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Dutch Newspaper Reports on High Cost of Blood

Ethical Debates on Donor Compensation

Does saving lives trump naysayers?

Demystifying Residual Risk
Understanding the probability of an infectious donation

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Meet the PPTA Staff
Sonia Balboni, Manager, Source and Standards
Johan Reinhoudt, Executive Director, PPTA Europe

UPCOMING EVENTS AND SYMPOSIA
WE ARE APPROACHING THE END OF ANOTHER YEAR.
My grandmother always told me that time goes faster when you are getting older, and indeed, she was right. Time flies.

This year was a very special one. We celebrated our 20th anniversary, in June, in Washington, DC, and we are still getting compliments for it. It was a nice occasion to recognize many individuals who have been instrumental to establish what is now called PPTA. Also, it provided a great opportunity to gather so many stakeholders to celebrate this special event.

On October 1st, I was pleased to recognize in person one of the founders of the Association, Mr. Guelfo Marcucci. In the presence of his family I presented him with a special recognition to honor him. Once again, Guelfo Marcucci, thank you very much for your vision to help build the strong Association we have become.

While I was writing this column on a plane ride from Europe to the USA, I read a short news article in a Dutch newspaper, De Telegraaf (November 14, 2012). It was a small article and I translate:

“Hospitals pay a too high price for blood, because blood bank Sanquin is the only organization in The Netherlands is allowed to supply red blood cells”. That is the conclusion of Professor Barbara Baarsma of the University of Amsterdam. She investigated why blood is so expensive in our country. Not enough competition is her conclusion. In The Netherlands, a hospital pays around 120 Euro for a unit of red cells. That is double than what is paid in Germany and Belgium....”

I remember that I raised this issue in my column in the summer 2008 edition of THE SOURCE. I also questioned the allocation of costs and wrote about the potential of cross subsidies. I still believe that the public activities (blood collection) and private activities (manufacturing of plasma protein therapies) need to be split into two different entities.

Now after two independent reports (Plexus and Conquaestor), a letter of the Minister of Health (demanding an internal acquisition price of Euro 85 per liter) and this independent publication, I feel that we are moving in this direction.

I want to thank all volunteers and staff in our Association for another great year. ☺
INTERNATIONAL PLASMA PROTEIN CONGRESS

March 5, 2013 | 9:00 - 17:30 hrs
SESSION 1: KEYNOTE SESSION | 09:00 - 10:30 hrs
• Keynote, Irish Ministry of Health
• Industry Update, PPTA Chairman

HILFENHAUS AWARD | 10:00 - 10:30 hrs
SESSION 2: REGULATORY CONTRIBUTION TO PATIENT ACCESS | 11:00 - 12:30 hrs
• Provisions for clinical trials in Europe – Regulator perspective, A. Hilger, European Medicines Agency
• Provisions for clinical trials in Europe – Industry perspective, M. Particco, Kedrion
• Pharmacovigilance, European Medicines Agency
• FDA: Latest Development, Food & Drug Administration

SESSION 3: PLASMA PROTEINS: TREATMENT FOR RARE DISEASES | 14:00 - 15:30 hrs
• Impact of rare disease policies on access to therapies, R. Gatermann, CSL Behring
• Sustainable Rare Disease Model – EU Level, TBC
• Experience and lessons learned from Ireland, TBC
• Plasma Proteins – cost effective evidence based treatment, A. Farrugia, PPTA

SESSION 4: INTERNATIONAL ISSUES | 16:00 - 17:30 hrs
• China Haemophilia – emerging need, D. Kong, China Hemophilia Rehabilitation Federation
• PID Treatment in North Africa, A. Bousfiha, Moroccan Society for PID

March 6, 2013 | 8:45 - 16:00 hrs
SESSION 5: KEYNOTE SESSION | 08:45 - 09:45 hrs
• Plasma Proteins and Rare Diseases – what can we learn from experience?, P. Robert, The Marketing Research Bureau

SESSION 6: SELF-SUFFICIENCY | 09:45 - 10:45 hrs
• National, Regional, Global – debate, J. Bult (PPTA) and B. Perry (IPFA)

