

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

WINTER 2013



Donors Making a Difference in People's Lives

Value of DHQ Implementation
to Donors & Industry

IPAW Report

Impact of German Elections
on Reimbursement



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In My View

BY JAN M. BULT, PRESIDENT AND CEO

Every Saturday I read with great interest a Dutch newspaper column written by Professor Bob Smalhout, a professor of anesthesiology in The Netherlands. He writes about various topics affecting the Dutch Society with wit and his own brand of wisdom. His past subjects have dealt with politics, finances, medical issues, human interest or anything else that Professor Smalhout considers relevant to the readers.

In 1972, he was ostracized by the medical community when he spoke in his inaugural speech about patients in the Netherlands dying needlessly during or after surgery because of medical errors. This year Professor Smalhout was (finally) professionally recognized by the Dutch Society of Anesthesiologists and awarded an honorary membership.

His popular weekly columns gained recognition for exposing mistakes and misconduct among the high and low in Dutch society. Long recognized for his ability to take the moral high ground and 'tell it like it is' stance on controversial issues, Professor Smalhout has earned the admiration of many readers.

He recently wrote about the tragic suicide of a general practitioner in The Netherlands who was suspected of medical malpractice when dealing with the very difficult situation with a patient suffering from terminal esophageal cancer. The entire medical information became public without respect for the wishes of the patient and his family. The inhabitants of the small village where the physician lived and his family were in shock after this "attack" on someone who had served the community so well for more than 2 decades. Professor Smalhout very clearly expressed his opinion about this terrible event in his column.

The issues he writes about can be very different. He criticized an UN initiative to terminate an old Dutch tradition for children in December where Sinterklaas (Santa Claus) brings gifts to all children with the help of his aides on December 5th. I have witnessed this tradition as a child and adult and I have seen enormous smiles on the faces of all children who welcome Sinterklaas on his white horse in the

country in the midst of November. From that moment on until December 5th, the children put carrots in their shoes and place them in front of the chimney so that the aides can come through the chimney, take the carrots for the horse and leave small presents for the children as a Thank You. Within hours after the announcement of the UN initiative, millions of people supported a petition to keep this tradition alive but it seems not to affect the opinion of the UN initiative taker, someone who has never set foot on Dutch soil.


Now you may ask yourself what these stories have to do with our field of expertise of patients with (ultra) rare diseases treated with plasma protein therapies. Well here is the parallel I see.

Professor Smalhout makes the point that in many cases decisions are made and opinions expressed by individuals who do not have the benefit of full knowledge of the subject matter. And that is the same for us. Many times we see statements that seem to be driven by politics and emotions rather than science. This happens on all levels. We sometimes see statements from WHO, National governments, Parliamentarians, Policy makers and others that create more hurdles to Patient Access to care. We are still dealing with endless debates about compensation and volunteerism for donors, whereas the true debate needs to be how to ensure that all patients have access to care in all sites of service.

I have always respected Professor Smalhout for his directness and addressing critical issues. It doesn't mean that I always agree with all of his statements, but his approach of being forthright and honest is something that I admire.

Professor Smalhout, thank you for your inspiration and please go on! ●

Jan M. Bult, President & CEO



Industry's Experiences Implementing PPTA's Donor History Questionnaire (DHQ)

BY MICHELLE MASON

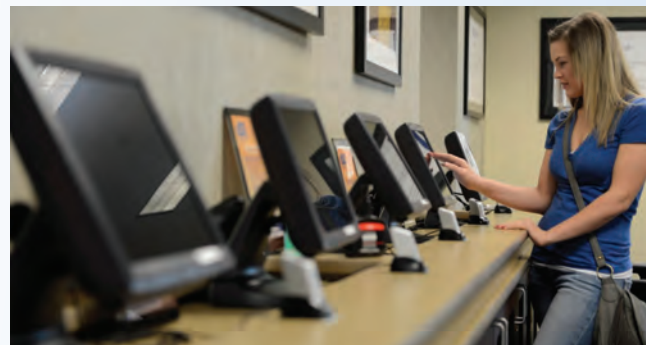




In February 2013, the U.S. Food and Drug Administration (FDA) published a guidance¹ for industry entitled "Implementation of an Acceptable Full-Length and Abbreviated Donor History Questionnaires and Accompanying Materials for Use in Screening Donors of Source Plasma" that recognizes PPTA's version 1.2 DHQ, dated September 2012, as an acceptable mechanism that is consistent with FDA's requirements and recommendations for collecting Source Plasma donor history information. The "accompanying materials" include a medication list and travel and risk posters. These materials are aids for helping prospective donors identify medications they have taken, places they have lived in or traveled to and behaviors they may have practiced. The information on the posters helps the prospective donor decide whether he/she is an acceptable candidate to donate plasma. Questions on the questionnaires also help determine if the donor is healthy to donate plasma. The goal of obtaining a health history from a donor is to ensure that the donation process will be a safe and satisfying experience.

PPTA has worked with its Donor History Questionnaire Committee (DHQC) (formerly Task Force), AABB (previously known as the American Association of Blood Banks), and FDA for over 10 years to produce these standardized materials to streamline the donor screening process by developing an abbreviated questionnaire for frequent donors, improve donor comprehension of the questions by enabling self-administration by donors, simplify the format and standardize the donor education materials to meet FDA's donor eligibility requirements. The PPTA DHQ documents can be found online at: <http://pptaglobal.org/safety-quality/donor-history-questionnaire>

The PPTA DHQ documents provide plasmapheresis establishments a process for administering questions to the Source Plasma donors to determine their eligibility to donate as per the donor suitability (eligibility) requirements established by the FDA and described in Title 21, Code of Federal Regulations 640.63. The questionnaires were designed to be used by a health historian in direct donor questioning or by self-administration either in paper form or by an electronic process, known as a computer-assisted self-interview (CASI)² process. Even though the FDA recognizes that the acceptable PPTA DHQ documents provide an effective tool for screening donors, they do not require companies to implement these exact documents, however it is encouraged by PPTA to the extent possible.



Donor using the electronic DHQ.

ED Q Donor II PDN: A3888510

You will be asked a series of questions regarding your current health and medical history. The first question is: Are you feeling healthy and well today?

Yes No Not Sure

Below the question is a cartoon illustration of a person jumping joyfully.

At the bottom are buttons for Previous, Play Audio, and Exit.

One example of a donor history question as presented in the electronic format.

Travel Poster II			
Country	Type of Travel or Residence	Time Period	De
<ul style="list-style-type: none"> Cameroon Central African Republic Chad Congo Equatorial Guinea Gabon Niger Nigeria Senegal Togo Zambia Benin Kenya 	Born in		
	Lived in	Since 1977	
	Travel to and/or Received a blood transfusion in	Since 1977	
	Sexual contact with anyone who was born in or lived in since 1977	Since 1977	
<ul style="list-style-type: none"> England Northern Ireland Scotland Wales The Isle of Man The Channel Islands Gibraltar 	3 months or more	Between 1980 and 1996	
	Received a blood transfusion in	Since 1980	

