

# THE SOURCE

THE MAGAZINE FOR THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SUMMER 2011



## Keeping Health Care Legislation on Track

In both Europe and  
the United States

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**HTAs: What Do They Mean for  
Plasma Protein Therapies?**

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**Hurdles of Implementing  
Healthcare Reform  
at the State Level**

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**European Court of Justice  
Weighs in on  
Donor Compensation Debate**

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# IN MY VIEW

## DR. OTTO SCHWARZ: AN INFLUENTIAL INDUSTRY LEADER

### IN THIS COLUMN I WOULD LIKE TO PROFILE

Dr. Otto Schwarz, a man who was a pivotal influence during the early days of our industry. He is someone whom I admire and learned from. He and Dr. Hans Eibl were the owners of Immuno AG, a leading European manufacturer with headquarters in Vienna, Austria. In 1992, Dr. Schwarz was also one of the founders of the International Plasma Products Industry Association (IPPIA), the industry association representing the manufacturers of plasma protein therapies. This organization was the forerunner of PPTA.

I had the opportunity to meet with Dr. Schwarz recently in Vienna. He is a gentleman in his eighties with enormous wisdom and experience. He is definitely one of the persons who helped shape the industry into what it is today. It is true to say that most of the current standards that are in PPTA's Quality Standards of Excellence, Assurance and Leadership (QSEAL) program originated with his determination to improve the quality and safety of the source plasma collections.

He was the first to recognize the importance of qualified donors and how to best apply nucleic acid amplification (NAT) technology. This new

technology significantly improved the ability to detect viral infections earlier than serology testing. The introduction of these steps was an early demonstration of the industry's commitment to safety.

In 1997, his company was acquired by Baxter, then under the leadership of John Bacich. The lengthy negotiations were successful and both parties came to an agreement in Zurich, Switzerland. It was a coincidence that I happened to pass by the hotel where the final negotiations were completed and saw a tired John Bacich leaving at 7:00 in the morning. After the acquisition, Dr. Schwarz stayed involved in the new company for another five years to provide the incoming leadership with his expertise.

Dr. Schwarz has always been an avid art collector and a patron of young artists. Lately, he has stopped collecting since "there are no more places in my house to hang paintings."

I asked Dr. Schwarz what his message would be to the industry today. He reminded me of the importance of the industry demonstrating leadership through an industry-wide mindset. Twenty years ago when the Association started, companies were not used to working together on issues of common interest. He showed the other companies that sometimes making a compromise will lead to a better outcome. As one of the first Chairpersons of IPPIA, he showed that first-hand.

During my visit to Vienna I met several knowledgeable people who have worked personally with Dr. Schwarz. Without exception they told me that Dr. Schwarz and Dr. Eibl put Vienna on the map of the plasma protein industry. Their significant role in building a reputable company is well recognized.

Dr. Otto Schwarz is a man rich in experience, who has led an influential life. I thank Otto for his many contributions and leadership to the industry over the decades.



PPTA's President and CEO Jan M. Bult with Dr. Otto Schwarz



# ALBUMIN:

## ONGOING DEVELOPMENTS

BY ALBERT FARRUGIA

### ALBUMIN IS, HISTORICALLY, AN ESTABLISHED THERAPY

in treating patients with a variety of severe illnesses. Many of these can be grouped as diseases of hypovolemia, in which the loss of fluid from the circulation leads to severe compromise of the blood's ability to deliver nutrients and oxygen to the tissues and organs. This leads to organ failure and possible death.

The most common reason for the loss of fluid from the circulation with resultant hypovolemia is massive injury leading to blood loss. A similar clinical circumstance occurs when burns lead to loss of the skin and allow large volumes of fluid to escape from the tissues and blood vessels. The use of albumin in these circumstances was established in World War II (see A. Farrugia, *The Source*, Winter 2009). More recently, many other conditions have been recognized as leading to hypovolemia, with all its harmful effects. Such a condition is sepsis, in which infection across the vascular supply (the blood vessels) leads to damage of the blood vessel wall and leakage of fluid. And as in loss through injury or burns, fluid loss in sepsis is a large cause of illness and death. Any treatment that can impede the progression of sepsis, however small, is bound to have a positive effect on health outcomes. Hence, the possibility that the administration of albumin, through ameliorating the hypovolemia, may improve the outcome in septic patients requires serious consideration. A body of clinical investigation has studied this issue. A large clinical trial conducted in Australia and New Zealand studied the effect of albumin treatment in many groups of intensive care patients<sup>1</sup>. In the group of patients who had sepsis, albumin resulted in a 10 percent improvement in patient survival. Recently, a group of French investigators studied albumin infusion just in septic patients<sup>2</sup>. Again, a 10 percent improvement resulted from albumin.

Some important things need to be mentioned here. In both clinical studies, and in similar uses by other investigators,

the measured effects of about 10 percent increased survival were not statistically significant; i.e., the effect could have occurred through chance. It is difficult to address this problem through a single study, as it requires the treatment of a very large population of patients with sepsis. However, the finding that several studies consistently show a 10 percent survival improvement with albumin is tantalizing. Some might consider a 10 percent improvement to be modest, but they would be wrong. It represents a very large decrease in the burden of this illness.

But tantalizing effects need to be corroborated and such corroboration is now available. When several clinical trials are similar in their design, patient populations, etc., they can be brought together using a technique called meta-analysis. This method pools the results from the individual trials and assesses whether these pooled results reflect a clinical effect. In recent meta-analyses of clinical trials in sepsis using albumin<sup>3</sup>, the beneficial effect from the individual trials was confirmed and yielded a result that was statistically significant for the combined results.

We, therefore, live in very exciting times for the venerable plasma protein therapy that is albumin. These findings show that there is plenty of scope for continuing to use this product. More of this scope will be described during the Plasma Protein Forum in June in Reston, Virginia. See you there!

ALBERT FARRUGIA is PPTA's Vice President, Global Access

<sup>1</sup> *N Engl J Med.* 2004 May 27;350(22):2247-56

<sup>2</sup> <http://clinicaltrials.gov/ct2/show/NCT00327704?term=Albumin+AND+sepsis&rank=15>

<sup>3</sup> *Crit Care Med.* 2011 Feb;39(2):386-91



# PROF. PIER MANNUCCI HONORED AT IPPC 2011 WITH HILFENHAUS AWARD

BY KARA FLYNN

**PROF. PIER MANNUCCI WAS HONORED BY PPTA** with the prestigious Hilfenhaus Award at the 2011 International Plasma Protein Congress (IPPC) in Lisbon, Portugal, for his work over the past 30 years as a respected authority on hemostasis and thrombosis and his development of new drugs to treat patients with hemophilia and von Willebrand disease.

Named after Dr. Joachim Hilfenhaus, former head of PPTA's Viral Safety Working Party, who died in 1996, the Hilfenhaus Award recognizes individuals who have made contributions to patient access to safe plasma protein therapies.

The last two award winners were Prof. Johannes Oldenburg, a well known researcher in the fields of immunohaematology and transfusion medicine and Dr. Alain Fischer, who was honored for his outstanding research in the area of pediatric immunology.

Prof. Pier Mannucci studied medicine and surgery at the University of Milan. In the field of Internal Medicine, he was the scientific director (from 1994 till 2000) of the *Annali Italiani di Medicina Interna* (Italian Annals of Internal Medicine), the official scientific body of the Italian Society for Internal Medicine and became the President of this society in 2003.

In addition to serving on the editorial boards of numerous scientific publications, Prof. Mannucci is the author of 746 publications. The main focus of his publications has been hemophilia (especially the outcomes of treatment), von Willebrand disease (molecular basis and therapy), venous thromboembolism (the risk factors) and hereditary thrombophilia. He was among the 215 most cited researchers from 1981 and 1999 in the category of clinical medicine. In 2003, he has been nominated expert at the Consiglio Superiore di Sanità (Italy's High Health Council).

Prof. Mannucci has received numerous awards for his work, including the Robert Grant Medal and the International Prize on Hemophilia, and has served as president of the International Society of Thrombosis and Haemostasis. Currently, Prof. Mannucci serves as Chairman of the Department of Internal Medicine and Medical Specialties at the University of Milan.



