

THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

Spring 2011

Sailing to New Heights in Industry Standards

**IQPP: A Long-Term Program
Consistently Providing
Membership Value**

**The Importance of
PPTA Stakeholder Meetings
in the U.S.**

**PPTA Brings Value to Members
through Critical Meetings
with FDA and EMA**





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

CONTINUED CHALLENGES OF DOING BUSINESS IN JAPAN

IN THE PREVIOUS EDITION OF THE SOURCE I wrote about the challenges of doing business in Japan. One of the issues that I mentioned is the Japanese labeling requirement. Manufacturers of plasma protein therapies are required to print on the label whether the origin of the starting material is “Kenketsu” or “Hikenketsu.” In the column I wrote that Kenketsu applies to therapies manufactured by domestic manufacturers and Hikenketsu to therapies manufactured by foreign manufacturers. The Japanese Ministry of Health, Labour and Welfare (MHLW) communicated their displeasure with this and requested that PPTA print a correction. According to the email from the MHLW, the correct translation is:

Kenketsu : Voluntary, non-remunerated donation

Hikenketsu: Not voluntary, non-remunerated donation

I am happy to print the correction but must say that it is very confusing now. The etymology of the words kenketsu and hikenketsu are really not important. The basis for my argument is the artificial differentiation between two safe and effective therapies resulting in reduced demand for one of them. Does someone really believe that our members are using donors who donate against their will? That is far from the truth. The vision of “non voluntary” donors being chased while their plasma is forcefully “donated” is more than a little farcical. Seriously, however, I continue to believe that there should be no labeling requirement at all, since it can be used to discourage the import of safe and effective therapies to Japanese patients.

MHLW has set up a panel to make recommendations about the future supply of plasma protein therapies in Japan. PPTA was invited to address the panel on February 7.

We prepared a talk about the differences that we see in Japan and other parts of the world in regard to plasma collection and the use of therapies. In our presentation we addressed several challenges in Japan, such as:

- No increase in plasma for fractionation for many years
- Immune globulin consumption has been stable for decades, unlike the rest of the world
- Primary immune deficiency in Japan is under diagnosed
- Self sufficiency policy has had a negative impact on access to therapies
- Limited scale of economics for domestic manufacturers
- Discouragement of innovation for foreign manufacturers
- Plasma protein therapies are used by small patient populations

- Low number of repeat donors in Japan = high dependence on new donors
- Limited collection of hyper-immune plasma in Japan
- Availability of hyper-immunes is limited
- Anticytomegalovirus (Anti-CMV) Immunoglobulin is not available at all
- Anti-Zoster not available at all
- Current Yakka system does not work for plasma protein therapies

Even though we were scheduled for a 20 minute presentation including question and answer session, the panel wanted to discuss a wide variety of issues and the session lasted an hour. The questions ranged from basic information about plasma collection practices (recovered and source) to specific questions about clinical studies on albumin and albumin usage. Perhaps of greatest interest to members of the Panel were answers to questions relating to the global increase in consumption and usage of intravenous immune globulin (IVIG). During our presentation, we demonstrated that not only was Japan’s IVIG consumption below average for the rest of the developed world, but also showed that it is one of the very few countries that have not experienced an increase in usage over the past 20 years. We explained to the Panel that this is contrary to most of the IVIG using countries of the world where the use of IVIG has been steadily increasing due to recognition of its clinical benefits in terms of labeled indication, and the increase in the awareness and diagnosis of diseases that are treatable by IVIG. The final question asked of PPTA was the status of self-sufficiency policies as a global matter of policy. We responded by re-affirming the importance of local supplies of whole blood and transfusable components, but also noted that we as an industry are committed to global self-sufficiency and patient access to all safe and effective therapies. We are grateful that there are countries with committed donors who all donate voluntarily allowing us to manufacture therapies that can be used by patients all over the world including Japan.

We believe this meeting was successful in bringing our points of view across and we are looking forward to further constructive dialogue with MHLW with the goal of bringing more therapies to the patients who need them.



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CRITICAL INDUSTRY MEETING BRINGS TOGETHER STAKEHOLDERS

BY CHARLES WALLER

SINCE ITS EARLY INCARNATIONS

in the 1990s, the International Plasma Protein Congress (IPPC) has grown into an important part of the international plasma protein industry's annual calendar. The IPPC has evolved through the years and is a high-value conference that should not be missed.

A major source of interest flows from the diversity of the delegates. Patients and their representatives, regulators and politicians are encouraged to attend through attractive reduced fees and many are welcomed as PPTA's guests.

PPTA was founded on the basis of openness and honesty in the belief that good decisions, regulations and policies will flow from good and reliable information.

The old situation of limited discussion, uninformed debate and dogmatic adherence to traditional views was responsible for some truly bad laws and regulations that even today continue to waste time and resources. In a world challenged by limited resources waste in pursuit of political dogma must be rejected.

IPPC has played an important role in many ways. We have been able to move "log jammed" issues, throw light on new and emerging issues, hear from the chief executives, and other opinion leaders from the four corners of the world.