Travel poster

Source Plasma Industry Risk Poster I	
means any of the following (whether or not a condom or barrier device was used): <small>use (contact between penis and vagina)</small> <small>th or tongue on someone's vagina, penis, or anus)</small> <small>se (contact between penis and anus).</small>	
OT donate PLASMA, whole blood or platelets if you	
	<ul style="list-style-type: none"> Had HIV/AIDS (see list of symptoms below) Had a positive test for HIV (AIDS virus) Had a positive test for hepatitis Had hepatitis (after your 11th birthday) Used needles to take drugs, steroids or anything not prescribed by your doctor Used clotting factor concentrates for a bleeding disorder Had a transplant such as organ or bone marrow
7	<ul style="list-style-type: none"> Received money, drugs or other payment for sex (Male donors) Had sexual contact with another male, even once
8	<ul style="list-style-type: none"> Have given money, drugs or other payment for sex Have been treated for <ul style="list-style-type: none"> syphilis gonorrhea "Lived with" a person who has hepatitis (lived at same residence and shared kitchen and bathroom) Had a blood transfusion or received other blood products Received during surgery bone, tissue or skin Had an accidental needle-stick involving exposure to blood Had contact with someone else's blood Had a tattoo applied

Risk poster

There are PPTA member companies that have already implemented the PPTA DHQ (or a modified version) in their establishments and some that have not yet implemented but have informed PPTA that there are plans to do so in the future. Member companies have shared their experiences with implementing the DHQ and the feedback received was positive from both center and donor perspectives. The actual implementation process of the DHQ in the centers was quick and easy. The donors especially loved the DHQ when presented in an electronic process with the abbreviated questionnaire for frequent (qualified) donors. Center staff expressed appreciation of the abbreviated questionnaire in that they do not have to repeat the same questions as in the Full-Length Questionnaire over and over again each and every donation. Companies report that obtaining regulatory approvals has been simplified by FDA's acceptance of the PPTA DHQ. More importantly, by using the electronic DHQ, the collection facility's compliance risk has been greatly reduced in that questions cannot be inadvertently skipped. In a non-automated process, if missed questions are not caught, the result might be a biological product deviation (BPD) that requires a report be filed to FDA. PPTA member companies agree that using the DHQ in electronic format is a win-win situation for all participants involved. ●

MICHELLE MASON, Coordinator, Regulatory Policy & Global Access

¹ <http://www.fda.gov/downloads/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/LicensedProductsBLAs/BloodDonorScreening/UCM341088.pdf>

² Guidance for Industry: Streamlining the Donor Interview Process: Recommendations for Self-Administered Questionnaires, July 2003 <http://www.fda.gov/biologicsbloodvaccines/guidancecomplianceregulatoryinformation/guidances/blood/ucm075086.htm>

Full-length Donor History Questionnaire I – Source Plasma Industry	
	Yes No
Are you	
1. Feeling healthy and well today?	<input type="checkbox"/> <input type="checkbox"/>
2. Currently taking an antibiotic or other medication for infection?	<input type="checkbox"/> <input type="checkbox"/>
3. Currently taking any other medications?	<input type="checkbox"/> <input type="checkbox"/>
Please read the medication list	
4. Are you now taking or have you ever taken any medications on the medication list?	<input type="checkbox"/> <input type="checkbox"/>
Please review the Risk Poster	
5. Did you review the Risk Poster?	<input type="checkbox"/> <input type="checkbox"/>
6. Do you have any questions about anything mentioned on the Risk Poster?	<input type="checkbox"/> <input type="checkbox"/>
In the past six weeks	
7. Female donors: Have you been pregnant or are you pregnant now? (Males: check "I am male.")	<input type="checkbox"/> <input type="checkbox"/>
In the past two months	
8. Have you had any vaccinations or other shots?	<input type="checkbox"/> <input type="checkbox"/>
9. Have you had contact with someone who had a smallpox vaccination?	<input type="checkbox"/> <input type="checkbox"/>
10. Have you donated whole blood, platelets or plasma at another center?	<input type="checkbox"/> <input type="checkbox"/>
In the past four months	
11. Have you donated a double unit of red cells using an apheresis machine?	<input type="checkbox"/> <input type="checkbox"/>
In the past 12 months, have you	
12. Had a blood transfusion?	<input type="checkbox"/> <input type="checkbox"/>
13. Received during surgery bone, tissue or skin?	<input type="checkbox"/> <input type="checkbox"/>

Full length Donor History Questionnaire

Abbreviated Donor History Questionnaire For Frequent Plasma Donors	
Current Health	Yes No
1. Are you feeling healthy and well today?	<input type="checkbox"/> <input type="checkbox"/>
2. In the past six weeks, have you been pregnant or are you pregnant now?	<input type="checkbox"/> <input type="checkbox"/>
Please Read the Medication List	
3. Are you now taking or have you ever taken any medications on the medication list?	<input type="checkbox"/> <input type="checkbox"/>
Changes in health	
Since you last donated plasma:	
4. Have you had any new medical problems or diagnoses?	<input type="checkbox"/> <input type="checkbox"/>
5. Have you had any new medical treatments, vaccinations or medications?	<input type="checkbox"/> <input type="checkbox"/>
6. Have you had contact with someone who had a smallpox vaccination?	<input type="checkbox"/> <input type="checkbox"/>
7. Have you donated whole blood, platelets or plasma at another center?	<input type="checkbox"/> <input type="checkbox"/>
Risk Activities	
Please review our Risk Poster	
8. Did you review the Risk Poster?	<input type="checkbox"/> <input type="checkbox"/>

Abbreviated Donor History Questionnaire





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Our Mission

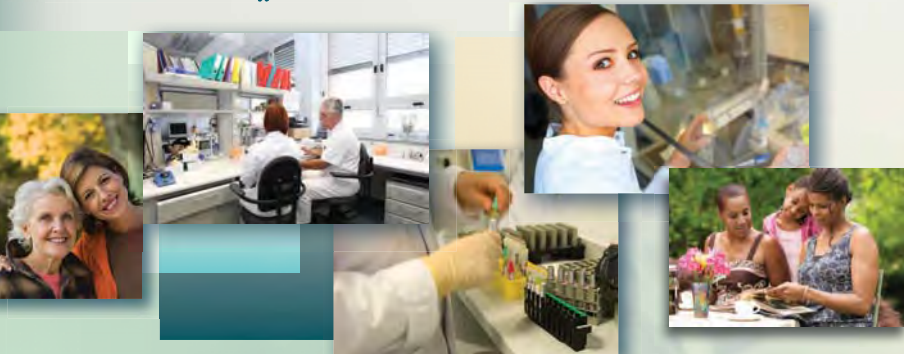
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Affordable Care Act (ACA): Open Enrollment Challenges

BY CARRIE FIARMAN

On October 1, 2013, open enrollment began for the Affordable Care Act's (ACA) state and federally run Health Insurance Marketplace (Exchanges) and will continue until March 31, 2014. Throughout the first two weeks, numerous reports of ongoing website crashes and the continuing inability to determine what benefits are included in the plans offered have dominated headlines. While data is beginning to trickle out of states, the White House will not release any numbers on enrollees until November. Even with initial numbers from the states, it remains unclear how many people have enrolled in a new plan through the state and federal Marketplaces. Individuals need to sign-up for plans offering minimum essential coverage, unless they file for an exemption, by the end of March or face a penalty of \$95 or 1% of their taxable income, whichever is greater. The penalty grows each year and, by 2015, will be \$325 or 2% of taxable income. By 2016, it will be \$695 or 2.5% of taxable income. Subsequent years will be calculated based on a cost-of-living formula.

Coverage through the Marketplaces will begin in every state on January 1, 2014 through a combination of state-run, federally-run or federal/state partnership marketplaces. In a federally-facilitated marketplace, the Department of Health

and Human Services (HHS) will perform all marketplace functions. States entering into a partnership marketplace may administer plan management functions, in-person consumer assistance functions, or both, and HHS will perform the

remaining functions. Currently, there are 17 state-based marketplaces, seven partnership marketplaces, and 27 federally-facilitated marketplaces.