KARA FLYNN is PPTA's Director, Global Communications



# PATIENT COLLABO

BY JULIE BIRKOFER AND LAURA SAVINI

## AROUND THE WORLD, COUNTRIES ARE STRUGGLING TO PROVIDE PATIENTS

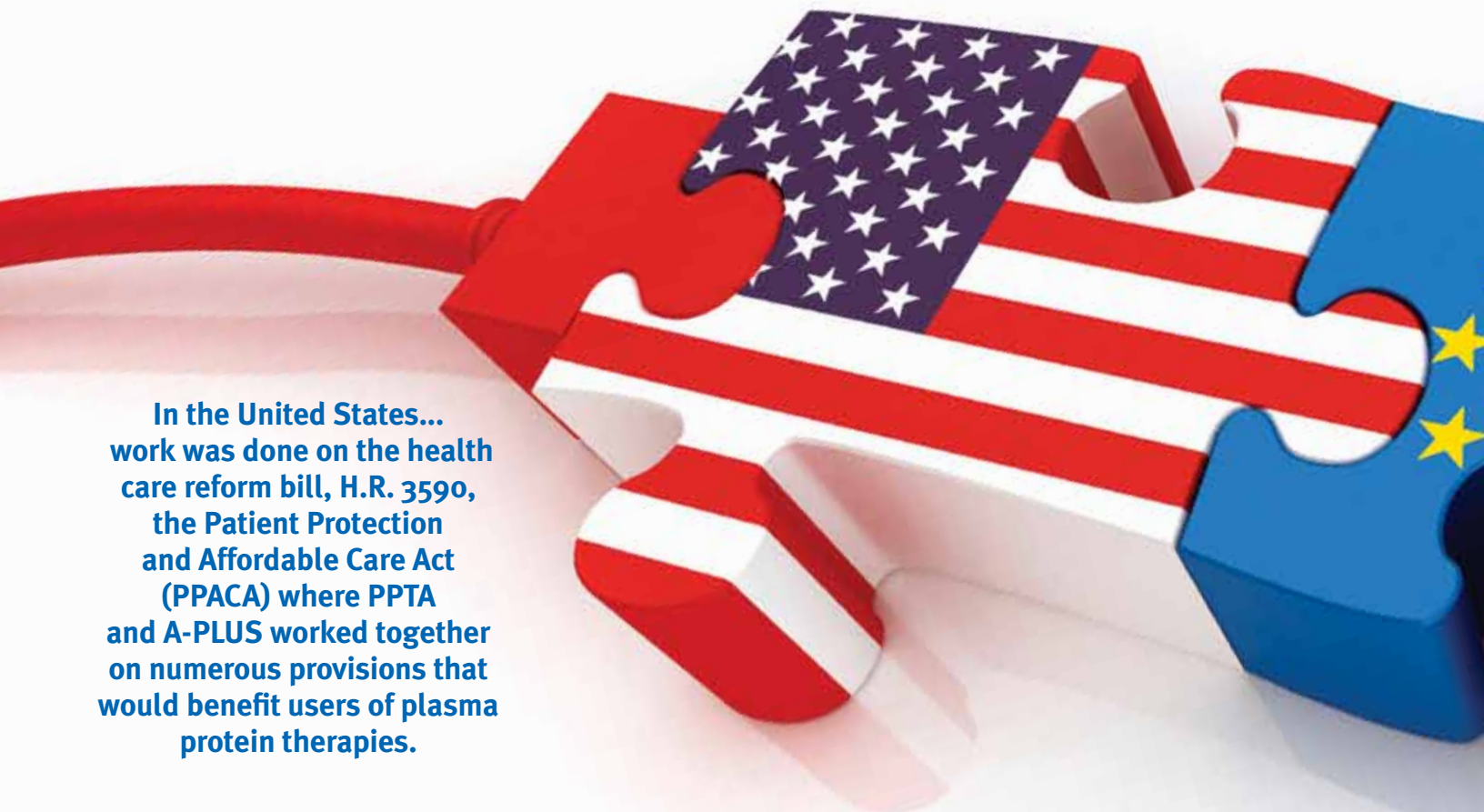
with access to needed health care, while simultaneously managing intense budgetary pressure. This pressure has generated several responses from stakeholders to continue to receive the same amount and level of service as before. As a result, both in Europe and in North America, patient groups are joining forces to collaborate on advocacy projects with common goals.

This trend is also seen among patients treated with plasma proteins: the coalitions PLUS<sup>1</sup> (Plasma Users) and A-PLUS<sup>2</sup> (American Plasma Users Coalition) set a recent example of this phenomenon.

In the United States, A-PLUS and PPTA have been working on several articles. For example, work was done on the health care reform bill, H.R. 3590, the Patient Protection and Affordable Care Act (PPACA), where PPTA and A-PLUS worked together on numerous provisions that would benefit users of plasma protein therapies. Stakeholders led the advocacy efforts to eliminate lifetime limits on insurance benefits and to combat the denial of insurers to cover illnesses based on preexisting conditions. These insurance reforms will help to give all Americans access and maintain the medical care they need. Furthermore, PPTA

and A-PLUS are working together on the annual pharmaceutical fee issue, a tax provision contained in the law. Several members of A-PLUS have weighed in with Members of Congress. Here is content from one letter from a Stakeholder: *"Upon review of PPACA's language concerning the pharmaceutical fee and the way in which orphan drugs are exempted, however, we remain concerned that the language of the exemption for orphan drugs, as constructed, does not protect all products that treat only rare diseases. Although the provision does exclude 'sales of any drug or biological product with respect to which a credit was allowed for any taxable year under Section 45C of the Internal Revenue Code of 1986,' it does not take into account those products that treat only rare diseases that were not eligible for that tax credit. This*

**In the United States... work was done on the health care reform bill, H.R. 3590, the Patient Protection and Affordable Care Act (PPACA) where PPTA and A-PLUS worked together on numerous provisions that would benefit users of plasma protein therapies.**




# RATION GLOBALLY

would leave patients and their physicians with no alternative therapies and hinder increased research and development.”

In Europe, PLUS has created a space for stakeholders involved in plasma proteins to meet and discuss their differences. For the past two years, stakeholders such as representatives from patients’ groups, blood donors, and the industry have used the PLUS platform to meet in Dublin to discuss common issues. One of the key outcomes from this meeting has been the recognition for patients to be an integral part of political and regulatory decision-making related to plasma protein therapeutics, especially on issues concerning sustained supply of these therapies. This could not have been achieved by one patient group alone and shows the power behind patients’ cooperation.

However, in order to work together, patient groups need to first be recognized as valuable stakeholders, which is well established in North America but is a recent trend in Europe. As stated by the European Patient Forum (EPF), an European coalition of 44 European patient organizations: “it was during the G10 Process and the Review of the Pharmaceutical Legislation in 2001/2003, [that] patient groups realized they were not appropriately represented at EU level and at the same time appreciated how difficult it was for the EU institutions to nominate a European patient representative.” For example, the European Medicines Agency (EMA) only

began involving patients in some of its working groups in 2006. In its Road Map to 2015 the EMA states that “An element of growing importance is the involvement and participation of civil society representatives (patients/users of medicines and healthcare professionals) in the Agency’s activities.” This shows not only a change in attitude from government and European agencies but also in patients’ attitudes, as they are empowered to make their voice heard. Taking part in official committees at the EMA and similar bodies also means being able to represent patients on a broader level, and to gather other patients’ views and opinions on certain topics, as explained by one of the patients’ representatives on these committees at a recent PPTA meeting.

Patient cooperation is a trend that is bound to grow. The industry will need to continue taking it into account in order to keep the dialogue with patients groups alive and relevant. 

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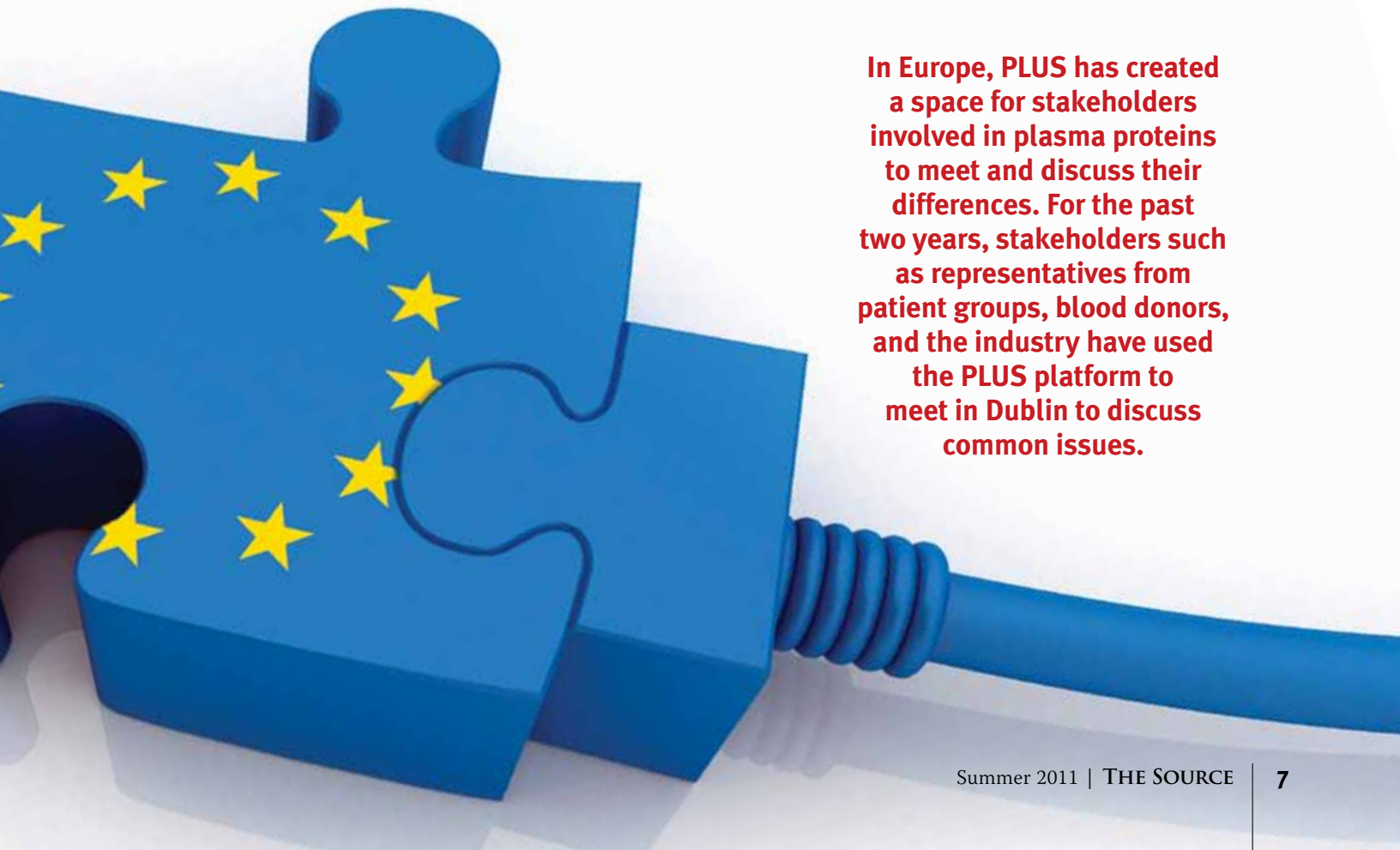
JULIE BIRKOFER is PPTA’s Senior Vice President, North America and LAURA SAVINI is PPTA Europe’s Manager, National Affairs

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**1** European Hemophilia Consortium, International Patient Organization for Primary Immunodeficiency.