The number of business meetings that are ancillary

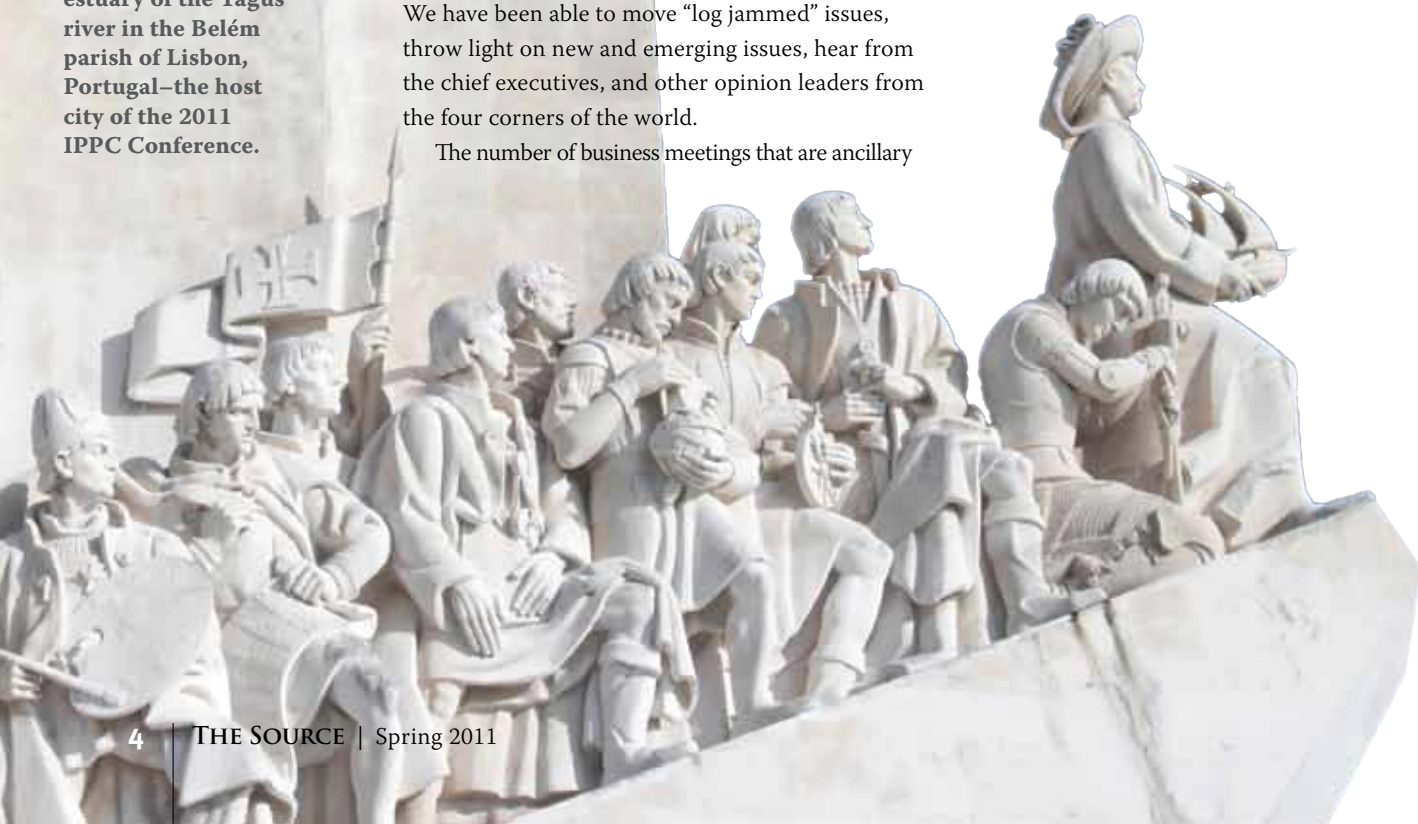
to the event itself indicates the increased importance and influence of the event. The fact that numerous leaders throughout all sectors of the industry and stakeholders make time to attend this meeting affirms the value.

Each year around 300 people attend. They are the industry stakeholders in the plasma protein industry and their continuing engagement remains at the heart of what makes the IPPC so important to PPTA.

IPPC creates a stimulating environment where topics at the forefront of the patient access agenda are discussed. PPTA strives to develop an agenda rich in new information about the latest scientific developments and technologies impacting this unique biotech sector. This year's IPPC again will deliver timely messages relevant to all stakeholders within the global community. If you want to meet the international stakeholders in the plasma industry then there really is only one place where you have to be. ☞

CHARLES WALLER is PPTA's Vice President, PPTA Europe

Shown here: Padrão dos Descobrimentos (Monument to the Discoveries) is a monument that celebrates the Portuguese who took part in the Age of Discovery. It is located on the estuary of the Tagus river in the Belém parish of Lisbon, Portugal—the host city of the 2011 IPPC Conference.



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PLUS GATHERS FOR SECOND ANNUAL MEETING *in Dublin, Ireland*

BY CHARLES WALLER

PPTA PARTICIPATED IN THE 2ND MEETING ORGANIZED BY THE PLASMA USERS GROUP (PLUS)

in Dublin, Ireland. The purpose of the meeting was to develop a set of principles on plasma donation from the point of view of patient organizations and PLUS was eager to continue the “constructive dialogue” that commenced in 2010.

The 2011 draft consensus statement recognizes the importance and “essential” nature of remunerated plasma donations in maintaining supply. The new draft paper was developed for assessment by the participating Associations, including PPTA. It is planned that when, and if approved by the supporting associations and industry groups, it will be published in *Vox Sanguinis* later this year. The draft statements reflect the diverse nature of the participating groups.

Unlike in 2010, all participants agreed that the new draft statement represents a text that participants are prepared to recommend to their respective organizations.

PPTA endorsed the need to ensure that patients are an integral part of political and regulatory decision-making related to plasma protein therapeutics, especially on issues concerning the sustained supply of plasma protein therapies.

The discussions in Dublin in January 2011 raised a number of issues that were of concern to patients and other involved stakeholders alike. One key concern of PLUS related to the need to facilitate the



utilization of all high-quality plasma. Patients expressed concern that regulations in many countries currently do not permit the processing of imported plasma and there was unanimous support for regulation supporting safe manufacture. There was also agreement that further discussions are needed to consider how regulation can better support an increased global supply of safe plasma-derived medicinal products.