SESSION 7: PLASMA | 11:15 - 12:30 hrs
• Donor Motivation, M. Macis, John Hopkins University, Baltimore
• Donor Safety, J. Penrod, PPTA
• Plasma Center Inspections – where are we going?, TBC

SESSION 8: CLINICAL DEVELOPMENTS | 14:00 - 16:00 hrs
• Alzheimer’s disease and Albumin, A. Paez, Grifols
• German Initiatives in PID Treatment, V. Wahn, Charite
• Neurology and Immunoglobulin, TBC

CLOSURE OF IPPC2013 | 16:00 hrs

For more information visit our website at WWW.IPPC.NET
THE SOURCE PLASMA COLLECTION INDUSTRY COMPENSATES DONORS. Beginning with that simple statement, we can trace the threads of many controversies and questions surrounding the industry. Countless position papers, policies, and protocols have sprung up in answer to the phenomenon; entire regulatory and legal structures have developed as a result. Few practices can generate such a degree of debate and concern, and yet compensated donation is also a practice which saves and improves the lives of millions of people around the world. This article presents a brief treatment of the discourse and suggests that a modified consideration of the nature of the industry and plasma therapies should inform ethical views.

Framing the Dialog
Many questions feed the donor compensation debate: Is the plasma safe? Is there enough plasma to make the therapies? What kinds of people donate? Similarly, many half-truths exist as rumors haunting the ends of these questions, such as plasma’s uses, the characteristics of plasma donors and donation, and the character of the industry. Preconceptions and misconceptions distort honest dialogue about compensation. All of the questions asked should be given full attention and answered in a straightforward fashion, but the largest question remains: Is it ethical to compensate donors?

Human action invariably carries the weight of ethical concern. We cannot hope to answer all of the questions now, especially since individuals firmly rooted in a specific mindset will not only find it impossible to budge from that position, but also be incapable of taking on any perspective other than their own.

The debate includes several different models with which one can examine the ethics of compensated plasma donation; one can speak in terms of deontological, virtue ethics, consequentialism, or many of the other schools of thought. Some questions also exist on the periphery, which help define the boundaries of the discussion: legality, safety, economics, medical diagnosis, epidemiology, and so on.

Questions about legality align strongly with those of ethics. The ethics of a society or a nation result in policies, which are given shape and life by the law. The degree to which the law gives full voice to the policies is a question that is at least as old as organized society itself, and cannot be addressed here. Instead, what we can say in regard to the industry is that differing regulatory and legal structures have allowed plasma collection to occur and have, in turn, saved many thousands of lives. Implicit in this argument is, of course, a utilitarian strain, which perhaps is the basis of many legal formulations.

Plasma Safety
But is the plasma safe? After the decades of safety relating to the plasma collection and therapeutic manufacturing industries, all of the technological advancements in testing, viral inactivation, and strong measures including robust regulatory structures and complementary industry standards programs, fortify the efforts to make source plasma as safe as it can possibly be. In fact, with no reported viral transmission in any plasma protein therapy manufactured by PPTA members since 1994, the safety record of therapies made from source plasma stands among the best in the world.

Some may object to the argument that plasma therapies have extraordinarily high levels of safety, noting that source plasma goes through a multitude of viral inactivation, purification, and processing steps, and thus, comparisons of viral transmission are not inherently valid. In an important sense, this is correct. They are, in fact, two different types of products. In fact, comparing source plasma to transfusable components is senseless, on an ethical basis. Proclamations such as that of the World Health Organization (WHO) not only strive to make a comparison with an underlying erroneous premise, but also compare two types of products that should...
be medically and scientifically differentiated. Differentiating compensation policies and the ethical bases for each paradigm is crucial to fully appreciate of the needs of the patients for plasma therapies. Much like no one medicinal product treats every ailment, why should the expectation be that different products be evaluated on an identical basis?