A central goal of the ACA is to significantly reduce the number of uninsured by providing affordable coverage options through Medicaid and the Exchanges. The ACA expands Medicaid coverage for most low-income adults to 138% of the federal poverty level (FPL) (\$15,856 for an individual or \$26,951 for a family of three in 2013). Following the June 2012 Supreme Court decision, states face a decision about whether to adopt the Medicaid expansion. According to Kaiser Family Foundation, 25 states are moving towards expanding Medicaid or an alternative approach.

To help make plans more affordable, premium tax credits are available to help subsidize coverage for those who are between 100-400% of the FPL. Additional cost-sharing subsidies can substantially reduce the deductibles, copayments, coinsurance and total out-of-pocket spending limits for people with incomes between 100-250% of the FPL. Cost-sharing reductions will be applied automatically for consumers who qualify based on income, but only if they purchase a silver-level plan. Further, individuals between 100-400% of the FPL will see a reduction of out-of-pocket liability with caps that range between one-third and two-thirds of the maximum.

After analyzing enrollment in several different states, lingering questions remain on how these plans will affect access to plasma protein therapies. Because there is so much variation from state-to-state, consumers are lacking consistency on what benefits may be included in plans available in their state or even in their county. This lack of clarity only makes it more difficult for someone with a rare and chronic disease to determine what plan might provide them with adequate coverage.

One issue that has been reported since enrollment began is that many of the websites, including the federally-facilitated marketplace through Healthcare.gov, initially required browsers to create an account to review plans and compare plan information. Healthcare.gov has since created an option to browse plan premiums without logging in, but unfortunately you still cannot access plan details. Some websites currently are not allowing online enrollment. Because many individuals are unable to search plans without creating a log-in, it makes it extremely difficult to get a full picture of the plans offered and the premium levels.

Additionally, if a browser is able to compare plans on a state-facilitated marketplace website, often plan coverage details are extremely limited—with information only on deductibles, out-of-pocket maximums and possibly a premium price. One can also attempt to look up information on the health insurance company's website. As an alternative, an attempt can be made to evaluate what is covered through the state's benchmark plan.

One chief concern with evaluating benchmark plans is that according to the Center for Consumer Information and Insurance Oversight (CCIIO), because Essential Health Benefit

EHB-benchmark plan benefits are based on 2012 plan designs, and include state-required benefits that were enacted before December 31, 2011, some of the benchmark plan summaries may not reflect requirements effective for plan years starting on or after January 1, 2014. Plans for 2014 may be substantially different. According to CCIIO, the EHB-benchmark plans may include annual and/or lifetime dollar limits; however, in accordance with 45 CFR 147.126, these limits cannot be applied to the essential health benefits. Annual and lifetime dollar limits can be converted to actuarially equivalent treatment or service limits. The EHB-benchmark plan serves as a reference plan, meaning that plan benefits must be “substantially equal” to the benchmark's benefits and reflect both scope of services and limits. Plans are compared based on actuarial value calculations. Because of this, even though the benchmark plan may cover some services, other plans in the marketplace may not be required to cover the same exact services – making it extremely difficult to get a broad picture of patient access to specialists and plasma protein therapies and the affordability of expansive coverage.

In evaluating benchmark or sample plans from Maryland, Florida, Texas and Oregon, PPTA found a lack of consistency regarding information on prescription drug coverage for self-administered plasma protein therapies. For example, which therapies are included in formularies and cost-sharing or co-pay amounts for those therapies was an issue that was identified. One thing that has become clear in the early stages of open enrollment is that it is extremely difficult and time-consuming to compare what plans might be available to consumers and how they may cover a chronic condition. The need to dig deeper into these plans is extremely important for patients who need access to specialty care and rely on plasma protein therapies.

Even though open enrollment has already begun, there are outstanding questions resulting from a lack of regulatory guidance from key agencies charged with overseeing the law, its rollout and implementation. Guidance has yet to be issued on patient assistance programs in Alternative Benefit Plans (ABPs) – leaving manufacturers with uncertainty on how to incorporate some patient assistance program elements and potentially leading to interruptions in access. Also, the ACA requires HHS to develop plans to transition patients from the Pre-existing Condition Insurance Plan (PCIP) program into Exchanges; however no guidance has been issued. This remains an area of concern for the users of plasma protein therapies, particularly the hemophilia community and in states that do not expand Medicaid eligibility.

These are only a few key concerns identified with the ACA and how its rollout and implementation may affect access to plasma protein therapies. The Association continues to identify and raise problems and solutions with the appropriate federal regulatory agencies. ●

CARRIE FIARMAN, *Manager, Federal Affairs*



Giovanni RINALDI

BY JAN M. BULT

Giovanni Rinaldi, a native of Lucca, Italy and a pharmacist by training, started working for Kedrion in 1964 in the marketing and sales division. In 1975, he was involved with the opening of Kedrion's first U.S. based collection center. From that time on, he represented the company in the American Blood Resources Associations (ABRA), the predecessor to PPTA, representing the private sector plasma collection industry.

In Europe, the political environment was completely different from the U.S. and was negative, particularly towards the private sector. Dr. Otto Schwarz from Immuno AG invited several representatives from the plasma industry to come to Vienna and convinced them that an association was needed to represent industry. This new initiative should counter the negative feelings against the private sector. It was at this meeting that the representatives decided to form the International Plasma Products Industry (IPPIA), another precursor to PPTA. It was well understood that one important focus would be to raise the quality of plasma protein therapies and Rinaldi added, "The increased quality of plasma protein therapies is impressive. Since that meeting there has been a significant improvement that includes various elements such as donor selection, testing, inventory hold and collection center epidemiology."

“The increased quality of plasma protein therapies is impressive.”



Throughout the years, Giovanni Rinaldi has been very active in Association efforts. Until the end of 2013, he serves on the PPTA Global Steering Committee (PGSC), the Europe Board of Directors (BODI) and the European Health Policy Steering Committee (HPSC).

He considers the development of the Nucleic Acid Test (NAT) and the inventory hold and their incorporation into the IQPP and QSEAL standards as examples of great industry achievements. His proudest personal achievement was recognizing the potential of intra-muscular immunoglobulin anti-hepatitis B and encouraging Kedrion to invest in the development of this product.

Giovanni Rinaldi has always been a loyal supporter for this industry. With his enormous experience he was able to provide good advice to all of us. He always impressed me because he was able to look at the industry needs and not only to company needs.

Looking forward, Rinaldi believes that industry must respond to the challenges driven by diagnostic improvements and the subsequent increase in clinical needs for plasma protein therapies. He personally believes that the public sector in Europe will ultimately be privatized and create more competition in Europe.

After nearly 40 years of service to the Association and industry, Giovanni will resign his positions by year end. “Kedrion is a well-established international company. It is the right moment to let a new generation of Kedrion employees appreciate the experience of working within PPTA,” Rinaldi said. ●

JAN M. BULT, *President & CEO*

Looking forward, Rinaldi believes that industry must respond to the challenges driven by diagnostic improvements and the subsequent increase in clinical needs for plasma protein therapies.

aving

International Plasma AWARENESS WEEK

BY SONIA BALBONI

The first ever International Plasma Awareness Week (IPAW) ran from October 13-20, 2013. PPTA, members and stakeholders in the U.S. and Europe participated by sponsoring donor appreciation events, open houses for stakeholders and involving government officials and the media at plasma collection facilities in Europe and the United States. The week kicked off in Denver on October 13 with an evening reception at the Brown Palace Hotel, held in conjunction with the annual PPTA Source Business Forum. Additionally, a workshop was held in Vienna with participants representing the industry, regulatory authorities, patient organizations and donors. A historic total of 142 individuals attended the events.