Issues that delegates had differing views on included the value and practicality of self-sufficiency and the potential impact on supply due to the presence of two independent collection systems, one for blood and one for plasma, in a country.

PLUS intends to organize a further meeting in 2012 to encourage more detailed discussion on the issues of concern to the patient community and to bring a small group of stakeholders, 20 invited guests together, to discuss possible solutions in a spirit of cooperation.

CHARLES WALLER is PPTA's Vice President, PPTA Europe

THOSE *Attending* INCLUDED

PLUS MEMBERS

Brian O'Mahony (Convenor) European Haemophilia Consortium (EHC) and Irish Haemophilia Society

David Watters International patient Organisation for Primary Immunodeficiencies (IPOPI)

Larry Warren and **Johan Prevot** IPOPI

Uwe Schlenkrich European Haemophilia Consortium (EHC)

David Page World Federation of Hemophilia

OTHER PARTICIPANTS AND THE ORGANIZATION REPRESENTED

Mark Skinner A-PLUS and World Federation of Hemophilia (WFH)

Charles Waller and **Albert Farrugia** PPTA

Bob Perry and **Theo Evers** The International Plasma Fractionation Association (IPFA)

Roger Dodd International Society of Blood Transfusion (ISBT)

Niels Mikkelsen International Federation of Blood Donor Organizations (IFBDO)

Jim MacPherson America's Blood Centers

Alison Turner National Blood Authority, Australia

Patrick Robert Marketing Research Bureau

Cees van der Poel European Directorate for the Quality of Medicines and HealthCare (EDQM)

Paul Strengers ISBT

Gilles Follea European Blood Alliance (EBA)



The Dunboyne Castle, Duboyne, Ireland.
The PLUS meeting location.

PPTA Brings Value to Members Through **CRITICAL MEETINGS WITH FDA AND EMA** on Regulatory Issues

BY ALBERT FARRUGIA, ILKA VON HOEGEN, AND MARY GUSTAFSON

PPTA IS COMMITTED TO STRONG REGULATORY OVERSIGHT OF PLASMA PROTEIN THERAPIES.

The world's foremost regulatory agencies in the United States, Europe, Japan and Australia regulate PPTA member companies' therapies provided in those areas.

Therefore, the Association's interaction with these agencies is crucial to the industry.

The Association presents issues of common concern for the industry to these bodies and works to resolve problems and seek reasonable regulatory policies in the interests of patients, industry and agencies.

This work is accomplished in several ways:

1 PPTA has annual liaison meetings with both the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA). These meetings provide an opportunity for both the industry and the Agencies to discuss various topics of concern for the purpose of clarifying issues and seeking a common understanding. Meetings have also been held with the Japanese Ministry of Health Labour and Welfare and the Australian Therapeutic Goods Administration. These annual meetings are generally judged valuable by both the industry and the regulators; views have been exchanged and issues of common interest have advanced towards resolution.

Recent meetings have included the 2010 FDA/PPTA Liaison meeting in October 2010, where the FDA's draft Guidance on Process Validation was discussed. The industry elicited the Agency's agreement that timely release

of the final Guidance was essential to facilitate planning in manufacture, and this was reflected in the final Guidance's publication January 2011. Having the final guidance allows member companies to undertake new projects with increased predictability.

In Europe, the PPTA has engaged recently with the EMA on the proposed Annex on plasma-derived protein manufacture to the European code for Good Manufacturing Practice ("Annex 14"). This important Annex recognizes the unique nature of plasma protein therapies but, as initially proposed, would restrict considerably the capacity of the industry to perform contract manufacture for countries outside the European Union. This would impair access to therapy for patients in countries unable to manufacture plasma protein therapies, particularly emerging countries, an issue of interest to the World Health Organization (WHO) given the commitment of the 63rd World Health Assembly to making plasma for fractionation available for all countries. As a result of productive

discussions between the involved parties, it was decided in October 2010 that the draft Annex should progress without making any statement on import criteria for plasma fractionation programs for third countries. PPTA remains committed to introducing appropriate wording into the Annex 14, and continues its interactions with the European Commission to this end.

2 PPTA presents for the industry in regulatory agencies' meetings and workshops. Depending on the scenario, PPTA may provide a statement, an oral presentation on policy or a combined scientific presentation of data derived from member company studies. Some examples of past meeting participation include EMA workshops on epidemiology and FDA advisory committee meetings addressing concerns about variant Creutzfeldt Jakob disease and West Nile Virus, where the Association's collated data was pivotal in contributing to FDA's decision to exempt

source plasma for manufacture from the need to be screened for this agent.

3 PPTA writes letters to regulators presenting industry comments to proposed regulations and guidance documents and also to address specific regulatory concerns. Providing comment letters to agencies requires monitoring of Agency document releases and coordinating responses with the PPTA regulatory steering committees and task forces. In this context, interaction with PPTA's albumin task force in Europe has elicited from the EMA that the Agency considers that the basic volume expansion indication covers much of the usage relating to indications such as hepatology and severe sepsis. This is considered to be a very positive development by the Association as it widens the

scope of albumin usage considerably. PPTA has also engaged with a number of international regulators to heighten awareness regarding the risks posed by hydroxyethyl starches (HES) and the implications of recent findings of scientific fraud involving these products. This has been followed by the German Physician Association's withdrawal of its current albumin usage guidelines, admitting their lack of credibility as their author has been found to be guilty of scientific fraud. The Association committed to consulting the PPTA in a new iteration.

4 Sometimes PPTA will request an ad hoc meeting with regulators to resolve problems that are either too urgent or too complicated to address in correspondence. Recently, a proposed infectious disease standard for biological source materials was proposed by the

Australian TGA which, if implemented in its original state, would have severely limited access to plasma protein therapies in Australia. The Association's representatives held a meeting with the Administration that led to a considerable modification of the standard and adequate consideration of the special features of plasma for fractionation.