**2** A-PLUS represents: GBS/CIDP Foundation International, Committee of Ten Thousand, Hemophilia Federation of America, Immune Deficiency Foundation, Jeffrey Modell Foundation, National Hemophilia Foundation, Platelet Disorder Support Association, Patient Services Incorporated.

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## TWO DAY EVENT FEATURES PANELS ON ISSUES IMPACTING INDUSTRY, STAKEHOLDERS

BY KARA FLYNN

**PPTA'S INTERNATIONAL PLASMA PROTEIN CONGRESS (IPPC) 2011**, held on March 15-16 in Lisbon, Portugal, attracted a record number of attendees with over 300 people participating in the discussions and hearing presentations on a number of topics.



Left to right: Johan Prévot of the International Patient Organisation for Primary Immunodeficiencies (IPOPI), Jacqueline Kerr, Paul-Ehrlich-Institut, Jan M. Bult, PPTA, and Johannes Blumel, Paul-Ehrlich-Institut.

Highlights of the event on March 15 featured an opening session headlined by Carrock Sewell, of the Scunthorpe General Hospital who spoke about the United Kingdom's Demand Management Plan. A panel on the world without industry standards featured perspectives from Jan M. Bult, discussing the role, implementation and future of industry standards; Jacqueline Kerr, Paul-Ehrlich-Institut, who shared views on whether industry standards matter from a European regulator's viewpoint; and Johan Prévot of the International Patient Organisation

ANDRIY BOZHOK / ISTOCK



for Primary Immunodeficiencies (IPOPI), who discussed industry standards from a patient's perspective. A panel that took place on the second day of the conference featured a noteworthy discussion on plasma that included perspectives from Albert Farrugia, PPTA's Vice President, Global Access on donor motivation; Ruth Offergeld, Robert Koch Institut, on the usefulness of donor surveillance and William G. Murphy of the Irish Blood



Attendees at one of the many sessions at the IPPC 2011 Congress.



Carrock Sewell  
Scunthorpe General Hospital

Transfusion Service, on challenges faced by the blood and plasma collection communities in understanding and managing risks in their respective sectors.

The full conference program featured several noteworthy sessions in-

cluding topics addressing access to care, regulatory considerations, pediatric to adult treatment and new developments and international aspects with regard to hemophilia care in Russia and the supply of plasma derivatives in low income index countries.

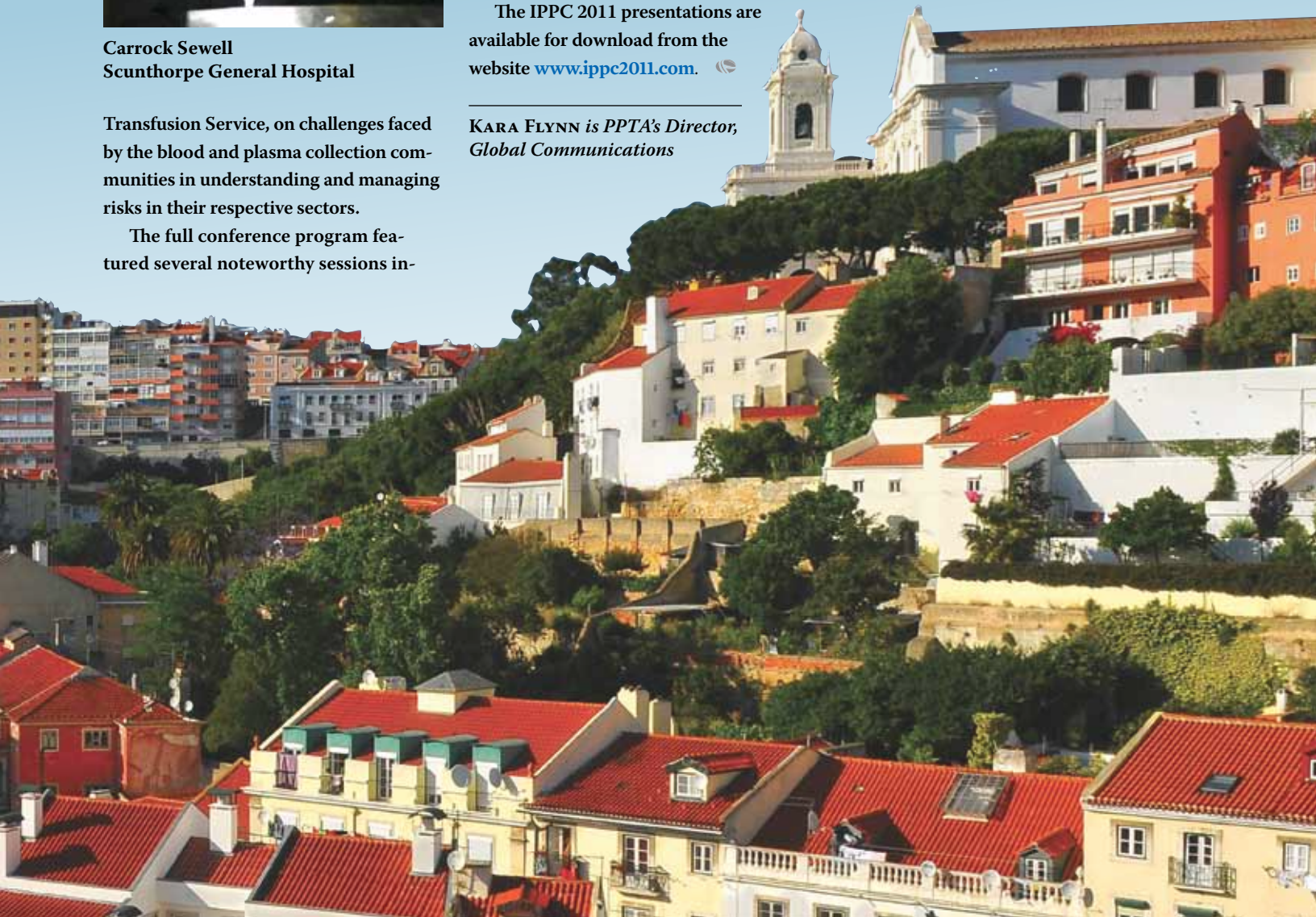
Delegate feedback of IPPC 2011 was very positive and a solid program, high-level speakers, a truly dynamic location and an impressive number of relevant attendees combined for the most successful event in PPTA's history.

The IPPC 2011 presentations are available for download from the website [www.ippc2011.com](http://www.ippc2011.com).

*KARA FLYNN is PPTA's Director, Global Communications*



**Please join us  
next year for  
IPPIC 2012  
on March 13-14  
in Madrid, Spain.**



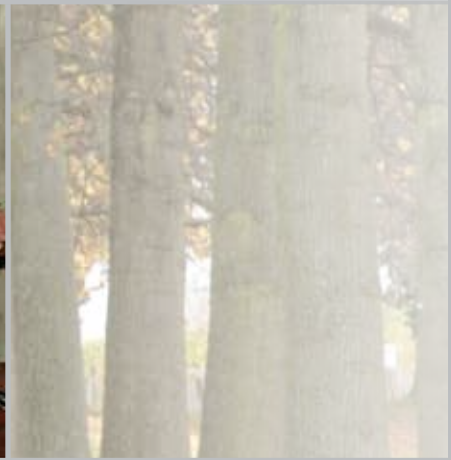


# HEALTH TECHNOLOGY ASSESSMENTS

## What Do They Mean for Plasma Protein Therapies?

BY JULIE BIRKOFER AND ALBERT FARRUGIA

**HEALTH TECHNOLOGY ASSESSMENTS (HTAs)** have joined the list of buzz words associated with health care reimbursement. Plasma protein therapies (PPTs) are not excluded from this involvement. It is therefore important to understand some of the basic principles around HTAs.



## What are HTAs?

HTAs are a group of methods designed for evaluating health care interventions. They can be applied for examining the use of prevention programs, for using medical devices, for using drugs and for medical and surgical procedures. HTAs generally involve the use of *economic evaluation* – i.e., how much money is involved – and also draw on the *evidence* of the use of interventions.

## Why the interest in HTAs?

As the population ages, the cost of health care increases – most health care is consumed by seniors. As medical technologies come into use, costs increase, prescribers and patients expect access to the latest innovations. The demands and expectations on health care are nearly infinite. At the same time, the capacity of payers (whether government or private) to reimburse consumers is under severe strain. Particularly during the current period of international economic downturn, methods are being sought to assist policy makers and payers to, effectively, establish ways of prioritizing interventions and, bluntly, ration health care and the allocation of scarce financial and human resources. *Inevitably, the use of HTAs leads to some patients receiving resources and treatments, and others not.*

## Types of HTAs – Cost Effectiveness Analysis

HTAs involve the use of economic analysis and clinical evidence. One major HTA is *cost-effectiveness analysis* (CEA). In CEA, the costs of a treatment are determined and compared to the benefits as measured through some clinical outcome. It is the purpose of CEA to quantify the value of such costs and benefits. Examples of CEA in the field of plasma protein therapies would include the costs of avoiding a bleed in hemophilia when using prophylaxis, or the costs of immunoglobulin therapy to prevent infections in primary immunodeficiency (PID). The parameter resulting from the comparison of costs and benefits is called *the cost effectiveness ratio*. Since many CEAs are used to compare two interventions, this involves the generation and comparison of two cost effectiveness ratios, to assess the advisability or otherwise of choosing one intervention versus another. An example could be assessing the treatment of hemophilia using prophylaxis versus in-demand treatments, or comparing two different dosages for preventing infection in PID.