A Global Need

An indisputable fact stands as a further guide to the debate: without plasma protein therapies, people will die. With insufficient diagnosis, people will suffer. With inaccurate assessments as to clinical need, people will remain ill, often gravely so. The framing of this line of argument fits squarely within the realm of a utilitarian view. It helps us answer questions as to the needs for plasma protein therapies for patients and for compensation of donors. The element of donor compensation, however, comes under greater scrutiny: While it would be difficult indeed to ethically claim that patients who need life-saving therapies should not receive such treatment, it does not answer to whole question as to why donors should be or need to be compensated. For many, the fact that the therapies can be created without plasma from compensated donors is ipso facto proof that compensation is unnecessary.

Economics would suggest otherwise. Over the past several years, plasma collections in the United States have hovered around 20 million annually. The usage of the finished products has shown similar trends. Diagnosis of these conditions is a fundamental initiative within many patient groups, along with education regarding the need for policies that support access and the right of patients to choose the course of their therapies. Despite questionable attempts to use disputed medical diagnosis as a cat’s paw for an economic argument, the reality of the patients’ needs speak for themselves. We in the plasma protein therapies industry have no hesitancy in engaging on ethical issues, to patients, and ultimately to the donors. One size does not fit all, and applying the same phrases to understanding the industry but reflects the separate nature of the therapies. This also means that differentiation of plasma and plasma products is not only key to understanding the industry but reflects the separate nature of the industry. One size does not fit all, and applying the same phrases without considering the distinctions is to do a disservice to the industry, to patients, and ultimately to the donors.

Not Just One Motive

The economic discussion is more than the macro-level view of patient treatment and access. We should also examine the smaller scale, the individual decisions and circumstances that lead to an individual course of action for a particular person; in this case, we can look at donor motivation. One important question is whether a person can have more than one motive for taking a course of action. For many of us, this describes earning a living: it generates income in return for performing a task or making a product and for many it also gives a sense of well-being, of contribution, of accomplishment. Deriding multiple motivations would mean that we would all either have to work for free, or consign ourselves to jobs we hate solely to earn enough money to survive. Just as the interaction between an individual and his or her environment forms a conception of necessities and desires, an overly simplistic view dictates a centralized approach that does not appreciate the full complexity of everyone’s individual life. We all do things for a variety of reasons; therefore, our expectations of a plasma donor’s motivations should not be so simplistic. Some people get motivation from money, others from the recognition accorded by the voluntary sector. Are we to conclude that individuals as research shows, would certainly not donate if this recognition is not accorded, are to be excluded?

Where does this leave us? Ethical questions are complex, certainly, and perhaps the best course is to understand that ethical theory does not provide complete answers to all of our many questions. Many persuasive, practical reasons exist for the presence of policies favoring donor compensation, along with sound ethical reasons. The clearest path forward, however, is to forge an understanding that the reality faced by patients relying on plasma therapies is defined by the uniqueness and separateness of the therapies. This also means that differentiation of plasma and plasma products is not only key to understanding the industry but reflects the separate nature of the industry. One size does not fit all, and applying the same phrases without considering the distinctions is to do a disservice to the industry, to patients, and ultimately to the donors.

Joshua Penrod, Vice President, Source
Prof. Albert Farrugia, Vice President, Global Access

1 Harvey Alter, Blood Products Advisory Meeting, April 28, 2011.
8 Nicola Lacetera, Mario Macis. Incentives for altruism? The case of blood donations. On http://www.voxeu.org/article/incentives-altruism-case-
THE IMPORTANCE of monitoring the risk of infectious disease transmission by blood transfusion is well established and calculations of this residual risk are routinely performed since donor infectious disease incidence rates change and test sensitivities improve. For blood products that are transfused the residual risk represents the chances of a patient receiving a potentially infectious unit. However, for source plasma, collected units are not transfused and residual risk has a different meaning. The Residual Risk (RR) becomes the estimated probability of a potentially infectious unit entering the manufacturing pool. This article describes how RR is used to help ensure plasma safety.