PPTA will continue its collegial and productive interaction with regulators. *"Let us never negotiate out of fear. But let us never fear to negotiate."*

— John F. Kennedy, 1961

ALBERT FARRUGIA is PPTA's Vice President, Global Access, ILKA VON HOEGEN is PPTA Europe's Senior Director, Quality and Safety and MARY GUSTAFSON is PPTA's Vice President, Regulatory Policy



**"Let us never
negotiate
out of fear.
But let us
never fear
to negotiate."**

— John F. Kennedy, 1961



A LONG-TERM PROGRAM CONSISTENTLY PROVIDING MEMBERSHIP VALUE

BY JOSHUA PENROD

THE INTERNATIONAL QUALITY PLASMA PROGRAM (IQPP)

has been in place since the early 1990s, formed of the plasma collection industry's desire to demonstrate an active commitment to safety and quality of source plasma. During the first decade of its existence, the industry added several important components to the program, generating, in large part, the bulk of the standards known and understood around the world by the industry, patients, and regulators to be a symbol of the industry's commitment to improvement and enhancement.

In recent years, the IQPP has been reviewed, studied, and optimized with the changes in technology and needs of the industry and its stakeholders. The industry added the Cross Donation Management Standard in 2010, which helps to prevent over-donation, enhancing the levels of confidence in both the plasma collection facility and in the donor.

Currently, the IQPP consists of nine individual standards, all devoted toward the safety of the product, the safety of the donor, and the ultimate quality of the plasma collection facility. Audits are performed by unbiased, third-party auditors who contract with PPTA to perform the audits; all auditors have many years of experience and are

certified to perform quality audits. Audits are also performed on a regular basis and plasma centers are scored according to their performance on the audit. Similarly, IQPP audits are performed on corporate headquarters of PPTA member companies subscribing to the program as well; this helps generate a complete picture for review.

The Standards which make up the body of the IQPP have been covered elsewhere in recent articles and other publications, so we won't recount them in detail here. Instead, we'd invite the reader to examine the IQPP first hand, at www.pptaglobal.org/standards. For convenience, however, the nine Standards currently part of IQPP are:



- | | |
|--------------------------------------|---|
| 1 Community Based Donor Standard | 6 Use of the NDDR Standard |
| 2 Qualified Donor Standard | 7 Personnel Education and Training Standard |
| 3 Viral Marker Standard | 8 Professional Plasma Facility Standard |
| 4 Donor Education Standard | 9 Quality Assurance Standard |
| 5 Cross-Donation Management Standard | |

Value to Patients

IQPP's value to the patients takes many forms and has changed over the years of its existence. What remains the same to the patient communities is the assurance and confidence that IQPP is there as a central part of the industry's commitment to assuring patient well-being. This assurance and confidence is well-founded and longstanding.

In dialogue with PPTA, many patient group representatives have emphasized the importance they place on IQPP. With regular updates to stakeholders, PPTA and members of the industry educate the community about the IQPP program and its benefits. On several occasions in the recent past, PPTA, its members, and the stakeholder groups have used the IQPP as a common ground of conversation to discuss pressing issues involving all parties. These included discussions related to viral marker rates and epidemiology, donor recruitment practices, and the quality and safety of donors.

The IQPP, in its totality, represents a separate commitment by the industry to the patient community for ongoing efforts at improvement in the quality and safety of collected plasma. IQPP fits with the regulatory networks and the methods and practices of the companies to help contribute to this enhanced margin and safety and the confidence expressed by the users of these life-saving therapies.


Value to Regulators and Policymakers

PPTA has presented the IQPP to regulatory authorities on several occasions, in Asia, Europe, and the United States Food and Drug Administration. The IQPP frequently serves as a model for thinking through issues of safety and the correct, most effective approach to resolve questions involving the practice of plasma collection and regulatory concerns. The plasma industry efforts in standards have also received recognition by regulatory authorities, such as demonstrated in Europe in 2002, in a CPMP position statement that discussed the safety and quality of plasma collected from both remunerated and non-remunerated sources. The statement noted: "For plasmapheresis donors . . . there are additional voluntary standards to ensure that plasma originates from a low risk donor population. These include qualified donor programs and inventory hold of donations." (See EMEA/CPMP/BWP/1818/02/Final)

The long-term impact of IQPP as it fosters a greater appreciation among policymakers and regulatory authorities is clear. Many of the advocacy efforts advanced by the Association to legislators and legislative staffs depend on the showing of the quality and safety for the products, their uniqueness and their high-impact value. IQPP bolsters the high individualized and specialized nature of the industry, the products, and the patients who use them.

Value to Industry

IQPP continues to pay dividends of goodwill and confidence on the part of the industry with each passing day. Examining the ways in which the program has manifested value time and again during its history underscores the discussions involving the assurances given to the regulatory authorities, the patient groups, and the public. But another important component showing value to the industry, regulators, and patient groups is the benefit shown to the donors with the IQPP. The Donor Education Standard works in conjunction with regulatory requirements to inform donors about risky behavior, offering them a chance not only to choose not to donate, but also to have a better understanding about what relates to risk. In addition, the recent Cross-Donation Management Standard helps facilities ensure that that donors donate within the safe limits of regulatory requirements. Companies use this in conjunction with their own educational efforts to communicate with donors regarding donation practices and ensuring that the donation experience remains a fulfilling one.

All of these components of IQPP, along with sound regulatory structures and industry practice and commitment to quality, help provide assurance to patients, donors, regulators, policymakers, the industry, and the public at large that the plasma collected and therapies created are as safe and high quality as possible. 