## Cost – Utility Analysis and the QALY

Since CEA is widely used in policy making to develop priority scales for interventions, difficulties arise when widely different outcomes such as bleeds in hemophilia, infections in PID, respiratory volume in alpha-1-antitrypsin etc., come to be compared for their cost effectiveness. For this purpose, a special form of CEA has been developed, *Cost Utility Analysis* (CUA) in which costs are estimated for an intervention's ability to affect the overall quality of life of the patients involved. Rather than determine, for example, the cost of a treatment in terms of bleeds avoided or infections prevented, the cost of a year of the treatment's effect on the quality of the patient's life is calculated. To do this the *quality adjusted life year* (QALY) is

estimated. One QALY can be considered as a year in perfect health. For example, if the cost of prophylaxis in hemophilia is \$40,000 per QALY, it would mean that spending \$40,000 would result in one year of perfect health for the person with hemophilia. Similarly, if, again as an example, the QALY for a specific dose of immune globulin in PID is \$7,000 per QALY, it would mean that \$7,000 would result in one year of perfect health for a patient with PID. This allows all costs to be compared directly, given the commonality of the QALY.

## HTA: Role in Decisionmaking

Since President Barack Obama signed the health care reform bill (ACA) into law last year, many provisions are intended to contain costs of federally funded programs while ensuring the delivery of quality health care. Two key provisions of the law in which policy-makers will rely upon to make decisions regarding patient access to plasma protein therapies are: comparative effectiveness research (CER) and the Independent Payment Advisory Board (IPAB). HTAs will likely play an important role in both and shape policies that impact patient access to plasma protein therapies.

As part of its section establishing an independent patient-centered CER body, the ACA includes a rare disease advisory panel,

**Since President Barack Obama signed the health reform bill (ACA) into law last year, many provisions are intended to contain costs of federally funded programs while assuring the delivery of quality health care.**

which U.S. stakeholders and PPTA championed. This provision requires the appointment of an expert advisory panel during each instance a rare disease is being considered for a CER study for the purpose of assisting in the design of the research study and determining the relative value and feasibility of conducting the research study.

While the CER provisions have strong safeguards for patient access, the IPAB provisions fail to protect patients in a similarly adequate manner. The purpose of the IPAB that will be created as a result of the passage of health care reform is to reduce the per capita rate of growth in Medicare spending. Beginning in 2014, the IPAB would submit to Congress and the Administration, any recommendations to this effect if the Chief Actuary of the Centers for Medicare and Medicaid Services (CMS) determines in the previous year that such a growth rate will increase faster than an established inflation rate. These recommendations would automatically go into effect the following year unless subsequent legislative action is taken by a certain date.

In this article, we have attempted to describe the basic principles of some HTAs. In future issues of *The Source*, we will continue to describe other HTAs and their direct application in plasma protein therapies.



JULIE BIRKOFER is PPTA's Senior Vice President, North America and ALBERT FARRUGIA is PPTA's Vice President, Global Access.



A woman with short brown hair, wearing a red sweater, is seated in a red medical chair. She is donating blood, with a red tube connected to her arm. She is holding a red ball in her right hand. The background is a plain white wall. The title 'BLOOD CENTERS' is overlaid on the right side of the image. The word 'BLOOD' is in red, and 'CENTERS' is in black. A large blue cross is partially visible on the right side of the image.

# BLOOD CENTERS

BY SYBILLE BECK



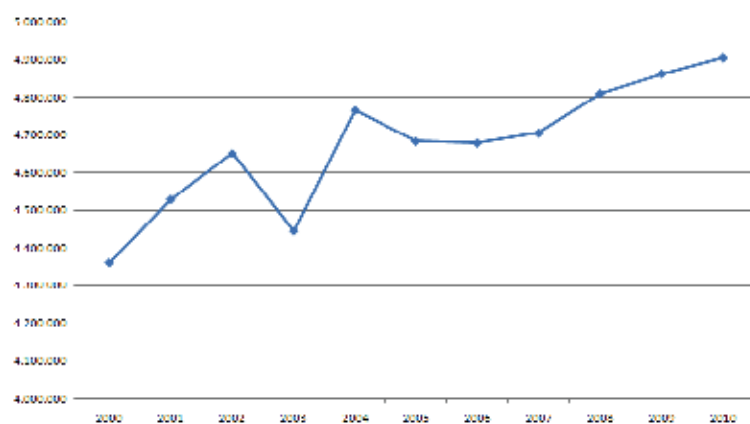


country collecting and exporting more plasma for fractionation than the United States. The history of this successful program goes back to the 1970s and yet the U.S. is able to maintain an average of 45.946 whole blood donations per 1,000 inhabitants<sup>6</sup>, which would still be far above the average calculated by the EDQM.

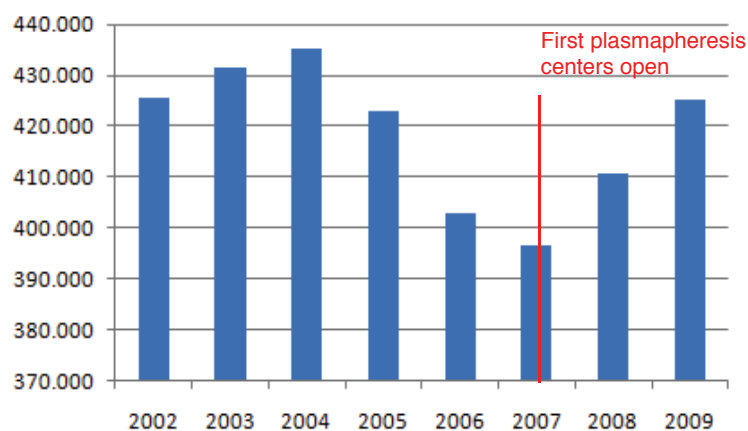
### The Czech example

While we draw our conclusion, that the co-existence of two sectors in Germany and Austria is not harmful to whole blood collections on the simple fact that the levels of donations are among the highest in Europe and the experience that increased awareness for whatever donation (blood or plasma) leads to a beneficial spillover effect to the other donation, the Czech Republic offers an interesting snapshot of how donations develop when plasma centers are newly established. The first independent plasma centers in the Czech Republic only opened at the end of 2007 offering an observation period of two years based on the

**FIGURE 2—WB donations in Germany 2000–2011**



**FIGURE 3—Development of whole blood donations<sup>5</sup> between 2002 and 2009.**



\*Source: Ústavu zdravotnických informací a statistiky České republiky 2009

**TABLE 1—Regions in the Czech Republic and number of plasma centers**

Region	Number of plasma centers
HL. m. Praha	2
St_edo_esk	0
Jiho_esk	1
Plze_sk	1
Karlovarsk	0
Ústeck	1
Liberecký	1
Královéhradeck	0
Pardubick	1
Vyso_ina	0
Jihomoravsk	1
Olomouck	1
Zlinskí	1
Moravskoslezsk	1

excellent reports complied by the Czech authorities.

Much more interesting in terms of the effects of the coexistence of blood banks and source plasma centers in close proximity are the regional data provided:

In four out of the 14 regions there are no plasma centers, with the 11 plasma centers spread over 10 regions. Fig. 4 shows the developments of whole blood donations in all regions and it is clear that in 2009 all regions with a plasma center documented increased whole blood donations, with the largest increase in the Prague area where two centers are located. Conversely, in three out of four regions where there are no plasma centers, the number of blood donations actually declined.

### Conclusions

Many seem to have the impression that it is a law of nature that the opening of a plasma center means that blood donors stop donating blood and choose to donate plasma instead. Clearly, in the Czech Republic this is not the case. Blood donations increased after the opening of plasma centers and also following a period of decreasing blood donations.

Experience consistently shows that an active plasma donation program based on voluntary compensated donors is not a threat to the collection of blood for transfusion. On the contrary, based on the experiences cited above, countries with established plasma donation programs (Austria, Germany and the

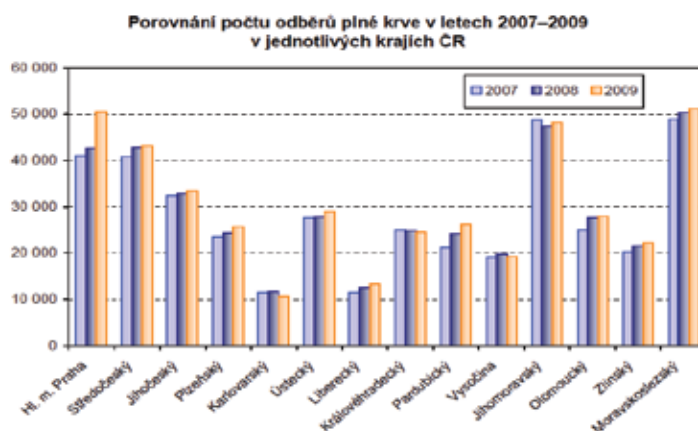
U.S.) and a country that is having its first experiences with voluntary, compensated plasma donors, the level of blood donations actually increases. The two sectors can legitimately be said to be mutually beneficial.

Unfounded generalizations are unhelpful and might lead to decisions based on perceptions rather than real evidence and data, which can limit the availability of scarce materials to the detriment of patients in need.

Considering the large number of under-treated and untreated people needing plasma protein therapies this must be good news and it should not be seen as a threat to blood donation programs.