3 O'Brien SF, Yi QL, Fan W, Scalia V, Kleinman SH, Vamvakas EC. Current incidence and estimated residual risk of transfusion-transmitted infections in donations made to Canadian Blood Services. Transfusion 2007;47:316-
In spite of the high levels of sensitivity in methods used for screening blood and plasma, false negative results may occur because screening tests are unable to detect the infection until a donor's blood or plasma reaches a certain level of analyte detectability. This period between infectiousness and detection is referred to as the infectious window period. The presence of certain viruses in asymptomatic donors who are negative on the screening tests (window period donations) constitutes the major risk of transmission of viruses in blood and plasma products. These must be accounted for in the calculations which are performed.

A number of safety measures have been taken in a way that closes gaps between safety measures. This creates a multi-layered approach which includes stringent behavior and laboratory screening procedures to help ensure plasma safety. Also, the plasma industry has taken additional steps to address safety concerns. These are covered by the International Quality Plasma Program (IQPP) and the Quality Standards for Excellence, Assurance and Leadership (QSEAL).

Two components of these are important in discussing residual risk: the Qualified Donor standard and the Inventory Hold standard. The Qualified Donor standard requires that a donor successfully pass two separate screenings, including infectious disease testing, to be accepted as a plasma donor. Only, source plasma from Qualified Donors is used for fractionation, minimizing the risk of a window period unit. In 1996, the plasma protein industry instituted a 60-day hold policy whereby all source plasma units are held for a period of 60 days after collection during which any additional information received such as test results and post-donation information can be considered prior to release of the unit for manufacturing. The hold period has proven to be effective in interdicting potentially infectious units and has greatly reduced the residual risk of an
infected unit entering the manufacturing pool. This assessment is the purpose of the residual risk calculation.

The window period risk for infection (RR) is an easy calculation, at least for whole blood:
\[
RR = \lambda \times wp
\]
where \(\lambda\) = incidence rate in repeat donors (new cases/person -years of observation) and \(wp\) = the window period for the tests of record.

For whole blood and blood products, an adjustment for first-time donors is added to account for the fact that donations from these individuals are transfused and first-time donors have higher incidence rates since they are not pre-screened. No correction is required for source plasma since only Qualified donations are used.

Accounting for the effect of the inventory hold complicates the calculation of RR. If a donor tests positive for a tested virus, all of the donor’s units in storage are removed from distribution. Donation frequency becomes a critical factor. Donors who return at shorter intervals who subsequently make a serological or Nucleic Acid Test (NAT) positive donation would likely have more donations interdicted by the hold period. To account for the hold, PPTA developed an empirical model for risk estimate that looks at all donations over a given time period. Overall, industry RRs are calculated annually. Thus the estimates represent the effect of changes in the donor population along with differences in donation frequency. In addition, RR can be used to assess the impact of industry safety initiatives and test procedures.

The European Medicines Agency (EMA), in their “Guidance on epidemiological data on blood transmissible infections”4 requests Plasma Master File (PMF) holders to estimate “the risk of infectious donations of repeat tested donors passing through routine testing, due to collection of donations that are truly negative to the tests in use.” These are the window period donations. This calculation, however, differs considerably from the source plasma estimate since it discounts the hold period and includes as repeat donors a proportion of donors who would not be Qualified. This represents the risk that a seroconverting donor gave a non-detectable infectious unit during the window period and will be higher than our estimate of the probability of an infectious unit being released for manufacturing. EMA acknowledges the industry source plasma risk reduction steps and for submissions under the Guideline request that their benefit be presented in terms of the overall safety strategy.

How are we doing in ensuring product safety? Figure 1 shows the Residual Risk for HIV and HCV for U.S. Source Plasma collections for 2001-2010. The low and decreasing risk of a potentially infectious unit being released for manufacturing is currently less than 1 per million donations for HIV and about 1.5 per million for HCV. From 2001-2010 the rates have decreased 42% and 66% for HIV and HCV respectively. Similarly, the decrease in the RR for HBV (Figure 2) is 58%. The RR for HBV is higher due to the substantially longer window period.

An important point to note is that the risk estimates do not represent the risks after fractionation. Fractionation essentially removes or inactivates the three viruses and reduces the risk of viral transmission essentially to zero for the end product.