JOSHUA PENROD is
PPTA's Vice President, Source

BY ILKA VON HOEGEN

CROSS-DIMENSIONAL ALLIANCES

Working Together: A European Perspective



A GLANCE AT PPTA CORRESPONDANCE OR PROMOTIONAL MATERIALS

reveals the focus of this Association and the medicinal products produced by its members: biological lifesaving therapies either recombinant or fractionated from a very unique starting material—human plasma. In essence, what PPTA represents is not "Big Pharma," but a very specific niche sector serving patients with rare diseases. To further highlight their uniqueness these plasma protein therapies are exempted from biological products regulations from the European Medicines Agency (EMA) and World Health Organisation (WHO) biosimilars guidelines.

On the other hand, plasma protein therapies also fall under the scope of broader pharmaceutical legislation applicable to all medicinal products with respect to marketing authorization and changes to these, which are regulated in the European Union (EU) Variations regulation. How can such a small sector influence these overarching provisions in the overall rumble of "Big Pharma" influence?

One has to look at the issue from a different angle. Market authorization of a medicinal product and maintenance throughout its life cycle are subject to the same procedures for all medicinal products. It is obvious that manufacturers have common interests: efficient procedures of market access and maintenance at affordable costs. The revision of the EU Variations Regulations in 2008 to establish a simpler, clearer and more flexible legal framework, while guaranteeing the same level of public and animal health protection was of particular interest to the manufacturers of biological medicinal products, i.e. recombinant and plasma-derived, because this product class has never enjoyed the pragmatic approach applied to classical pharmaceuticals. Any variation to a Marketing Authorization automatically triggered a Type II variation and it appears questionable, whether such an expensive and time consuming procedure is always justified, particularly for minor changes without any impact on the quality, safety or efficacy of the final product.


As per the initiative of PPTA since 2004, a number of Associations representing biological manufacturers such as EuropaBio, International Plasma Fractionation Association (IPFA), European Vaccine Manufacturers (EVM) and others formed an alliance

to promote their objective for a simpler, clearer and more flexible legal framework to reduce the regulatory hurdles for biological products where appropriate. Due to the EU Commission's precautionary attitude towards biological medicinal products these hopes were not fulfilled: At a specific meeting on biological products between the associations, EU Commission and EMA in the course of the public consultation period for the new Variations regulation it was regulators remained firm in their belief that the level of experience with biological products is not sufficient to permit a relaxation of regulatory surveillance of these products. But would there at least be a reduction in the fees payable for variations procedures?

In times with budget restrictions in all areas of the public sector an institution that is dependent on the support from national authorities and independent experts such as the EMA is faced with particular challenges to keep their performance and expertise according to the state-of-the-art. At the EMA workshop "Outcome of the Evaluation of the EMA, the Future Sustainability of the System" EMA representatives informed attending pharmaceutical manufacturers that the balance between fees and the level of service is no longer provided. Bottom line, prices are going up, but by 400%? Such a significant increase was experienced by one PPTA member company when they filed a group of variations to the Plasma Master File (PMF). Instead of the expected invoice of 57,200 Euros the company was asked to pay 237,900 Euros, because EMA charged each variation in the group separately, which was previously not the case. This change in procedure was not

announced and thus took manufacturers completely by surprise. PPTA immediately contacted the other associations and together a very strong complaint was submitted to the EU Commission and other relevant institutions and committees. Subsequently, PPTA and EuropaBio met EU Commission and EMA representatives for more in depth discussions.


In October 2010, the EMA published amendments to the Management Board implementing rules on the Agency's fees in relation to extensions of marketing authorization, Type II variations, annual fees, variations to the PMF and Vaccines Antigen Master File (VAMF). For PMF and VAMF the introduction of a maximum fee of 57,000 Euros for a group of variations (containing at least one Type II variation) is a great achievement, which will facilitate the filing of Type II variations to a PMF between annual updates. Since the VAMF is not a procedure that is used by vaccine manufacturers, PPTA was the only group that benefited from this particular amendment. Some other outcomes of the revision are also notable, such as the grouping and work sharing procedures, where now the fee for the third variation is reduced from 57,200 Euros to 19,100 Euros.

This example shows how a constructive bundling of forces between the different representations of pharmaceutical manufacturers enables small sectors such as the one represented by PPTA to be heard and to influence regulatory decision making processes. 

ILKA VON HOEGEN is PPTA Europe's Senior Director, Quality and Safety

THE IMPORTANCE OF STAKEHOLDER MEETINGS IN THE U.S.

PPTA recognizes the importance of working with Stakeholders. But, what does this really mean? How do we “work” with Stakeholders and what are they? Let’s take a look.



ACCORDING TO MERRIAM WEBSTER’S DICTIONARY, the word stakeholder dates back to 1708 and is associated with a person who is “entrusted with the stakes of bettors.” Now, this strict definition is interesting because of its use of the word “entrusted.” As we know, trust is a basic building block of which most successful relationships are built. I think it is important to broaden how the term is used today in the context of an advocacy. After an Internet search, I came across an article titled, “Identifying health care stakeholders: a key to strategic implementation.” The Abstract from this article states:

“As the number and complexity of stakeholders for health care organizations has increased, health care managers have become more aware of the ability of these groups to thwart or facilitate the implementation of strategic plans. Most stakeholder models have focused on identification of groups within the usual, global definition of affecting or being affected by an organization’s actions. The authors argue that stakeholders management is critical to the implementation of strategic plans... The situational evaluation of stakeholders is critical to successful implementation of strategy.”