Audience of annual PPTA Source Business Forum

“Patients throughout the world rely on plasma protein therapies which are developed through the generosity and commitment of plasma donors. PPTA is proud of the contributions industry makes to saving and improving lives,” said Joshua Penrod, PPTA Vice President, Source.

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“Patients throughout the world rely on plasma protein therapies which are developed through the generosity and commitment of plasma donors. PPTA is proud of the contributions industry makes to saving and improving lives,”

— *Joshua Penrod, PPTA Vice President, Source*

IPAW will be held annually and has three primary goals:

- » Raise global awareness about source plasma collection
- » Recognize the contributions of plasma donors to saving and improving lives
- » Increase understanding about lifesaving plasma protein therapies and rare diseases

Over the course of the week, industry members and stakeholders staged celebratory events. Many stakeholder organizations also participated in the event, through newsletter articles, website and social media posts, as well as global outreach. These included:

- » Alpha-1 Association
- » GBS/CIDP
- » Hemophilia Federation of America
- » Jeffrey Modell Foundation
- » LA Kelley Communications
- » Immune Deficiency Foundation
- » IPOPI
- » National Hemophilia Foundation
- » Primary Immunodeficiency UK

Media attention focused on the event was impressive. More than one dozen press releases were issued, originating in Austria, Belgium, the Czech Republic, Germany and the United States. There were approximately 2,900 web postings and 458 items in print/broadcast media, in the U.S. and abroad. Over 400 one-minute radio spots aired in select cities around the United States.

Legislatures recognized the event as well. Governors in fourteen states in the U.S. issued proclamations (CT, DE, IL, MA, MS, MT, NV, NJ, NC, OK, OR, PA, RI, TX). Additionally, the Honorable Lynn Jenkins entered a statement in the United States Congressional Record, recognizing the event and noting the value of plasma protein therapies and the donors of plasma used in their manufacture.

A future issue of the Magazine will include greater detail on the inaugural IPAW and plans for the future. ●

SONIA BALBONI, *Manager, Source & Standards*

A large, ornate statue of Minerva, the Roman goddess of wisdom and warfare, stands prominently on the left side of the page. She is depicted wearing a detailed golden helmet and holding a golden apple in her right hand. The statue is set against a clear blue sky, and a thick orange curved line runs vertically along its right side, separating it from the main text area.

IPAW IN EUROPE

BY ALEXA WETZEL

The Association, together with Pharmig and IG Plasma, sponsored a workshop and reception as part of International Plasma Awareness Week (IPAW). The reception took place on October 17 in Vienna, and was attended by Industry, regulatory authorities, patient organizations and donors.



IPAW participants in Vienna

The keynote was given by Dr. Jan O. Huber, Secretary General from the Pharmig (Representation of the Austrian Pharmaceutical Industry). He reviewed the history of Plasmapheresis in Austria, pointing out that Austria has been a precursor in regulating this activity. In 2014, fifty years of plasma donation in the country will be celebrated.

PPTA President Jan M. Bult emphasized the importance of recognizing the need for plasma protein therapies and committed, reliable plasma donors. Bult stressed the dedicated work of industry over time which has resulted in increased safety of the therapies. He also observed that plasma donors deserve the greatest respect for their valuable contributions which give life to so many patients.

“We are delighted that this esteemed coalition has joined us in recognition of the important role of plasma protein therapies in saving and improving patients’ lives. It is equally important that we also recognize the lifesaving role of the plasma donor ... I urge everyone to say to every donor – ‘thank you’,” Mr. Bult said.

Professor Helmi Storch (German Arbeitsgemeinschaft Plasmapherese) gave an overview on plasma collection in Germany and Dr. Matthias Gessner [Spokesperson of the Austrian Interessengemeinschaft Plasma (Austrian plasma collectors group)], Baxter AG, reviewed plasma collection in Austria.

Alexander Szivak from AGES (Austrian Agency for Health and Food Safety) observed the strict regulatory requirements for plasma collection. He observed that the objective of the regulatory agency is to monitor and ensure the safety of the donor and the patient, as well as the availability, safety and quality of the product.

Next, Primary Immune Deficiency (PID) patient Karin Modl, representing the Austrian Support Group for Primary Immunodeficiency (ÖSPID), explained her long struggles before ultimately being diagnosed with PID. She discussed how much her life has improved with plasma protein therapies and wholeheartedly thanked the donors for her enhanced quality of life.



In Vienna: Patient Representative Karin Modl and Donor Peter Messner

Following this, Dr. Hermann Wolf (Immunological Clinic in Vienna) discussed his experiences with plasma protein therapies and particularly with Immunoglobulin and the treatment of PIDs. Last, Michael Moser, a committed donor for over 20 years, shared his motivations for donating plasma, noting that compensation had never been among his reasons. For him, helping patients improve their lives is his reward. Mr. Moser expressed his gratitude for the opportunity to meet a donor at the event, and felt that every donor should have the same opportunity.

The workshop ended with warm expressions of gratitude towards the commitment and dedication of plasma donors. An evening reception followed. ●

ALEXA WETZEL, Junior Manager, Source Europe



Dr. Matthias Gessner, Jan M. Bult and Professor Helmi Storch

PPTA President Jan M. Bult stressed the importance of recognizing the need for plasma protein therapies and committed, reliable plasma donors.





PLUS – The Platform of Plasma Protein Users

BY JOHAN PREVOT, CO-AUTHORED BY BRIAN O'MAHONY

PLUS, the Platform of Plasma Protein Users, represents organizations of patients with treatable, rare diseases linked by common therapies based on products manufactured from human plasma.

PLUS has been active since 2010 when it convened the first Consensus Stakeholders Meeting in Dublin, which focused on the collection of blood and plasma and the manufacture of plasma products. Since then, the consensus meetings have been held every year. Key stakeholder organizations active in the field of blood and plasma derived medicinal products participate with a view to discuss developments affecting patient communities and to identify consensus principles.

Two consensus statements on vital issues relating to the collection of blood and plasma and the manufacture of plasma products were published in *Vox Sanguinis* as an outcome of the discussions held during the two first meetings.

These statements were extremely valuable as they provided, for the first time, a summary of key principles upon

which the various stakeholder organizations agreed on (some with qualification).

In 2012, the meeting looked at “Optimized Supply of Plasma Derived Medicinal” products. The consensus statement has been submitted for publication and should be published shortly.

This year, the PLUS Stakeholders Consensus Meeting was held in Estoril, Portugal in September. The meeting was organized to identify new topics that the PLUS consensus stakeholders’ platform should look at in the future; subsequently, two sessions were organized. The first focused on risk-based decision making (RBDM) and specifically the Alliance of Blood Operators (ABO) RBDM project that aims to develop an integrated risk management strategy. It was agreed that the project could be a significant first step to

establish a framework for managing the interrelationship of risk tolerance, supply of blood and plasma derived medicinal products and economic considerations in the future. PLUS will circulate an open letter outlining PLUS' position on this topic following the discussions at the meeting and will encourage the inclusion of the various stakeholder groups that participate in the PLUS consensus meetings into the ABO project. A follow up meeting may also be envisaged in 2014. The meeting also featured a session on "Treatment in 2020: Patient & Physician Views." The various patient communities attending the meeting highlighted their priorities, as well as the opportunities and threats to an optimal supply of plasma derived medicinal products (PDMPs) in the future.

In addition, PLUS has been involved in a range of policy discussions at the European Union (EU) level. PLUS has agreed to a consultation mechanism with the European Commission (EC) to ensure the rare plasma-related disorders' patient views are always taken into account in the decision-making process of relevant policies.