SYBILLE BECK is PPTA Europe's Assistant Director, Source Europe and Germany

FIGURE 4—Regions with/without plasma centers



1 Emmanuel JC, Global Access To Plasma Therapeutic Products,- Understanding The Void ; presented at International Plasma Protein Congress, Lisbon, March 2011

2 P.Flanagan, ISBT Board response to the Dublin consensus statement, Vox Sang (2011) 100 (2), 250–251

3 van der Poel CL, Janssen MP, Behr-Gross ME, The Collection, Testing and Use of Blood and Blood Components in Europe - 2006 Report, available at [http://www.edqm.eu/medias/fichiers/The\\_Collection\\_Testing\\_and\\_Use\\_of\\_Blood\\_and\\_Blood\\_1.pdf](http://www.edqm.eu/medias/fichiers/The_Collection_Testing_and_Use_of_Blood_and_Blood_1.pdf)

4 Paul-Ehrlich-Institut, Berichte nach § 21 Transfusionsgesetz (TFG), 2010 report available at [http://www.pei.de/cdn\\_101/nn\\_156172/SharedDocs/Downloads/21tfg/tabellen-abbildungen-tfg21-10,templateId=raw,property=publicationFile.zip/tabellen-abbildungen-tfg21-10.zip](http://www.pei.de/cdn_101/nn_156172/SharedDocs/Downloads/21tfg/tabellen-abbildungen-tfg21-10,templateId=raw,property=publicationFile.zip/tabellen-abbildungen-tfg21-10.zip)

5 Ústavu zdravotnických informací a statistiky České republiky 2009

6 Cruz JR, Pérez-Rosales MD, Availability, safety, and quality of blood for transfusion in the Americas, Rev Panam Salud Publica/Pan Am J Public Health 13(2/3), 2003







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# EUROPEAN COURT OF JUSTICE WEIGHS IN ON DONOR COMPENSATION DEBATE

BY JOHN DELACOURT

## IN A CASE WITH SIGNIFICANT IMPLICATIONS

**for the plasma protein therapies industry, the European Court of Justice has ruled that an Austrian law banning the importation of blood products not obtained from donations made “without any payment whatsoever” violates EU rules on the free movement of goods between Member States.**

**In *Humanplasma GmbH v. Republik Österreich*,<sup>1</sup> the Court held that the health and safety concerns raised by the national authorities do not outweigh the free trade priority embodied in Article 28 of the TFEU. Although the Austrian law at issue related specifically to blood products for direct transfusion, the Court’s analysis—and ultimate rejection—of the health and safety rationales for restrictions on compensated whole blood donation applies even more strongly to donations of source plasma.**

## Case Background

The case emerged from Humanplasma’s efforts to supply blood products to the Wiener Krankenanstaltenverbund (Vienna Hospital Association). In November 2005, the Hospital Association sought bids on a contract to supply leukocyte-depleted erythrocyte concentrates. Humanplasma was the low bidder, and confirmed that its proposal was in compliance with all applicable provisions of the *Arzneiwareneinfuhrgesetz* (Medicinal Imports Law). However, before Humanplasma could fulfill the contract, the Medicinal Imports Law was amended.

The amended law stated that “When blood products are imported for direct transfusion they may in any case not be placed on the market unless the blood was donated without any payments whatsoever having been made.”<sup>2</sup> Because Humanplasma’s products were obtained primarily from German blood donors to whom some compensation was provided, it was unable to comply with this condition and its previously successful bid was rejected. In response, the company filed an objection with the *Vergabekontrollsenat für Wien* (Public Procurement Review Tribunal, Vienna), which concluded that the bid had been properly rejected.

Humanplasma then appealed the Procurement Tribunal’s decision to the *Landesgericht für Wien Zivilrechtssachen Wien* (Regional Civil Court, Vienna), asserting for the first time that the provisions of the Medicinal Imports Law on donor compensation violated EU law. The Regional Court, in turn, stayed the proceeding and referred the following question to the European Court of Justice for a preliminary ruling:



“Does Article 28 EC (in conjunction with Article 30 EC) preclude the application of a national provision under which the importation of erythrocyte concentrates from Germany is permitted only where the blood was donated without any payment having been made (with not even expenses being covered), that being a condition which is also applicable to the obtaining of erythrocyte concentrates within Austria?”<sup>3</sup>

### The Court’s Decision

The Austrian authorities argued that the ban on donor compensation advanced an objective warranting an exemption from Article 28’s guarantee of the free movement of goods between Member States – namely, the protection of human health. The Court’s analysis therefore focused on whether the provision in question – a total ban on all forms of donor compensation – was appropriately limited or, rather, went beyond what was necessary to achieve this objective.

The Court held that the provision was *not* appropriately limited, stating with surprising directness that “the obligation that the blood donation must have been made without any of the costs incurred by the donor being reimbursed is . . . not necessary in order to ensure the quality and safety of the blood and the blood components.”<sup>4</sup>

The Court reasoned, first and foremost, that the principal safeguard on the quality and safety of blood products is a strict regime of post-donation testing.<sup>5</sup> The Court noted that the E.U.’s own blood directive mandates such testing, and requires that it evolve to reflect the scientific and technical state-of-the-art. The Court also observed that the E.U.’s blood directive does not require that donations be “completely unpaid,” but rather contemplates such compensation as small tokens, refreshments, and reimbursement of travel costs.<sup>6</sup> Finally, the Court explained that the rigidity of the Austrian law made it an outlier, as a number of other Member States – all of which regard the safety of the blood supply as a national priority – permit the reimbursement of at least some of a donor’s costs.<sup>7</sup>

### Impact of the Decision

Although the Humanplasma decision relates to blood products for transfusion, and could have gone even further in its endorsement of compensated donation, it is nevertheless likely to prove

useful to supporters of compensation for source plasma donors. A number of the positions adopted by the Court arguably provide the legal framework for future challenges to restrictions on donor compensation, and will almost certainly influence the related legislative and public policy debates. These include the following:

- **Donor Compensation Is Not a Safety Issue** – The Court stated unambiguously that the Austrian law’s restrictions on donor compensation were “not necessary” to ensure quality and safety. This is a major blow to one of the two central justifications for restrictions on donor compensation, the other being the claim (unsupported by data) that compensated blood and plasma donations “crowd out” uncompensated donations<sup>8</sup>.
- **Post-Donation Safeguards Are Critical** – Much of the Court’s comfort with the safety of compensated donation stemmed from its confidence in the European-level regulatory framework for post-donation testing. This rationale applies even more strongly in the plasma therapies context, where final products are subjected to two sets of post-donation safeguards: (1) pathogen testing, and (2) pathogen inactivation. In contrast, the viral inactivation procedures that are now industry standard in the plasma therapies context are often unavailable in the whole blood context, as they may result in the destruction of critical blood components.
- **Compensation Is Not the Same as Payment** – Although the specific examples of permissible donor compensation offered by the Court included only small tokens, refreshments, and reimbursement of travels costs, and did not expressly include cash payments, the Court did appear to endorse the broader principle that reimbursement of donor costs is acceptable and non-problematic. In doing so, the Court seemed to accept a principle long advocated by supporters of donor compensation: that “payment” (for the biological materials themselves) is not the same as “compensation” (for the donor’s time and inconvenience). It remains to be seen whether the Court’s decision will be construed as going this far. To the extent that it is, this rationale also applies more strongly in the plasma therapies context, as plasma donors are encouraged to donate on a qualified, repeat basis, and each individual donation session take longer. Consequently, a typical plasma donor incurs more reimbursable costs, in terms of time and inconvenience, than a typical whole blood donor.

JOHN DELACOURT is PPTA’s Senior Director, Legal Affairs

<sup>1</sup> Case C-421/09, Humanplasma GmbH v. Republik Österreich, CELEX No. 609J0421 (Westlaw) (Dec. 9, 2010).

<sup>2</sup> *Id.* at ¶ 10.

<sup>5</sup> *Id.* at ¶ 42.

<sup>3</sup> *Id.* at ¶ 23.

<sup>6</sup> *Id.* at ¶ 44.

<sup>4</sup> *Id.* at ¶ 43.

<sup>7</sup> *Id.* at ¶ 41.

<sup>8</sup> Sybille Beck, Blood Centers and Plasma Centers: Mutual Benefit, *The Source*, Summer 2011, Page 12.





# Hurdles of Implementing HEALTH CARE REFORM at the State Level

BY BILL SPEIR

**IN THE UNITED STATES**, the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act (known collectively as the “ACA”) require state governments to make numerous decisions about health care reform implementation and what roles they will play and what roles they will leave to the federal government.

The health care reform law requires much of state policymakers. States must consider how they will implement the new health insurance requirements, how they will fund Medicaid expansion, and what role they will play in the administration of health insurance exchanges.

To help them with the numerous decisions they must make, many states have established health care reform committees to examine the federal law and provide guidance for the state on what is best for the state and its residents. The chart below identifies the states that have established committees. Some states have more than one committee because the governor and the legislature each established their own committee.

**These committees will need to:**

- Conduct a comprehensive review of the state’s current health care delivery system
- Identify strengths and weaknesses

- Develop a timeline for implementation
- Assess what resources are needed
- Involve stakeholders in the process
- Recommend implementing legislation
- Perform strategic planning on the operational, financial, and technical requirements to sustain a health benefits exchange.

The health insurance exchanges are getting a lot of attention since the states need to have made progress towards establishing an exchange by January 1, 2013 or the federal government will administer the exchange in their state. The first thing the state decision-makers must decide is whether they should establish their own exchange, or defer to the federal government.

If state decision-makers decide to administer their own exchange, they need to consider how to develop a health insurance exchange that meets the needs of their state’s residents, while meeting the requirements of the ACA. Core issues to consider in designing the health insurance exchange include how will the health insur-

ance exchange be governed; how will the health insurance exchange coordinate with the state's Medicaid program; and what role will the health insurance exchange play in the selection of health plans for individuals?

These committees and other state decision-makers face hurdles in implementing health care reform. These hurdles include lack of guidance from the federal government, funding, and politics.

### Lack of Federal Guidance

According to those attempting to set up state health insurance exchanges, the online process will resemble applying for a mortgage more than purchasing a plane ticket. This is because there are many questions surrounding the exchanges that are not answered by the federal health care reform law and therefore the development of regulations will be vital for final implementation.

The U.S. Department of Health and Human Services (HHS) is expected to release the state exchange regulations in the late spring. Many of the details on how exchanges are governed and operated will be up to the states. The forthcoming

regulations, however, will spell out the requirements state exchanges must meet in order to be acceptable under the health care reform law. Until these regulations are released, the state committees advising state decision-makers on health insurance exchange implementation cannot be sure what the state health insurance exchange must do to meet federal requirements.