The above definition reflects the purpose of PPTA’s outreach to stakeholders. Since the early 1990’s, PPTA has each year regularly convened with consumer organization representatives, regulatory agency representatives, representatives from the U.S. Department of Health and Human Services, industry experts and Association staff. Central to our legislative and regulatory successes are developing unifying action plans for common goals such as patient access to plasma protein therapies and alignment on advocacy issues. The single largest benefactor of Stakeholder Meetings are the patients who rely on life-sustaining plasma protein therapies. Stakeholder meetings help PPTA establish rapport with patient organizations to better understand the concerns of the patient community. PPTA can help individuals identify state legislative priorities and how these priorities affect strategic issues of patient access, while explaining important safety developments and industry standards programs.

Stakeholders are mission critical to PPTA

Two elements of the Association’s Mission Statement are: “Educating all stakeholders about the value of the therapies” and “Supporting government reimbursement practices that reflect the unique nature of plasma protein therapies.” These two provisions are the foundation of the Association’s state and federal based advocacy programs. PPTA has a wealth of information available on its web site, www.pptaglobal.org. Click *Patient Resources* and it will bring you to a page with a menu of options applicable to both the U.S. and Europe. There are several items of interest posted, such as comprehensive Fact Sheets and the informative “Plasma Protein Therapies Stakeholder Toolkit.” This informative advocacy tool is chock-full of documents that

can be used in visits with legislators and policymakers.

Stakeholder Meetings are important because they promote an understanding of where the Association's concerns and those of stakeholders align. Knowledge of this alignment allows us to coordinate messages to decision-makers. This is essential because decision-makers care more about the impact of public decisions on constituents than corporations. Therefore, Stakeholder Meetings are a vital component of our public advocacy strategy.

In the states, decision-makers are asked to balance their state budgets. Often this involves reductions in Medicaid spending. Limiting patent access to pharmaceuticals through various cost-containment strategies is a common technique for Medicaid savings.

When decision-makers are considering strategies that limit patient access to pharmaceuticals, it is important that the Association and our stakeholders educate the decision-makers with a similar message on the need for patient access to the medically appropriate plasma protein therapy because of high impact, lifesaving nature of these therapies. The Stakeholder Meetings allow for the necessary dialogue to prepare for these threats.

Coalitions Enhance Patient Access Priorities

Stakeholder meetings provide an important forum for industry

and patient advocacy organizations to learn more about each entity's legislative and regulatory priorities. The result is a coalition approach to address threats to patient access to care and to identify solutions with a shared voice. For instance, there are a number of provisions in the health care reform legislation representing positive steps for individuals with rare, chronic diseases treated with plasma protein therapies. Consumer organizations representing users of plasma protein therapies did an outstanding job working with Members of Congress to eliminate lifetime and annual limits on insurance benefits and to end the practice of denying coverage based on pre-existing conditions. PPTA came out in strong support of these initiatives and worked hard to secure the rare disease advisory panel in the comparative effectiveness research provision in health reform. These reforms will help all Americans access and maintain the medical care they need for future generations.

PPTA, its staff and members value the participation of stakeholders in these meetings and are committed to continuing to facilitate an open forum for patient groups and industry to address challenges with accessing plasma protein therapies and other critical issues important to patient health.

JULIE A. BIRKOFER is PPTA's Senior Vice President, PPTA North America



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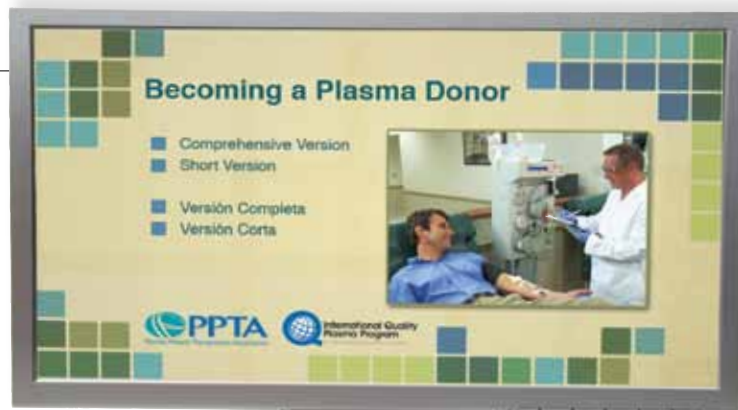
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INSIDE PPTA

PPTA NEWS FROM
AROUND THE GLOBE

PPTA RELEASES NEW VIDEO "BECOMING A PLASMA DONOR" ►

Earlier this year, PPTA released a new DVD "Becoming a Plasma Donor," which features information on plasma donation and what a donor can expect if they want to become a plasma donor at one of 400+ U.S. Food and Drug Administration-licensed and International Quality Plasma Program-certified plasma collection centers in the U.S. The ten minute video also discusses how critically important plasma donors are to the production of safe, effective, lifesaving therapies that help individuals with rare, chronic, life-threatening diseases. PPTA's DVD highlights how plasma is donated and what happens when someone arrives at a plasma collection center. Featured in the video is a plasma donor who takes viewers through the steps that someone would experience when they donate plasma. The DVD is available in English and with Spanish subtitles, and a German language version of the DVD filmed in Austria and Germany is also



expected to be available from PPTA later this year. To view the DVD online, please go to www.donatingplasma.org, select Newsroom and then Videos. To obtain a copy of the new DVD, please contact Diana Krueger at dkrueger@pptaglobal.org or (443) 433-1107.

▼ PPTA RECOGNIZES STAFF PERFORMANCE

The following staff were promoted in 2011 in recognition of their contributions to organizational excellence:

Congratulations!



Sybille Beck
Assistant Director
Source Europe
and Germany



Albert Farrugia
Vice President
Global Access



Mike McCormick
Director
IT & Facilities



Charon Smith
Manager
Accounts
Receivables/
Payables



Jay Greissing
Senior Director
Federal Affairs



John McKnight
Director
Federal Affairs



Bill Speir
Director
State Affairs



Kym Kilbourne
Director
Public Affairs
North America



Laura Savini
National Affairs
Manager



Alexa Wetzel
Assistant

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



* **SPA PLUS**

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www.bindingsite.com/spaplus
for more information.

