patient access, was

a focal point. Staff was able to meet with and inform key decision-makers about the unique nature of the plasma protein therapeutics industry.

▶ In late October, the **Center for Medicare and Medicaid Services** (CMS) released both the outpatient prospective payment system (OPPS) and physician fee schedule (PFS) Final Rules. Unfortunately, CMS rejected PPTA and other stakeholder arguments for maintaining the IVIG preadministration code for 2009 in both the PFS and the OPPS. Even more significantly, CMS rejected the APC Advisory Panel's recom-

mendation to continue to pay for separately

payable drugs and biologicals at ASP +5 percent in 2009 as well as PPTA and other stakeholder arguments to restore this payment level to ASP +6 percent, and will instead reimburse these products at ASP +4 percent in 2009 as it proposed.

> FDA has released a draft "Guidance for Industry: Nucleic Acid Testing (NAT) to Reduce the Possible Risk of Parvovirus B19

Transmission by Plasma-Derived Products." This draft guidance accepts Parvo NAT testing as an in-process test, but encourages testing procedures that will "allow for meaningful notification of blood and plasma collection establishments of positive test results within the dating period of components." The specific recommendations include a limit of 104 IU/mL in manufacturing pools and a sensitivity of 106 IU/mL for individual units. The Federal Register notice can be found at the following link: http://edocket.access.gpo.gov/2008/pdf/E8-17431.pdf and the Draft Guidance at: http://www.fda.gov/cber/gdlns/natparo.pdf



MEET THE PPTA



Brenda Norman and Mary Straub

Brenda Norman is the Data Manager for three very different PPTA programs, Viral Marker Data, Supply Data and the Patient Notification System.

As the manager for Viral Marker Data, Brenda ensures that all companies with centers that have applied or are IQPP-certified report their data on a monthly basis. In addition, Brenda works with Georgetown Economic Services, LLC to gather industry supply data from PPTA members and participating companies.

by Kara Flynn

The data is aggregated before it is supplied to the U.S. Food and Drug Administration (FDA) for their records and posted to PPTA's website with the most current numbers when they are available. The Patient Notification System (PNS) is operated by Stericycle, Inc., and Brenda is the administrator of the system. PNS is free to consumers, strictly confidential and registrants can choose e-mail, telephone or fax to be notified within 24 hours if there is a recall or withdrawal of plasmaderived or recombinant analog therapies. Brenda recently celebrated 10 years with PPTA.

Mary Staub is PPTA's Manager of the International Quality Plasma Program (IQPP). She will celebrate 18 years with the Association in

Brenda Norman
and Mary Straub
are two PPTA
staff members
who are little seen
by members but
who have critical
Association
functions.

January. Over the years, her industry knowledge and responsibilities have grown. Mary manages the administration of the IQPP, which includes oversight of the independent auditors, setting audit schedules, tracking certification fees and issuing the IQPP certifications. In addition, she reviews all IQPP audit findings, sends observation letters to industry contacts for IQPP and reviews all corrective action responses. Mary also serves as a liaison between the PPTA Standards and Programs Manager

and auditors and facilitates training for them as required under IQPP. In their roles at PPTA, Brenda and Mary are important team

members whose positions focus on the quality and safety of plasma protein therapeutics—our industry's foremost concern.

Tell us about your background.

Brenda Norman: I've had the opportunity to live in Oklahoma, New York, California, Virginia, and currently Annapolis, Maryland. In my junior year of high school, my family moved to Virginia where I went to Robinson Senior High in Fairfax. I have been married for 24 years, and have two great kids. My son Christopher is in his junior year at Towson University studying Kinesiology to become a physical therapist. My daughter Samantha is in her junior year at Broadneck High School and will also study Kinesiology when she goes to college. I have a passion for fitness and living life to the fullest. I've run six marathons (26.2 miles), five fifty-milers, two 50K

races, a handful of triathlons, and lots of smaller races. In addition to that, I have coached both my kids in soccer, t-ball and assisted in cheerleading. I also work part-time at Curves for Women as a fitness coach. My husband Scott is the quiet one who enjoys boating, fishing, crabbing and snow skiing.

Mary Straub: I am one of the few Washington, D.C. natives who was born in the city and lived in this area all my life. My previous work experience was with the federal government where I started at the Department of Justice straight out of school, and then went on to work for Federal Energy Regulatory Commission and NASA at the Goddard Space Flight Center. I then took a five year hiatus to be at home with my children. My husband, David, and I have been married for 33 years and have three children—two sons, Daniel and Michael, and a daughter, Christina. We also became proud grandparents to a perfectly adorable little boy, Denver, this past June.

What is your proudest professional achievement at PPTA?

BN: The highlight of my career at PPTA has been the opportunity to inform the Board directly in their meetings, as I feel this is a testament to my work.

MS: I would have to say that my proudest professional achievement would be the productive working relationships and respect I have established with my colleagues at PPTA, the IQPP auditors as well as our member representatives I have contact with throughout the industry.

What is most rewarding about working in this industry?

BN: My colleagues and the members I have worked with over the years are all incredibly talented individuals. Regardless of our job titles, the staff at PPTA's ultimate goal is to help manufacturers produce the safest therapies available. When I see how these therapies improve lives, I can honestly say I get a lump in my throat knowing I am a small part of keeping the therapies safe and helping them have a better quality of life.

MS: It makes me feel good to be a part of a team that is doing important work and continually strives to make progress and improvements in the plasma collection arena to bring life-saving therapies to patients who depend on them throughout the world.

KARA FLYNN is PPTA's Director of Global Communications.

EVELTS UPCOMING CONFERENCES & SYMPOSIUMS

2009

February 26–27	2nd Annual EAHAD Congress European Association for Haemophilia and Allied Disorders
	Munich, Germany
March 3–4	International Plasma Protein Congress Paris, France
March 23–25	21st Annual EuroMeeting of the Drug Information Association Berlin, Germany
May 18-22	VIII Latin American Meeting in Hematology, Immunology and Transfusion Medicine <i>Havana, Cuba</i>
May 28–30	57th Annual Congress of the Japan Society of Transfusion Medicine and Cell Therapy <i>Saitama, Japan</i>
June 2–3	Plasma Protein Forum Washington, D.C., USA
July 11–17	XXII ISTH Congress Boston, MA, U.S.A.
September 15–18	42nd Annual Meeting of the German Society for Transfusion Medicine and Immunehaematology (DGTI) Rostock, Germany
September 24–25	Sixth Global Forum on the Safety and Supply of Treatment Products for Bleeding Disorders <i>Montreal, Canada</i>
October 24-27	AABB Annual Meeting New Orleans, USA
October 25	Source Business Forum New Orleans, USA PPTA members only

2010

June 15–16	Plasma Protein Forum Reston, VA, USA
June 26–July 1	XXXIst International Congress of the ISBT Berlin, Germany
October 7–10	XIVth Meeting of the European Society for Immunodeficiencies Istanbul, Turkey
October 9–12	AABB Annual Meeting Orlando, USA
October 10	Source Business Forum Orlando, USA PPTA members only

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