### Funding


Forty-eight states have accepted at least \$1 million each from the federal government to help plan for the insurance exchanges. States applied to use those grants for a number of important planning activities including research to understand their insurance markets, efforts to obtain the legislative authority to create exchanges, and steps to establishing the governing structures of exchanges.

The states need funds to improve their technology infrastructure in order to implement health care reform. To meet this need, the federal government is providing "Early Innovator" grants totaling \$241 million to six states and a multi-state consortium led by the University of Massachusetts Medical School, for development of the technol-

ogy infrastructure needed to operate the exchanges. These "Early Innovator" states are expected to develop health insurance exchange IT models, building universally essential components that can be adopted and tailored by other states.

### Politics

It is difficult to implement a new policy if powerful decision-makers refuse to adopt the policy. Implementation of the ACA is no exception. Certain states are stopping implementation efforts because of the actions of certain elected officials.

Governors in Alaska, Georgia, Florida, Idaho, Montana, and Texas have refused to implement certain provisions of the ACA. Former Minnesota Governor Tim Pawlenty signed an Executive Order that prohibited all executive departments and state agency participation in federal health care reform unless required by law or directed by the governor's office. As a result, Minnesota was not awarded any grants for implementing the exchange through the U.S. Department of Health and Human Services. 

**BILL SPEIR** is PPTA's Director, State Affairs

## STATE COMMITTEES

**Alaska** Health Care Commission

**California** Health Care Reform Task Force

**Colorado** Interagency Health Reform Implementation Board

**Connecticut** Health Care Reform Cabinet

**Delaware** Health Care Commission

**District of Columbia** Health Care Reform Implementation Committee

**Idaho** Health Care Task Force

**Illinois** Health Care Reform Implementation Council

**Iowa** Legislative Health Care Coverage Commission

**Maine** Health Reform Implementation Steering Committee

**Maine** Joint Select Committee on Health Reform Opportunities and Implementation

**Maryland** Health Care Reform Coordinating Council

**Michigan** Health Insurance Reform Coordinating Council

**Minnesota** Health Care Reform Task Force

**Mississippi** Health Insurance Exchanges Study Committee

**Montana** Interim Committee within the Children, Families and Health and Human Services Interim Committee

**Nevada** Health Care Reform Policy Planning Group

**New Hampshire** Commission on Health Care Cost Containment

**New Mexico** Health Care Reform Leadership Team, in August 2010 became the Office of Health Care Reform

**New Mexico** Health Reform Working Group

**New York** Cabinet to Implement Federal Health Care Reform

**North Carolina** Health Reform: Overall Advisory Committee

**North Dakota** Interim Industry, Business, and Labor Committee

**North Dakota** Legislative Panel

**Ohio** Health Care Reform Stakeholder Forum was formed as part of the Ohio Health Care Coverage and Quality Council

**Oregon** Health Policy Board

**Pennsylvania** Commonwealth Health Care Reform Implementation Committee and the Health Care Reform Advisory Committee

**Rhode Island** Special Senate Commission

**Rhode Island** Healthcare Reform Commission

**Rhode Island** Joint Legislative Committee on Health Care Oversight

**Texas** House Select Committee on Federal Legislation

**Vermont** Governor's Health Care Cabinet

**Virginia** Health Care Reform Initiative

**Washington** Health Care Cabinet

**Washington** Joint Select Committee on Health Care Reform

**West Virginia** Governor's Office of Health Enhancement and Lifestyle Planning

**Wisconsin** Office of Health Care Reform

**Wisconsin** Special Committee on Health Care Reform Implementation

**Wyoming** State Leadership Team





# RESOLVING BARRIERS TO THE TREATMENT OF PRIMARY IMMUNODEFICIENCIES

Continued Progress in Germany

BY DR. INES SCHÖNDORF

## What are Primary Immunodeficiencies?

Primary Immunodeficiencies (PIDs) are rare, congenital disorders of the immune system. Patients suffer from increased susceptibility to infections, autoimmune diseases or cancer. Severe forms of PID cause death in early infancy. Timely diagnosis and optimal treatment are important to reduce morbidity and mortality. The use of immunoglobulin products is established for many PIDs, especially the most common forms such as Common Variable Immune Deficiency (CVID) and other deficiencies in the body's production of immunoglobulin, which is crucial for defense against infection.

## Current situation of patients with PID in Germany

As also recognized in the EU PID consensus conference report<sup>1</sup> PIDs are widely undiagnosed. The most obvious explanation for the situation is lack of awareness, hindering early diagnosis. In addition, insufficient knowledge about the diseases may lead to inappropriate therapies.

The network initiative FIND-ID has been formed to improve this situation<sup>2</sup>. Initiated by PID experts and patients, the steering committee currently consists of experts for adult and pediatric PID

patients as well as the patient organization. Two aspects were regarded as crucial to the success of the initiative: a proper analysis of the situation in Germany to arrive at the right measures and the early buy-in of all important stakeholders. Both aspects were met by conducting semi-structured, face-to-face interviews, for which a suitable questionnaire had been developed.

## Results of analysis

Analysis of the interviews revealed three major barriers to PID treatment: awareness, financing and structure of the care-system (see figure 1).

Interestingly, most interview partners underlined the need for more **awareness** among healthcare professionals for adult patients, while pediatricians are seen as being much more aware of those diseases.

With regard to **financing** the care of PID patients, the situation in Germany is very heterogeneous. There are outpatient clinics treating patients without proper reimbursement for the

medical care, thus generating financial losses. Some university hospitals have individually negotiated special flat-rates for the patients treated in their outpatient clinic. Pediatric centers can charge higher rates according to a special legal basis for interdisciplinary patient care in children. Others make use of a relatively new option of the so-called *specialized outpatient clinics according to §116b, SGB V*. This allows them to get reimbursement not only by a flat-rate, but for any procedure they perform, including laboratory assays. The reimbursement is based on a large catalogue (called "Einheitlicher Bewertungsmaßstab", EBM), listing all procedures and the corresponding reimbursement figures. To make it even more complicated, the figures not only depend on the activities performed, but also on the specialization of the caregiver, as

different figures apply to physicians of different medical specializations.

Though very complicated, this system at least allows for covering of costs. Hospitals which would like to use this option have to apply and to fulfill special criteria.

Without a system covering the expenses for diagnosis

**Without a system covering the expenses for diagnosis and treatment, it is hardly possible to increase numbers and capacities of specialized PID centers.**



and treatment, it is hardly possible to increase numbers and capacities of specialized PID centers.

Concerning the **structural** barriers, huge challenges are again faced by adult PID patients. While a number of university hospitals offer specialized centers for children, the number of specialized centers for adults is very limited. Long distances and limited capacities at those centers may hinder access. As a consequence, patients are treated by non-experts and/or practice “doctor-hopping.” In both situations, quality of care can’t be guaranteed. A further structural barrier is the transition from pediatric to adult care (“transition medicine”).

The situation is especially difficult for adult PID patients.

#### Measures by FIND-ID

The FIND-ID initiative has started to tackle those barriers simultaneously. To increase **awareness**, workshops have been performed, and extensive information on the website as well as printed material tailored to different stakeholders has been published. The initiative has been

presented through oral and poster presentations at different congresses. As many adult PID patients present with symptoms treated by ear, nose and throat (ENT) specialists, ENT became the focus for 2011, with both booths and symposiums at ENT-congresses.

With regard to **financing**, an extensive information package has been prepared and made available. It includes information about the legal background, calculation tools, application and reporting forms.

To improve the **structural** aspects FIND-ID has created a three layer network, embracing general practitioners and peripheral hospitals as well as specialized PID centers. It provides support for a new outpatient clinic for adult patients, which serves as a “model center.” Special workshops for caregivers new to the field of PID are planned. In addition, FIND-ID has started initiatives in the field of transition medicine, by presenting at a symposium on this topic.

The network initiative FIND-ID addresses awareness, financing and structural aspects.

#### Conclusion

In Germany, improvements in the field of awareness, financing and structure of care are needed to improve the situation for PID patients. The network initiative FIND-ID strives to adequately address all these fields.

DR. INES SCHÖNDORF is the medical coordinator for the network initiative FIND-ID in Germany

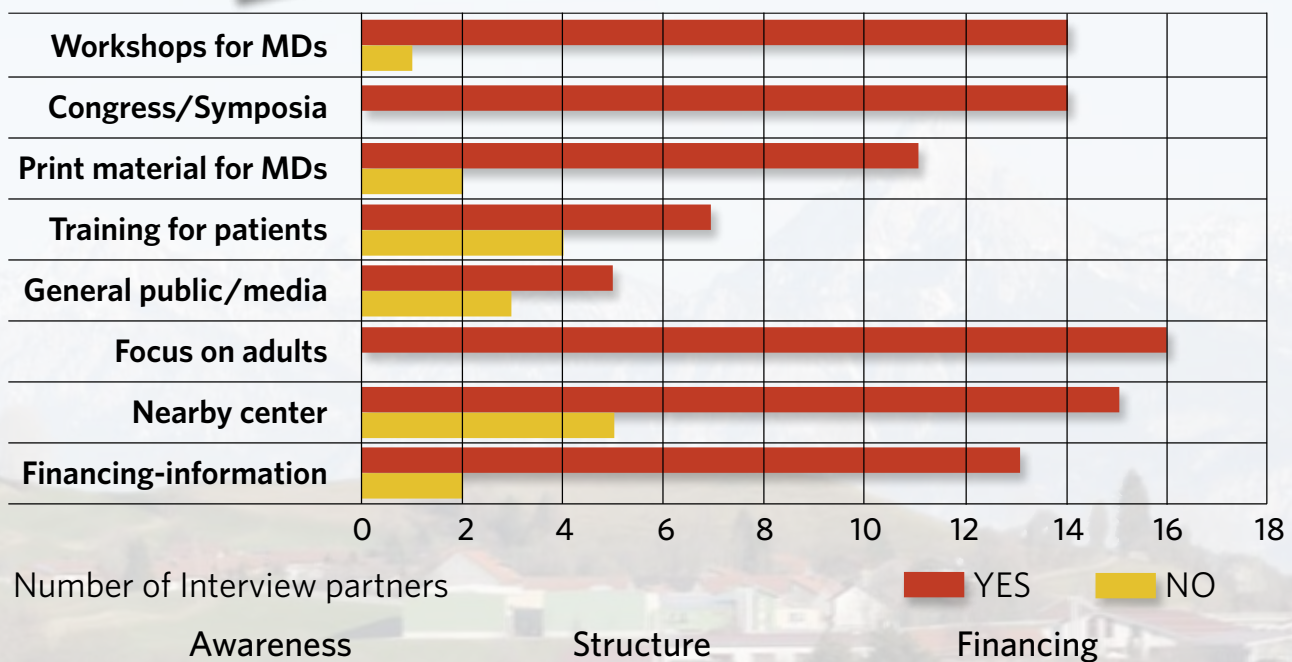
1 Expert Recommendations for better Management of Primary Immunodeficiency (PID), EU-PID Consensus Conference.  
<http://www.ipopi.org/news/epicc-statement.html>; [www.eupidconference.com](http://www.eupidconference.com)