Please enquire regarding FDA status.

www.bindingsite.com/manufacturers for more information



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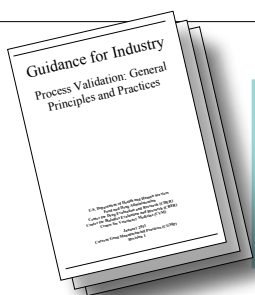
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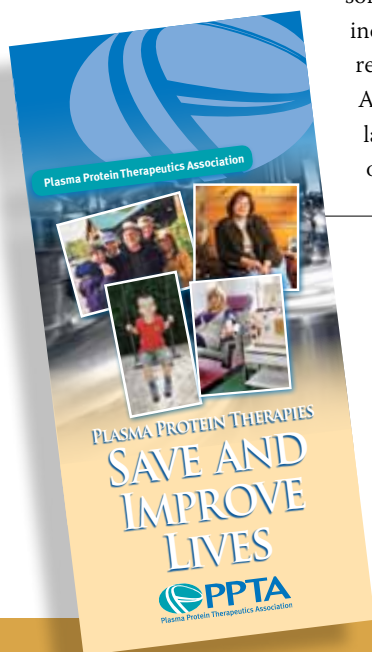
The Specialist Protein Company

INSIDE PPTA



▲ FDA PUBLISHES GUIDANCE

Recently, the U.S. Food and Drug Administration (FDA) published a Final Guidance titled “*Process Validation: General Principles and Practices*.” PPTA formally commented on the Draft Guidance, and it was discussed during last year’s FDA Liaison Meeting. FDA implemented some of PPTA’s suggested changes, including the addition of a glossary, references regarding Process Analytical Technology, and language regarding manufacturers of legacy products.



◀ UPDATED BROCHURE “SAVING AND IMPROVING LIVES” FOR EUROPEAN AUDIENCES

The Association recently updated the brochure “Saving and Improving Lives” for use with audiences in Europe. The 10-page brochure describes plasma protein therapies, their highly complex manufacturing process and voluntary industry standards. In addition, the life threatening chronic, genetic medical conditions treated with plasma protein therapies are highlighted with photos of real-life donors and patients. To obtain a copy of the brochure, please contact PPTA’s European office in Brussels, Belgium at +32.2.705.5811.



▼ GERMANY TO CONSULT PPTA IN DEVELOPMENT OF ALBUMIN GUIDELINES

PPTA’s approach to the German Physician Association (BAK), following the finding of scientific fraud by anti-albumin advocate Joachim Boldt, has been followed by the BAK’s withdrawal of the German albumin guidelines authored by Boldt. The BAK has written to PPTA, assuring that the Association will be consulted during the development of the next edition of the guidelines, which have become increasingly relevant and used by German reimbursement organizations.



GLOSSARY OF TERMS

CDC	Centers for Disease Control and Prevention
EMA	European Medicines Agency
EU	European Union
EVM	European Vaccines Manufacturers
FDA	U.S. Food and Drug Administration
HBIg	Hepatitis B Immune Globulin
HES	Hydroxyethyl Starches
HPSC	Health Policy Steering Committee
IPFA	International Plasma Fractionation Association

IPPC	International Plasma Protein Congress
MHLW	Ministry of Health, Labour and Welfare
IQPP	International Quality Plasma Program
PLUS	Plasma Users Group
PMF	Plasma Master File
TGA	Australian Therapeutic Goods Administration
VAMF	Vaccines Antigen Master File
WHO	World Health Organization

STAFF

LAURA SAVINI

MY NAME IS LAURA SAVINI.

I started working at PPTA in May 2009 as a National Affairs Assistant and I have recently been promoted to National Affairs Manager. Currently I have three main responsibilities, first of all I take care of PPTA European National groups, including Belgium, France, the United Kingdom and Greece. Work for these groups varies from long-term projects to flashpoints, so it is quite diversified and very interesting. Since the beginning of my tenure at PPTA I have been progressively assisting PPTA Europe Health Policy Directors, formerly Johan Prevot and now Siada El Ramly. This means being involved in the current projects of the Health Policy Steering Committee (HPSC) but also monitoring relevant European health policies and providing updates to the Committee. Finally, I also assist Kara Flynn, the Director of Global Communications in some of the European communications activities, such as the electronic newsletter, *Leader-*

ship Briefing, *The Source*, the *European Health Policy Update*, and other issues.

Tell us about your background.


I was born in Torino, Italy from an Italian father and Belgian mother. When I was 15 I moved to Brussels with my family and have been living here ever since. After high school, I enrolled in a secretarial school in Brussels. Following that, I felt I wanted to improve my English and live abroad for a while so I enrolled in a Master's program in Information Science in Wales, United Kingdom. This program lasted a year and focused on the different ways information is used (and most often underused) as a resource across one particular organization. My final dissertation was the analysis of a digital repository, at the time developed in the University where I was studying, aiming at storing academic papers and Ph.d students' thesis.

At present I live in Brussels with my boyfriend, Oliver. Mostly, I travel during my vacation time to visit my dispersed family: in fact, my brother and his family live in Thailand, my sister and her family live in Torino, plus Oliver is from Australia, so actually, there is a great deal of traveling! In my spare time I enjoy cooking and hiking.

What is your proudest professional achievement?

My proudest professional achievement was one of the first assignments I had starting at PPTA, which was the logistical organization of the 2009 planning meeting in Lucca, Italy. It was very gratifying planning a successful meeting in which the feedback was so positive from members.

What is most rewarding about working in this industry?

Knowing that in the end this business helps people with rare and life-threatening diseases. 