PLUS has recently taken part in the EC's consultation regarding the potential review of the Blood Directive. PLUS has also launched a Call to Action highlighting the priorities of the PLUS patient communities.

Key stakeholder organizations active in the field of blood and plasma derived medicinal products participate with a view to discuss developments affecting patient communities and to identify consensus principles.

Upcoming policy dossiers of relevance to PLUS include: the implementation of the cross-border healthcare directive, EU actions in the area of Health Technology Assessments and the review of the Blood Directive and the implementation of the EU Council rare diseases recommendations at national level. ●

JOHAN PREVOT, *Executive Director, International Patient Organization for Primary Immunodeficiencies*

BRIAN O'MAHONY, *President, European Hemophilia Consortium*



2013 Consensus Meeting



Participants at 2012 Consensus Meeting

PLUS member organizations include:



Inside PPTA



● European Directorate for the Quality of Medicines & Healthcare (EDQM)

OFFICIAL CONTROL AUTHORITY BATCH RELEASE (OCABR) NETWORK

MEETING WITH REPRESENTATIVES OF MANUFACTURERS OF BLOOD AND PLASMA DERIVATIVES

BY ILKA VON HOEGEN

For a number of years, the meeting between the OCABR network and manufacturers of plasma derivatives takes place annually. The meeting should be seen as an opportunity to openly exchange views and opinions, but not as a decision making forum. Both industry and regulators contribute to the agenda. It has to be highlighted that in some years there had been no meeting for the simple reason that the OCABR was running so smoothly, that no topics of mutual interest could be identified. The OCABR stipulates the provisions that ensure the safety of each batch of medicines distributed to patients in the European Union (EU). Some highlights from year's meeting are summarized below.

BATCH RELEASE

Batch release issues are a recurrent item on the agenda and manufacturers are encouraged to present any deviation from the OCABR procedure. This happens occasionally, when EU National Competent Authorities (NCA) interpret the OCABR provisions in a different way than intended.

Increasingly, the Official Medicinal Control Laboratories (OMCL) network provides services for outside NCAs, which is another indication for the efficiency and credibility of the current system. However, an OCABR batch release for plasma derived product where the product is intended for a non EU country cannot be performed because of the current legal provisions. In those cases, a contract can be put in place to overcome these limitations, for example, between Germany and Iran. When no contract is in place, a solution is to ask for a test report, which is carried out in accordance with the EU Batch release procedure.

While the key intention of the OCABR is to harmonize and reduce workload, there are still limitations: There are no

legal provisions that would permit EDQM to implement an overarching e-submission scheme. Therefore, manufacturers have to use the diverse OMCLs' approaches that are currently under development.

PATHOGEN SAFETY

Since January 2013, the monograph for Solvent Detergent (SD) plasma requires screening for Hepatitis E Virus-Ribonucleic Acid (HEV-RNA). It will soon be decided what limit would be appropriate for the HEV Nucleic Acid Test (NAT) assay. The decision will be made based on available scientific data, such as the data generated by the Paul Ehrlich Institut (PEI). Manufacturers are invited to share all available data with the responsible committee to assist in their decision making process.

IMMUNOGLOBULINS (IGS)

Regulatory authorities are now requesting full assay validation and batch to batch data for the determination of thromboembolic activity in IG preparations. Currently, no test is described in the monograph, but the ultimate goal is to do so. Manufacturers argued that the choice of test should be left to the company. Currently, there are two tests available. EDQM acknowledges the advantages of one of the two as a method for routine use. Any laboratory test is only as good as the standard reagents available. Currently there is only a working reagent available, but an International Standard would be a prerequisite. Consequently, the introduction of a test is seen as a long term goal.

Although there is neither an agreement of the most suitable test nor a standard reagent available, regulatory requirements have been put in place to investigate marketed product for their thromboembolic potential. This regulatory procedure has been concluded and all data from manufacturers has been



submitted to the European Medicines Agency (EMA). A workshop with all stakeholders is planned to evaluate the data, identify gaps and develop solutions. On the basis of this discussion, a proposal on the nature of the test will be developed.

Manufacturers voiced their concern that the decision will favor a test currently not in use or a variation of a test in place. This would require extensive and unnecessary revalidation.

The OMCL's currently have no intention to perform routine tests, but would implement a tool for phase II testing if there is indication increased thromboembolic risk for a given product.

At the annual OCABR meeting, an agreement by consensus was reached that from January 2014, OMCL's will test all IG preparations for anti A/anti B hemagglutinin. This decision was made on the basis of available data and Pharmacovigilance observations. The decision will be reviewed after 2 years. Also in this case, a prerequisite for the introduction of routine testing is the establishment of a reference standard.

A new cytometric method for anti A/anti B determination received a lot of attention from participants. Since there are no patents on the method, EDQM will consider introducing it into the relevant monograph as an alternative to the test currently

in use, which has a number of limitations. In this context, it was reconfirmed that any suitable method can be used, as long as it is validated and accepted by the Regulatory Authority.

COUNTERFEITING OF MEDICAL PRODUCTS (MEDICRIME)

The Council of Europe (CoE) drafted a convention which constitutes, for the first time, a binding international instrument in the criminal law field on counterfeiting of medical products and similar crimes involving threats to public health: http://www.coe.int/t/DGHL/StandardSetting/MediCrime/Default_en.asp.

The Member States have to sign the Convention, which does not imply automatically that the provisions are in force. Implementation into national law is required. At least 5 countries need to ratify the convention, of which 3 need to be CoE members. The convention will be presented at the upcoming meeting of the Observer States. Currently, the enthusiasm to sign the convention seems to be limited. ●

ILKA VON HOEGEN, *Senior Director, Quality & Safety*

● Preview: FDA/PPTA/NHLBI Workshop on Hemolysis in Immune Globulin Patients

BY MARY CLARE KIMBER

On January 28-29, 2014, PPTA will co-sponsor with the U.S. Food and Drug Administration (FDA) and the National Institutes of Health (NIH), a public workshop on risk mitigation strategies to address hemolytic complications of immune globulin (IG) infusions¹. This 1.5-day workshop will be held at the Lister Hill Auditorium on the NIH campus in Bethesda, Maryland. While IG-associated hemolysis is not a new issue, and all intravenous IG products contain a warning about the risk of hemolysis, FDA recently has expressed renewed concerns, particularly with hemolysis occurring after high-dose regimens and in older patients with auto-immunity. PPTA agreed to co-sponsor a workshop to address the issue and convened a Task Force to explore the issue and to aid in planning and executing the workshop.

The workshop will bring together global regulators, industry, the NIH National Health, Lung and Blood Institute (NHLBI), the blood community, academia, patients and the public. PPTA has been an active member of the workshop steering committee through Mary Gustafson (Vice President, Global Regulatory Policy) and Val Romberg (CSL Behring), along with representatives of FDA, Health Canada, the European Medicines Agency (EMA), the Paul-Ehrlich-Institut (PEI), Swissmedic, NIH and the Ottawa (Canada) Hospital. Additional representatives of global regulators, the blood community and academia also are expected to contribute to the workshop as speakers and/or panelists. The workshop agenda includes the following sessions: Pathogenesis and Epidemiology IG product-related Hemolysis, Product Risk Factors and Immune Globulin Manufacturing and Risk Mitigation².