2 FIND-ID – a physician-driven, multi-disciplinary and multi-professional initiative to increase the diagnosis-rate of Primary Immunodeficiencies (PID) in Germany. FIND-ID Group: Steering Committee: \*Wahn, V., Gründl, G., Niehues, T., Zepp, F., Franke, K., Schmidt, R., Warnatz, K., Med. Coord. Schoendorf, I. Poster presentation at the ESID congress October 2010, Istanbul



### FIGURE 1 - STAKEHOLDER SURVEY

Important issues of PID as possible objectives of FIND-ID







**Teaming with Entities Outside the  
Plasma Protein Therapeutics Sector**

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# COALITION BUILDING

## TO MODIFY THE ORPHAN EXCLUSION FROM THE ANNUAL PHARMACEUTICAL FEE

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BY JAY GREISSING

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### **AS PART OF THE AGREEMENT THE PHARMACEUTICAL RESEARCH AND MANUFACTURERS ASSOCIATION**

(PhRMA) reached with the Obama Administration during the health care reform negotiations, Senate Committee on Finance Chairman Sen. Max Baucus (D-MT) included an annual pharmaceutical fee in what became the Affordable Care Act. The Internal Revenue Service (IRS) will assess the fee, which will be treated as an excise tax, on the sales of branded prescription drugs into certain government channels. Although the tax is apportioned by the market share of each product, the Finance Committee staff realized it was necessary to protect continued innovation in the rare disease therapy space and so expressly excluded sales of “orphan drugs” from a manufacturer’s fee liability.

“Orphan drugs” are pharmaceuticals or biologicals that have received “orphan designation” from the U.S. Food and Drug Administration (FDA) when their sponsors filed the new drug application or biological license application. A drug that has received “orphan designation” is eligible for a number of incentives, including seven years of marketing exclusivity, grants, and a tax credit for its clinical testing expenses in bringing the product to market for that particular rare disease indication. The exclusion from the annual pharmaceutical fee inexplicably requires the manufacturer to have taken this orphan drug tax credit, rather than to have merely obtained “orphan designation” for its rare disease therapy.

The existing regulatory framework for obtaining “orphan designation” has effectively made it impossible for many plasma protein therapies, despite being exclusively FDA-indicated for the treatment of rare diseases, disorders, and conditions, to have ever taken

the orphan drug tax credit as required for the exclusion from the annual pharmaceutical fee. For a previously unapproved drug or for a new orphan indication for an already-marketed drug, the process of obtaining an orphan designation is relatively straightforward. Generally, the sponsor or manufacturer must only demonstrate that the product will satisfy the rare disease threshold of treating less than 200,000 patients in the U.S. The subsequent brands in the same therapeutic class seeking approval for the same rare disease indication must demonstrate “clinical superiority” to the first-to-market brand in terms of safety or effectiveness, or demonstrate it is making a “major contribution to patient care.” Because of this high threshold, FDA has rarely granted orphan designation to these second-to-market therapies. As a result, plasma protein therapies represent 33 of the 41 drugs identified that are exclusively FDA indicated for the treatment of rare diseases, disorders, or con-

ditions, but lack an "orphan designation" from the Agency.

There is a coalition forming with drug manufacturers outside of the plasma protein therapeutics industry to seek an amendment to the current orphan drug exclusion. Companies like Cephalon, Endo, Shire, Celgene, Millenium, and AstellasPharma U.S. are supportive of PPTA's policy proposal to expand the orphan drug exclusion to also exclude drugs approved or licensed by FDA for marketing solely for one or more rare diseases or conditions. The interest of these companies primarily lies with the decision by the Finance Committee to hinge the exclusion on the orphan drug tax credit and the interpretation of the IRS that the manufacturer had to have actually taken the credit, not merely qualify for it.

Many drug manufacturers were either unable to take the credit (for reasons beyond failing to obtain orphan designation), or made the business decision to take a more attractive credit. For example, the tax credit was not available for an 18 month period in the late 1990s. Prior to June 1, 1997, the Orphan Drug Act tax credit was not permanent. Between January 1, 1995 and June 30, 1996, Congress had failed to reauthorize this provision, making it impossible for manufacturers to claim the credit for clinical testing expenses incurred during that period. Additionally, most drug manufacturers did not claim the tax credit during the 1980s and early 1990s because they were receiving special tax breaks for having established manufacturing operations in Puerto Rico. More than 40 of

the world's largest drug manufacturers created thousands of jobs in Puerto Rico during this period in return for a tax exemption for all income derived from the specified facility. Manufacturers that elected the tax credit for doing business in Puerto Rico in a given year could not also claim the orphan drug tax credit for any qualifying clinical testing expenses incurred during that same taxable year. It is also important to note that during the first 12 years of the Orphan Drug Act, new market entrants were unlikely to claim the orphan drug tax credit because manufacturers could not carry unused credits forward or backward; thus, initially under the law, manufacturers had to have income and high enough tax liability to take the orphan drug tax credit, which was difficult for newer market entrants that lacked revenue. Finally, some manufacturers may choose to claim the research and development tax credit, rather than the orphan drug tax credit for its clinical testing expenses because they are unable to claim both credits for the same qualifying clinical testing expenses.

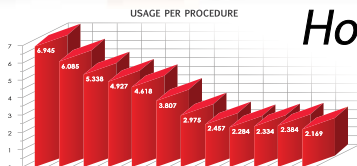
Regardless of the rationale, a broad coalition supporting the modification of the orphan drug exclusion is vital to its success. PPTA will strongly advocate for this policy that will reward past and encourage future innovation in developing therapeutic interventions for the treatment of rare diseases, disorders, and conditions. ☞

JAY GREISSING is PPTA's Senior Director, Federal Affairs

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# INSIDE PPTA

PPTA NEWS FROM  
AROUND THE GLOBE

## ▼ 31<sup>ST</sup> ISICEM CONGRESS HELD IN BRUSSELS, BELGIUM

PPTA organized a satellite symposium during the 31st International Symposium on Intensive Care and Emergency Medicine (ISICEM) Congress on March 24 in Brussels, Belgium. This event is organized by the Department of Intensive Care Emergency Medicine of Erasme University Hospital, Université Libre de Bruxelles in association with the Belgian Society of Intensive Care and Emergency Medicine. Started in 1980, this meeting, held every March, is now one of the largest of its field and attracts up to 5,600 participants. During the satellite symposium, key opinion leaders, Prof. Simon Finfer (Australia), Prof. Timothy W. Evans (United Kingdom) and Dr. Pietro Caironi (Italy) presented the latest developments on the use of albumin in sepsis. The satellite symposium was chaired by Prof. Albert Farrugia, PPTA's Vice President, Global



## ▼ PPTA MEETS WITH EUROPEAN MEDICINES AGENCY (EMA)

PPTA met with European Medicines Agency (EMA) experts for its annual liaison meeting. The meeting has become a valuable event, as EMA outlines its priorities for the year ahead. Subjects covered in the briefing included: contract fractionation and the related revision to Annex 14 of the Good Manufacturing Practices (GMP) requirements, EMA Road Map and fee structure, review of guideline 269/95 (EMA believe the industry should be pleased with the new version), Plasma Master File and epidemiology reporting. A report of the meeting is in preparation. PPTA raised some issues including the need to tidy up certain regulations that mistakenly require plasma for fractionation to be tested for Human T-Lymphotropic Virus I and II (HTLV 1 & 2). Additionally, PPTA staff stressed the urgent need to review the albumin guidelines in light of the new findings and questions surrounding the veracity of the work of Dr. Joachim Boldt, which promoted a starch based alternative to albumin. Finally, PPTA learned of a new 'Blood Cluster' harmonization initiative supported by both the U. S. Food and Drug Administration and the EMA and not including the industry.



## ▼ PPTA HOLDS CONGRESSIONAL STAFF BRIEFING

The Association, in collaboration with the patient community, hosted a Congressional staff briefing on March 31 focused on the uniqueness of plasma protein therapies and the importance of access. Presentations were made by patients including Lisa Miller, who has primary immunodeficiency; Michelle Rice, who has two sons with hemophilia and works with the National Hemophilia Foundation (NHF); and John Walsh, who has alpha-1 and also is the founder, president and CEO of the Alpha 1 Foundation.