EVENTS

UPCOMING CONFERENCES & SYMPOSIUMS

2011

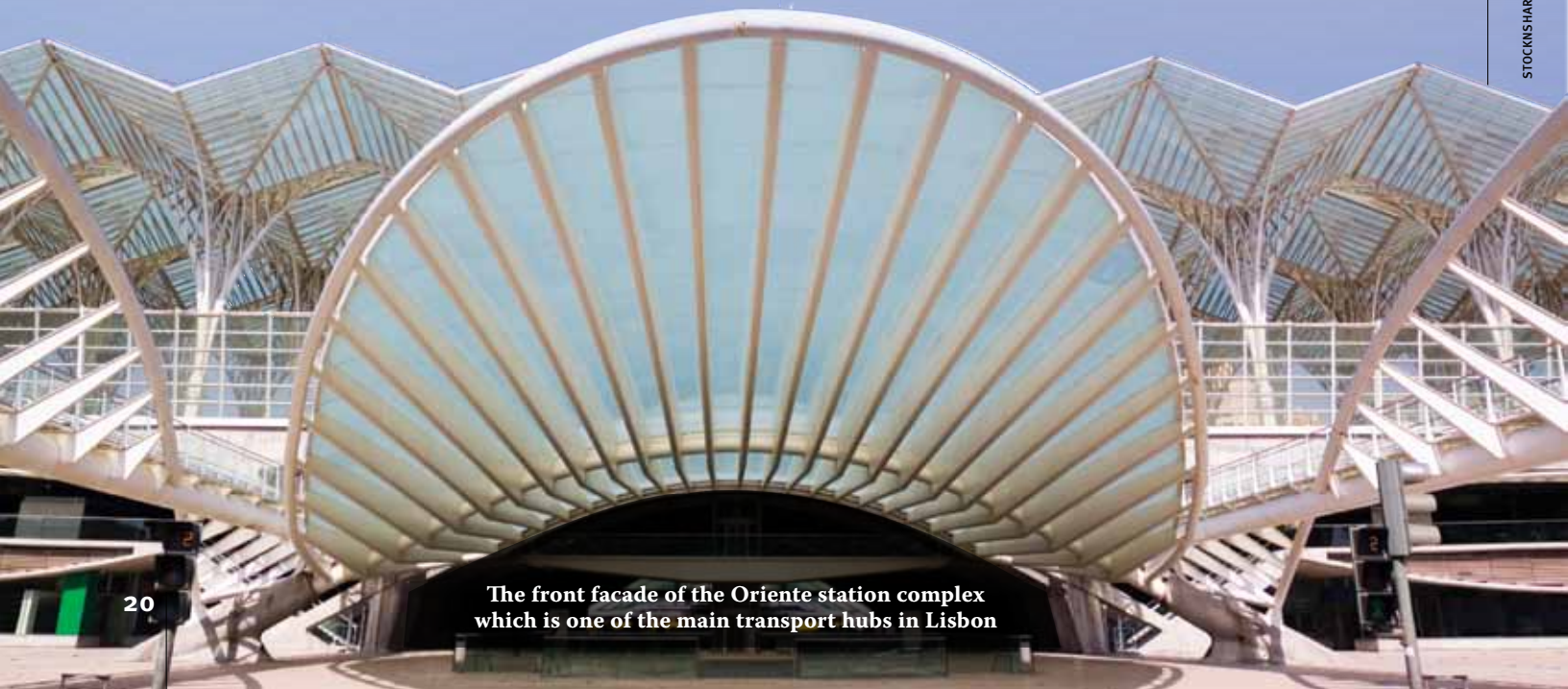
March 10 – 13	2nd African Society for Immuno-Deficiency Congress <i>Hammamet, Tunisia</i>
March 13 – 17	6th World Congress on Paediatric Critical Care <i>Sydney, Australia</i>
March 15 – 16	International Plasma Protein Congress <i>Lisbon, Portugal</i>
March 22 – 25	31th Symposium on Intensive Care and Emergency Medicine <i>Brussels, Belgium</i>
April 28 – 29	8th Annual Critical Care Symposium <i>Manchester, United Kingdom</i>
May 10 – 11	The 2nd International Congress on Transfusion Medicine-Plasma Industry <i>Tehran, Iran</i>
June 9 – 12	16th Congress of the European Hematology Association <i>London, United Kingdom</i>
June 14 – 15	Plasma Protein Forum <i>Reston, Virginia, United States</i>
June 18 – 22	XXIst International Congress of the International Society of Blood Transfusion (ISBT) <i>Lisbon, Portugal</i>

July 23 – 28	XXIII International Society on Thrombosis and Haemostasis <i>Kyoto, Japan</i>
October 1 – 10	24th European Society of Intensive Care Medicine Annual Congress <i>Berlin, Germany</i>
October 7 – 9	European Haemophilia Consortium Conference <i>Budapest, Hungary</i>
October 22 – 25	AABB Annual Meeting <i>San Diego, California, United States</i>
October 23	Source Business Forum <i>San Diego, California, United States</i>
November 20 – 23	XXII Regional Congress of the ISBT, Asia <i>Taipei, Taiwan</i>

2012

March 20 – 23	32nd International Symposium on Intensive Care and Emergency Medicine <i>Brussels, Belgium</i>
July 7 – 12	XXXIInd International Congress of the ISBT <i>Mexico City, Mexico</i>

SPECIAL OFFER: Readers that can identify the location of the bridge on the cover of this edition of *The Source* will receive 10% off registration fees for the upcoming Plasma Protein Forum to be held June 14-15 in Reston, Virginia, USA. Please provide your answer via email to Kara Flynn, PPTA at kflynn@pptaglobal.org by March 30, 2011.



The front facade of the Oriente station complex which is one of the main transport hubs in Lisbon



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