Along with global regulators, PPTA will present on epidemiology and risk factors for IG-related hemolysis during the first session of the workshop. To this end, the PPTA Pharmacovigilance Committee (PHVC) was convened to aggregate pharmacovigilance data for presentation at the workshop. The goals of the PHVC data collection are to perform a qualitative analysis of cases of IG-associated hemolysis reported to members via their spontaneous pharmacovigilance reporting systems for a ten-year period (2003-2012), to identify potential patient risk factors and other possible risk factors for IG-associated hemolysis, to analyze the associated profile

of IG indications and to examine the associated dose levels and administration rates. Pathogenesis of hemolysis also will be discussed during the first session of the workshop.

Additional PPTA and member presentations are planned for later sessions of the workshop. During the second session of the workshop, participants will discuss anti-A and anti-B haemagglutinins in IG products and other product risk factors for hemolysis. The workshop will culminate with a discussion of possible risk mitigation strategies.

PPTA is committed to promoting the availability of and access to safe and effective plasma protein therapies for all patients in the world. PPTA is partnering with global regulators, the blood community, academia, patients and the public in this effort and looks forward to a productive workshop. ●

MARY CLARE KIMBER, *Manager, Regulatory Policy*

¹ As of November 12, 2013, the Federal Register notice officially scheduling the workshop had not yet published.

² As of November 12, 2013, the workshop agenda had yet to be finalized.

Along with global regulators, PPTA will present on epidemiology and risk factors for IG-related hemolysis during the first session of the workshop.

● EMA Drug Shortages Workshop Focuses on Patient Safety

BY JOHN DELACOURT

Drug shortages do not recognize national boundaries and, not surprisingly, have drawn the attention of regulators on both sides of the Atlantic. On October 14, 2013 the European Medicines Agency (EMA) again addressed the issue by convening a workshop on “Product Shortages Due to Manufacturing and Quality Problems: Developing a Proactive Approach to Prevention.” PPTA was among the participating stakeholders and provided an overview of shortage preparedness efforts in the plasma protein therapies industry, with a focus on the Association’s North America Data Program. PPTA pledged that, with appropriate modifications to reflect differences in both the European marketplace and regulatory structures, the Association will soon implement a similar data program in Europe. This approach was welcomed by the assembled participants as advancing what EMA’s Brendan Cuddy, a principal workshop organizer, characterized as the “main goal”: patient safety.

BACKGROUND

In November 2012, EMA indicated its intention to closely examine the issue of drug shortages by publishing a “Reflection Paper on Medicinal Product Supply Shortages Caused by Manufacturing/Good Manufacturing Practice Compliance Problems.” The Reflection Paper expressed concern with unforeseen disruptions in product supply, which it characterized as “public health crises.” Such shortages, it argued, can lead to a litany of sub-optimal and risky practices from a patient care perspective, including both substantial delays in treatment and outright non-treatment. Even when an alternative medication is available, the substitute product may be less effective or produce switching-related side effects, ultimately leading to adverse outcomes that should have been preventable. In light of this situation, the Reflection Paper called for renewed, multi-stakeholder initiatives to develop tools and procedures to address shortages, ranging from improved assessment and enhanced risk mitigation techniques to broader and more rapid methods of communication. EMA acknowledged that similar initiatives had been undertaken in the past, but noted that they had often been “reactive rather than proactive.” EMA was clear that it regards the contributions of industry as central to this effort, stating expressly that “industry should propose solutions.”

DEFINING THE PROBLEM

One of the workshop’s most interesting exchanges involved differing views on the prevalence of drug shortages. According to a survey conducted by the European Association of Hospital Pharmacists (EAHP), fully 99% of respondents reported experiencing problems with shortages in the last year. Yet, after spending considerable time examining the issue, the European Federation of Pharmaceutical Industries and Associations (EFPIA) concluded that industry “does not see a systemic issue of shortages in the EU.” In between these two extremes, EMA indicated that, so far in 2013, it has received 17 reports of quality or manufacturing problems that may result in a product shortage.

continued on next page.



In November 2012, EMA indicated its intention to closely examine the issue of drug shortages by publishing a “Reflection Paper on Medicinal Product Supply Shortages Caused by Manufacturing/Good Manufacturing Practice Compliance Problems.”

"EMA Drug Shortages Workshop Focuses on Patient Safety" continued...

The disconnect here appears to be rooted, in large part, in inconsistent definitions of the term "shortage." Some workshop participants appeared to employ the term broadly, in a manner that encompassed virtually any difficulty in obtaining product, regardless of cause. Others appeared to adhere more closely to the focus of the workshop – the title of which specifically referenced shortages "due to manufacturing and quality problems" – by attempting, as best they could, to exclude from the discussion patient access issues resulting from changes in reimbursement rates or methodologies, or such practices as so-called "parallel import." It was agreed that this ambiguous terminology was not helpful to the discussion and that EMA would add developing a clear definition to "shortage" to its post-workshop objectives.

PROMISING APPROACHES

Although no workshop participant purported to have an all encompassing solution to the problem of drug shortages, a number of participants proposed promising approaches. Some strategies were as fundamental as making drug shortage prevention a priority of company management and developing a shortage contingency plan proactively. Others involved the development, or improvement, of manufacturer IT systems to more closely track critical inputs and rapidly identify problems in the production process. Strategies for establishing redundancy in the supply chain and manufacturing operations were also discussed, though the costs associated with this approach could be significant.

Another set of proposals focused on notification/assessment and stakeholder communication. In order to assess whether a particular access issue is, in fact, a shortage, both regulators and manufacturers need information regarding product supply. PPTA's presentation – which provided an overview of the mechanics and stakeholder benefits of its North America Data Program – was specifically tailored to address this concern. By providing aggregate data on all major categories of plasma protein therapies, PPTA's program enables public health professionals to distinguish rumors and non-specific marketplace concerns from a true shortage – a situation which, thankfully, has not occurred in the plasma protein therapies industry in over a decade. By posting the data on its web site, where it is available to patients, PPTA is also able to provide peace of mind to individuals dependent on these therapies

for their continued good health. The presentation was well received by the meeting participants and served as a helpful and timely response to EMA's challenge that solutions should come from industry.

NEXT STEPS

At the conclusion of the workshop, EMA provided an update on the status of the Implementation Plan that accompanied its November 2012 Reflection Paper. Two items of note are that work continues on both a Concept Paper on "proactive risk management for manufacturers" and a standard operating procedure for handling reports of shortages. PPTA will continue to engage with EMA staff regarding the content of these documents as drafting continues. PPTA will also continue to track ongoing, industry-led, multi-stakeholder efforts to address the issue of drug shortages, and to engage with FDA as implementation of the shortage notification provisions of the Food and Drug Administration Safety and Innovation Act proceeds. ●

JOHN DELACOURT, *Senior Director, Legal Affairs*

● Gerold Zerlauth Honored with Robert W. Reilly Leadership Award

BY JOSHUA PENROD



In the early 1990's, while working with CEO Dr. Otto Schwarz at the Vienna based Immuno AG, Gerold Zerlauth, Ph.D., Director of Baxter Plasma Control Europe, pioneered the use of polymerase chain reaction (PCR) technology for the use in the source plasma industry, which

paved the way for the introduction of European legislation on PCR in 1999. Until then, only serology testing methods were available which left a significant window period in the detection of viruses in blood or plasma donations. Dr. Zerlauth implemented the PCR methodology, including a three-dimensional pooling and deconstruction scheme in a way that allowed this cutting edge technology to be used in an economically feasible way for plasma screening. Since then, all plasma derived product manufacturers have adopted nucleic acid testing (NAT) technology and similar pooling techniques. It is now both an industry standard of the industry, as well as a requirement of most regulatory agencies including the Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Dr. Zerlauth is recognized by his peers as an expert in PCR technology, as well as other important aspects of plasma quality and safety and, not at least, for his achievements in European source plasma collection. He has remained a vocal proponent for all aspects of plasma safety and has been active in many plasma-related industry groups and standard setting organizations including: the PPTA European Plasma Collectors Committee, US Pharmacopeia, Working Group Standardization of Gene Amplification Techniques, Austrian Blood Advisory Committee, International Society Blood Transfusion (ISBT), and Austrian Working Group Plasma Proteins.