Dr. Craig Kessler, a hematologist/oncologist with Georgetown University Hospital and chair of NHF's Medical and Scientific Advisory Council also presented at the briefing and described the challenges with treating hemophilia. Julie Birkofer of PPTA moderated the panel. Staff from a number of Congressional offices attended including Reps. Pitts (R-PA), Frank (D-MA), Kinzinger (R-IL), Biggert (R-IL), Chu (D-CA), Walberg (R-MI) and Israel (D-NY) among others. Visit [www.pptaglobal.org](http://www.pptaglobal.org) and read the news release or listen to the audio of the one-hour briefing.



**PPTA collaborated with patient groups to host a Congressional briefing in March that focused on the unique nature of plasma protein therapies and the need to preserve patient access. Pictured from left to right are: John Walsh, Dr. Craig Kessler, Michelle Rice and Lisa Miller.**

# Anti-Tetanus toxoid immunoglobulin quantification

The accurate determination of Tetanus toxoid immunoglobulin levels in human serum and plasma is important both in the manufacture of Tetanus Hyperimmune Globulin and in the diagnosis of Primary Immunodeficiency.

Binding Site understands that the testing requirements of therapeutic immunoglobulin manufacturers and clinical laboratories are very different and is pleased to offer assays optimised for the needs of each.

Application:	Primary Immuno- deficiency Diagnosis	Plasma donor unit Screening	Plasma donor unit Screening
<b>Platform:</b>	ELISA	ELISA	Turbidimetry*
<b>Measuring Range:</b>	0.01-7 IU/mL	1.23-300 IU/mL and 0.25-60 IU/mL	1.56-50 IU/mL
<b>Calibrated to:</b>	NIBSC Tetanus antitoxin reference preparation 76/589	NIBSC Tetanus Immunoglobulin, Human TE-3	NIBSC Tetanus Immunoglobulin, Human TE-3
<b>Test per kit:</b>	1 x 96 well plate	10 x 96 well plates	5 x 200 tests
<b>Product Description:</b>	Tetanus toxoid IgG EIA kit	Tetanus toxoid IgG EIA kit	Tetanus toxoid latex SPAPLUS kit
<b>Product Code:</b>	MK010	MK010.4	LK710.S



\* **SPA**  
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## ◀ 1099 TAX-REPORTING PROVISION REPEALED

On April 14, 2011, President Barack Obama signed into law the “Comprehensive 1099 Taxpayer Protection and Repayment Exchange Subsidy Overpayments Act of 2011.” For more than a year, PPTA strongly advocated for the passage of this new law, which strikes from 1099 tax reporting “amounts in consideration for property,” a provision added in health care reform. Because courts have held that plasma donations are considered “property” for the purpose of income taxes, repeal of the expansion of the 1099 reporting requirement is vital to the preservation of human plasma collection and is a victory for patient access. Go to the Newsroom on [www.pptaglobal.org](http://www.pptaglobal.org) to learn more.

## NDDR UPGRADED ▶

The National Donor Deferral Registry (NDDR) upgrade was rolled out on February 27, 2011. Enhancements such as Social Security Number encryption and software upgrades improve the donor screening process for all involved. The advanced system provides increased efficiency and functionality to the industry’s database of permanently deferred plasma donors in North America.



## ◀ NEW SPANISH LANGUAGE TRANSLATIONS AVAILABLE ON PPTA WEBSITES

PPTA recently updated the Association’s global website and its website on plasma donation with Spanish language translations of some materials posted online to better serve consumers. PPTA updated the websites to provide information about the plasma protein therapeutics industry to a Spanish-speaking audience and to provide information to a diverse audience. Users wishing to view the materials online in Spanish can now click on links provided “En Español” that will show the translation in the Spanish language of their current page. Information on plasma donation and lifesaving plasma protein therapies is now available as a resource to the Spanish-speaking community. To view the translated materials online, please go to [www.pptaglobal.org](http://www.pptaglobal.org) or [www.donatingplasma.org](http://www.donatingplasma.org).



## GLOSSARY OF TERMS

**ACA** Affordable Care Act

**A-PLUS** American Plasma Users Coalition

**CEA** Cost Effectiveness Analysis

**CER** Comparative Effectiveness Research

**CMS** Centers for Medicare and Medicaid Services

**CUA** Cost Utility Analysis

**CVID** Common Variable Immune Deficiency

**EDQM** European Directorate for the Quality of Medicines and HealthCare

**EMA** European Medicines Agency

**EU** European Union

**FDA** U.S. Food and Drug Administration

**GMP** Good Manufacturing Practices

**HHS** Department of Health and Human Services

**HTA** Health Technology Assessment

**IMAB** Independent Medicare Advisory Board

**IPPC** International Plasma Protein Congress

**IPOPI** International Patient Organisation for Primary Immunodeficiencies

**IPPIA** International Plasma Products Industry Association

**IRS** Internal Revenue Service

**ISICEM** Intensive Care and Emergency Medicine Congress

**NDDR** National Donor Deferral Registry

**NHF** National Hemophilia Foundation

**PhRMA** Pharmaceutical Research and Manufacturers of America

**PID** Primary Immunodeficiency

**PLUS** Plasma Users

**PPACA** Patient Protection and Affordable Care Act

**PPT** Plasma Protein Therapies

**QALY** Quality Adjusted Life Year

**QSEAL** Quality Standards of Excellence, Assurance and Leadership

# STAFF

## JAY GREISSING



### MY NAME IS JAY GREISSING.

I started working at PPTA in August 2006 as Director, Federal Affairs and have recently been promoted to Senior Director, Federal Affairs. Over the years, I have come to appreciate the challenges that manufacturers face in bringing therapeutic interventions to market to treat rare diseases, disorders, and conditions. These challenges are increasing rapidly in the post-health care reform world in which the states are bankrupt and reducing health care expenditures, especially for Medicare Part B, continues to be a priority for Congress. I have several responsibilities including managing the Federal Affairs Steering Committee, but my primary role is to lead the federal affairs team in my dual capacity of handling health policy issues with several Federal agencies and legislative issues before Congress.

### Tell us about your background.

I was born in Washington, D.C. and I am a seventh generation Washingtonian. I am the oldest of seven children, six of whom are boys. I grew up nearby in Falls Church, Virginia and attended school at Saint Albans School for Boys in the District, where I won the Coach's Award in both football and basketball my senior year. During school, I developed a passion for oil painting, and began studying Japanese. Upon graduating from Saint Albans, I attended the University of Virginia where I majored in Asian Studies. After working in government affairs and public relations for two years following my graduation, I moved to New York to attend Fordham University School of Law. After sitting for the bar exam, I moved back to the Washington, D.C. area to begin working on the U.S. Senate Committee on the Judiciary for Chairman Orrin G. Hatch (R-UT).

At present I live in Washington, D.C. with my wife Kim and our two children, Maggie and Jack. In my spare time, I enjoy coaching my daughter in soccer and lacrosse, attending a wide variety of sporting events, and attending heavy metal music concerts.

### What is your proudest professional achievement?

In my nearly five years at PPTA, I achieved significant victories at both the Centers for Medicare & Medicaid Services and on Capitol Hill. For example, during the health care reform debate, I successfully worked with the staff of the Senate Committee on Finance to limit the Medicaid outpatient drug rebate percentage increase for the entire therapeutic class of blood clotting factors to only 17.1 percent, while the rest of the branded drug industry was increased to 23.1 percent. Additionally, I worked with Sen. Hatch to include in the new law a Government Accountability Office study of the 340B Drug Pricing Program. My proudest moment, however, was probably when the Finance Committee staff included language that I developed to require the new Patient Centered Outcomes Research Institute to convene a rare disease advisory panel on each occasion that a rare disease, disorder, or condition is considered for comparative effectiveness research.

### What is most rewarding about working in this industry?

The manufacturers we represent produce lifesaving therapies for patient populations who rely heavily

upon them. I have two younger brothers with mild hemophilia A. Although they only require treatment before and after surgical procedures, I now have a much greater appreciation for their experiences and the patient community as a whole after my time at PPTA.





# EVENTS

## UPCOMING CONFERENCES & SYMPOSIUMS

# 2011

<b>June 3 – 5</b>	College of Intensive Care Medicine of Australia and New Zealand (CICM) <i>Canberra, Australia</i>
<b>June 9 – 12</b>	16 <sup>th</sup> Congress of the European Hematology Association <i>London, United Kingdom</i>
<b>June 14 – 15</b>	<b>Plasma Protein Forum</b> <i>Reston, Virginia, United States</i>
<b>June 18 – 22</b>	XXI <sup>st</sup> International Congress of the International Society of Blood Transfusion (ISBT) <i>Lisbon, Portugal</i>
<b>July 23 – 28</b>	XXIII International Society on Thrombosis and Haemostasis <i>Kyoto, Japan</i>
<b>October 1 – 10</b>	24 <sup>th</sup> European Society of Intensive Care Medicine Annual Congress <i>Berlin, Germany</i>
<b>October 7 – 9</b>	European Haemophilia Consortium Conference <i>Budapest, Hungary</i>
<b>October 22 – 25</b>	AABB Annual Meeting <i>San Diego, California, United States</i>

<b>October 23</b>	<b>Source Business Forum</b> <i>San Diego, California, United States</i>
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<b>November 20 – 23</b>	XXII Regional Congress of the ISBT, Asia <i>Taipei, Taiwan</i>
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# 2012

<b>March 13 – 14</b>	<b>International Plasma Protein Congress (IPPC)</b> <i>Madrid, Spain</i>
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<b>March 20 – 23</b>	32 <sup>nd</sup> International Symposium on Intensive Care and Emergency Medicine <i>Brussels, Belgium</i>
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<b>July 7 – 12</b>	XXXI <sup>nd</sup> International Congress of the ISBT <i>Cancun, Mexico</i>
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<b>July 8 – 12</b>	World Federation of Hemophilia, World Congress <i>Paris, France</i>
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<b>October 3 – 6</b>	XV <sup>th</sup> Biennial Meeting of the European Society for Immunodeficiencies (ESID) <i>Florence, Italy</i>
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