In PPTA, he has served as the chairman of the European Plasma Collectors Committee (EPCC) since 2011. Under his leadership, EPCC has developed into an effective group and a respected partner in its interaction with stakeholders. This is even more important today as we face potential reform of the European Union's (EU) Blood Directive.

I'm delighted that a long-time contributor to the industry has been honored by the membership. His scientific achievements, as well as his leadership of the EPCC have been invaluable.

The Robert W. Reilly Leadership Award recognizes an individual "who has had a positive influence on the source plasma collection industry and who has demonstrated unquestionable professional character, ethics and commitment to the advancement of the plasma protein therapeutics industry and commitment to the goal of improving access for patients to these lifesaving therapies. The nominated individual may be an active or retired industry member, consumer representative, scientist or regulatory authority."

Dr. Gerold Zerlauth's contributions have made a significant difference, not only to our industry, but to the patients worldwide who rely on the quality of plasma protein therapies. ●

JOSHUA PENROD, *Vice President, Source*

PREVIOUS HONOREES

2011
Donald Baker

Baxter/Retired

2010
Ruedi Waeger

Talecris

2009
Bernard Horowitz

Consultant

2008
Peter Turner

CSL Behring

2007
Jean-Marie Vlassembrouck

*Baxter BioScience/
BioLife Plasma Services*

2006
David J. Gury

NABI Biopharmaceuticals

2005
Joseph Rosen

*Baxter BioScience/
BioLife Plasma Services*

2004
George Schreiber

Westat

2003
S Tyrone Foster

Aventis Bio-Services

2002
Richard Thomas

Bayer Corp

2001
Victor Grifols Lucas

Grifols

2000
Samuel Penninger, Jr.

Serologicals Corp

1999
Jack Ryan

Bayer Corp

1998
John Bacich

Baxter Healthcare Corp

● PPTA Business Forum

BY SONIA BALBONI

The annual PPTA Source Business Forum was held on October 13, 2013 in Denver, Colorado. Source Board of Directors Chair Shinji Wada addressed members and staff, highlighting the work of the Source division this year, especially in the area of donor safety. PPTA President and CEO Jan M. Bult summarized key issues involving the Association on a global scale, and saluted the long-anticipated arrival of a global event focusing on plasma donation - International Plasma Awareness Week (IPAW). IQPP Standards Committee chair, Ileana Carlisle, outlined developments with the IQPP certification program. She highlighted the development of guidance for reporting Hepatitis C Virus (HCV) confirmed donors in the absence of a recombinant immunoblot assay (RIBA) test, work of a task force to revise the IQPP standards for global relevancy and the commissioning of an automated solution for compliance with the IQPP Cross Donation Management Standard. Regulatory Policy and Compliance Steering Committee (RPSC) chair Roger Brinser gave an overview of the Committee's efforts with several projects. These included an ongoing study of ferritin levels in plasma donors, development of common industry terms for donor reactions and ongoing discussions with FDA on a number of issues of interest. European Plasma Collectors Committee (EPCC) chair, Gerold Zerlauth, Ph.D., discussed key developments in plasma collection for the region, including PPTA outreach to the European Blood Alliance (EBA), and discussions surrounding the possible revision of the European Blood Directive.

The event attracted a historic 87 participants, more than ever before. The program featured a panel titled, "Perceptions of Collection and Compensation." Joshua Penrod, PPTA, Vice President, Source, opened the panel with an exploration of various theories surrounding "compensation." Ethicist Dr. James Stacey Taylor, Ph.D., gave his views on ethical issues surrounding source plasma donation, advancing among other ideas his theories on the ethics surrounding informed consent. Dr. Graham Sher (Canadian Blood Services) explored the current debate surrounding source plasma donation in Canada. Professor Albert Farrugia, PPTA, Vice President, Global Access, examined how blood sector groups can construe differently the motives for plasma donation, and the varied messages that are conveyed to sway public opinion. The panel was moderated by Mary Gustafson, PPTA, Vice President, Global Regulatory Policy.



Dr. James Stacey Taylor, Ph.D. speaks on source plasma donation ethical issues



Source Board of Directors Chair, Shinji Wada alongside EPCC chair and 2013 Robert W. Reilly award recipient, Gerold Zerlauth, Ph.D. and PPTA President and CEO, Jan M. Bult

Dr. Taylor, Associate Professor of Philosophy at The College of New Jersey, has published extensively on ethical issues in medicine and lectures widely on medical ethics in both the U.S. and Europe. Dr. Sher is Chief Executive Officer of Canadian Blood Services (CBS) and current Director of the Board for the American Association of Blood Banks (AABB).

The program ended with a reception inaugurating International Plasma Awareness Week (IPAW), and honoring Dr. Zerlauth for receiving the Robert W. Reilly Leadership Award. ●

SONIA BALBONI, *Manager, Source & Standards*

Committee Spotlight

BY MARY CLARE KIMBER

REGULATORY POLICY AND COMPLIANCE STEERING COMMITTEE

The PPTA Regulatory Policy and Compliance Steering Committee (RPSC) advocates for rational regulatory policies that impact the industry by representing the Association before regulatory authorities in North America, primarily the U.S. Food and Drug Administration (FDA). To fulfill its central mandate to identify and prioritize key regulatory policy issues, RPSC monitors, assesses and guides the development of new and potential regulatory policies; develops regulatory initiatives and alternatives that will advance stakeholders' interests; and liaises with FDA.



PPTA and FDA meet at the 2013 Liaison Meeting on September 20

RPSC develops strategies and implementation plans that support projects under the PPTA Strategic Priority, Regulatory Policy and address related flashpoints. RPSC is a large PPTA committee that represents both collection and manufacturing interests. While some work involves the entire RPSC, other projects are addressed by Committee members with particular interests and industry subject-matter expertise. RPSC also guides the activities of other PPTA regulatory committees under identified regulatory policy priorities, including the Medical Policy Committee (MDPC) and the Donor History Questionnaire Committee (DHQC), which are comprised of industry subject-matter experts. Complex, multi-faceted regulatory policy issues often require RPSC coordination with other PPTA committees.

RPSC's day-to-day activities include providing regulatory contributions in the form of written comments, letters and statements on proposed rules, draft guidances and other documents to regulatory authorities, primarily FDA. PPTA recently has provided written comments on several draft guidances to FDA, including "Changes to an Approved Application: Human Blood and Blood Components Intended for Transfusion or Further Manufacture" (August 2013) and "Circumstances That Constitute Delaying, Denying, Limiting, or Refusing a Drug Inspection" (September 2013).

A hallmark of RPSC's activities is the PPTA-FDA Liaison Meeting held annually in the fall and led by the PPTA Vice President of Global Regulatory Policy and the Director of the Office of Blood Research and Review in the Center for Biologics Evaluation and Research, FDA. Liaison Meetings provide an opportunity for PPTA and FDA to identify priorities and other topics of interest that are important for the success and health of industry and patients and offer a venue for an open and constructive dialogue between PPTA and FDA to share views and concerns and to work toward meaningful solutions.

A significant RPSC accomplishment is the establishment of the PPTA Regulatory Workshop as an annual event held in conjunction with the PPTA Plasma Protein Forum. Developed by RPSC, this four-hour event serves to educate PPTA members and to bring together industry, regulators, and other stakeholders to discuss timely issues such as the PPTA Donor History Questionnaire (DHQ), U.S. and EU inspections and donor safety. For information on the 2014 PPTA Regulatory Workshop, please visit: <http://www.pptaglobal.org/meetings-events/regulatory-workshop>. ●

MARY CLARE KIMBER, *Manager, Regulatory Policy*

REGULATORY POLICY AND COMPLIANCE STEERING COMMITTEE

Charles Auger
Nelli Cherny
Karen Clifford
Karen Etchberger
Michael Hafley
Jon Knowles
Ron Kraiss
Mary Ann Lamb
John McVey
Robert Rave
Linda Roachvarg
Janice Smith
Jennifer Timmerman
Janis Wilson
Mary Gustafson,
PPTA Staff Liaison

For more information on RPSC, please visit: <http://www.pptaglobal.org/safety-quality/regulatory-policy/overview>

MEET THE PPTA Staff

Bruno Santoni

EXECUTIVE DIRECTOR EUROPE



Q When did you join PPTA?

I have started recently on the 9th of October 2013.

Q What are your first impressions?

First of all, everybody has been extremely dedicated and helpful to get me on board and up to speed. There was great team spirit from the PPTA staff and also from the members. I'm very glad I joined PPTA and I see several opportunities to further develop the actions of the PPTA.

Q What do you focus on in your role as Executive Director PPTA Europe?

The main goal is to enhance Patient Access to plasma protein therapies in Europe. My role is to coordinate the strategic vision and operational execution of PPTA Europe to achieve this. I'm working daily with my local team, the colleagues based in the US and the different board members. I primarily dedicate my time by providing leadership, ensuring coordination and conducting advocacy activities. Two major projects are currently on my priority list: the EU Blood Directive Revision and the IPPC congress on March 11-12 in Vienna. The congress is the event to attend in order to stay updated on the latest developments related to the Plasma Proteins Therapies and to network with key contacts! I consider also that it will be important to reevaluate the Patient Access environment at the country level and to see if there are specific needs and opportunities where PPTA can provide support.

Q Tell us about your background.

I was born in Belgium and my roots are Italian. I have a Master Degree in Chemical Science from the University of Liège. Soon after my studies, I attended sales and marketing training. My goal was to enter the medical industry and I successively worked for Smith & Nephew (Medical Devices), Baxter (Plasma and Recombinant portfolio), Shire (Haematology and Hyperactivity) and Eurogenerics (Generics). I have always been based in Belgium but worked

“My passion has always been to shape the healthcare environment in order to have an impact on the quality of care and therapeutic access that is provided to patients.”

several years for the Dutch market as well. I speak French, English and Dutch. I live in the Brussels area.

Q What is your proudest professional achievement?

It's the development of a Patient Adherence project in hematology. Generally speaking it is well known and proven that patient adherence to treatment is not optimal. I have thus developed a project to support and educate patients through written material and nursing support in order to improve their adherence, disease knowledge and potentially clinical outcome. I had established an internal multidisciplinary team and consulted physicians in order to consider all aspects of the projects. It ended in higher patient satisfaction rate and more optimal use of the related medicine.

Q What is most rewarding about working in this industry?

My passion has always been to shape the healthcare environment in order to have an impact on the quality of care and therapeutic access that is provided to patients. As an example, I will always remember when I came back to the office on a Sunday to send clinical literature to a pediatrician hematologist who was then able to save the life of a baby suffering from an ultra-rare deficiency. ●

GLOSSARY OF TERMS

AABB	American Association of Blood Banks
ABO	Alliance of Blood Operators
ABP	Alternative Benefit Plan
ABRA	American Blood Resources Association
ACA	Affordable Care Act
AGES	Austrian Agency for Health & Food Safety
BODI	European Board of Directors
BPD	Biological Product Deviation
CASI	Computer assisted self-interview
CBS	Canadian Blood Services
CCIO	Center for Consumer Information & Insurance Oversight
COE	Council of Europe
DHQ	Donor History Questionnaire
DHQC	Donor History Questionnaire Committee
EAHP	European Association of Hospital Pharmacists
EBA	European Blood Alliance
EC	European Commission
EDQM	European Directorate for the Quality of Medicines & Healthcare
EMA	European Medicines Agency
EPCC	European Plasma Collectors Committee
EU	European Union
FDA	Food and Drug Administration
FPL	Federal poverty level
HCV	Hepatitis C Virus
HEV-RNA	Hepatitis E Virus Ribonucleic Acid
HHS	Department of Health & Human Services

HPSC	European Health Policy Steering Committee
IFTF	Immune Globulin Flashpoints Task Force
IG	Immunoglobulin
IPAW	International Plasma Awareness Week
IPPIA	International Plasma Products Industry
ISBT	International Society Blood Transfusion
MDPC	Medical Policy Committee
NAT	Nucleic Acid Test
NCA	National Competent Authorities
NHLBI	National Health, Lung and Blood Institute
NIH	National Institutes of Health
ÖSPID	Austrian Support Group for Primary Immunodeficiency
OCABR	Official Control Authority Batch Release
OMCL	Official Medicinal Control Laboratories
PCIP	Pre-existing condition insurance plan
PCR	Polymerase chain reaction
PDMPS	Plasma Derived Medicinal Products
PEI	Paul-Erlich-Institut
PGSC	PPTA Global Steering Committee
PHVC	Pharmacovigilance Committee
PID	Primary Immune Deficiency
PLUS	Platform of Plasma Protein Users
RBDM	Risk based decision making
RIBA	Recombinant immunoblot assay
RPSC	Regulatory Policy & Compliance Steering Committee
SD	Solvent Detergent

Upcoming Events

CONFERENCES
& SYMPOSIUMS

February

26–28 EHAD Annual Meeting
Brussels, Belgium

March

11–12 International Plasma Protein Congress
Vienna, Austria

18–21 International Symposium on Intensive
Care and Emergency Medicine
Brussels, Belgium

May

1–4 Canadian Society for Transfusion
Medicine Annual Scientific Conference
Québec City, Canada

8–10 7th European Conference on Rare
Diseases & Orphan Products
Berlin, Germany

11–15 WFH 2014 World Congress
Melbourne, Australia

21–22 IPFA/PEI 21st International Workshop
on “Surveillance and Screening of
Blood Borne Pathogens”
Rome, Italy

31–June 5 33rd International Congress
of the ISBT
Seoul, South Korea

June

26–27 Plasma Protein Forum
Washington DC

October

25–28 AABB Annual Meeting
Philadelphia, Pennsylvania

26 PPTA Business Forum
Philadelphia, Pennsylvania

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Procleix®

Procleix® Solutions | SETTING NEW STANDARDS IN SAFETY & OPERATIONAL EFFICIENCY

Procleix® Plasma Testing Solutions

By Gen-Probe and Novartis Diagnostics

Optimize your plasma testing operations with a 5-NAT assay menu and high-throughput, fully automated and integrated NAT platform.

For high volume plasma testing operations Procleix NAT solutions streamline and standardize testing across sites worldwide with:

- a comprehensive assay menu*
- the ability to efficiently screen and discriminate on the same powerful and efficient platform
- the flexibility to easily adapt to growing volumes with minimal instrument and space requirements

Ask your local Novartis Diagnostics representative for more information or visit our website at www.novartisdiagnostics.com

*HIV-1, HCV, HBV, Parvovirus B19 and HAV



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