The risks of an infectious unit being released for manufacturing are rare. How do they compare to the odds of dying from some common and not so common events? (Table 1) Your risk of dying from a hospital borne infection over your lifetime is 26,316/1,000,000 compared to the risk of an infectious unit being released of between 1-13/1,000,000. The chance of being killed by a dog or an asteroid impact in a lifetime is about the same magnitude as having an infectious unit released for manufacturing. The low risk reflects the impact of measures industry has taken to maximize plasma safety. This low risk coupled with the critical and highly effective removal and viral inactivation ensure the safety of plasma protein therapeutic products.

Our discussion has been restricted to the classical incidence/window period method. Several modifications have been applied for estimating transfusion risk, but a detailed comparison is beyond the scope of this article. RR is but one of the tools in the armamentaria for guaranteeing plasma safety. For monitoring Source plasma the residual risk empirical method used has been widely accepted. It is hoped that this brief discussion has helped the reader appreciate the role of residual risk and has clarified the underlying model.

George Schreiber, Director, Epidemiology

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Table 1

<table>
<thead>
<tr>
<th>Odds of Dying: What we should fear</th>
<th>Odds per 1,000,000</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cancer (lifetime)</td>
<td>142,857</td>
</tr>
<tr>
<td>Hospital Infection (lifetime)</td>
<td>26,316</td>
</tr>
<tr>
<td>Complications of Medical Surgical Care (lifetime)</td>
<td>762</td>
</tr>
<tr>
<td>Exposure to Forces of Nature (lifetime)</td>
<td>574</td>
</tr>
<tr>
<td>Drowning in a Bathtub (lifetime)</td>
<td>9</td>
</tr>
<tr>
<td>Killed by a dog (lifetime)</td>
<td>9</td>
</tr>
<tr>
<td>Asteroid Impact (Lifetime)</td>
<td>5</td>
</tr>
<tr>
<td>Struck by Lighting (year)</td>
<td>2.5</td>
</tr>
</tbody>
</table>

**Versus Risk of:**

A Potentially Infectious Source Plasma Unit Making it to the Manufacturing Pool | 1 - 13

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Winter 2012

8 THE SOURCE | Winter 2012
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The Procleix Parvo/HAV Assay is an in-process test that is available for commercial sale in the U.S. The performance specifications have not been reviewed and approved by the FDA. The Procleix Parvo/HAV Assay is an in-process test and blood screening test available for sale in the EU. Intended uses and commercial availability vary by region.
In 2008, at the Annual General Assembly of the European Society for Immunodeficiencies (ESID), experts concerned with the low diagnosis rate of primary immunodeficiency (PID) in Germany, laid the foundation for what today is known as the FIND-ID project. Tim Niehues, Ph.D. of Helios-Clinic Krefeld, Volker Wahn, Ph.D. of Charité Berlin, and Gabriele Gründl from the patient organization, Deutsche Selbsthilfe Angeborene Immundefekte (dsai), as well as Fred Modell, Jeffrey Modell Foundation wanted to increase the diagnosis rate and improve treatment for PID patients in Germany. The idea of FIND-ID was born. PPTA and its membership agreed to support the initiative.

Today, FIND-ID is one of the key drivers for raising awareness and improving treatment in Germany. The goal of the network is to increase awareness of PID among physicians and overcome structural barriers which endanger adequate treatment for patients. The physician network has more than 65 members and is guided by a Steering Committee which consists of leading German pediatricians, Tim Niehues, Ph.D., Volker Wahn, Ph.D., and Fred Zepp, Ph.D. and internal medicine experts Karsten Franke, MD, Reinhold Schmidt, Ph.D., and Klaus Wannatz, Ph.D. Their commitment and voluntary work has been fundamental to the success of the initiative and has dramatically improved the situation in Germany.

The FIND-ID network has raised awareness by exhibiting at numerous medical congresses, and offering a number of medical workshops and symposiums. The FIND-ID website (www.find-id.net) specifically targets physicians and also includes information for patients. In addition, the FIND-ID Steering Committee provided input for articles which were then published in relevant scientific journals. A comprehensive press and media campaign supports all of these activities in an effort to reach the general public.

Despite the progress made, there is still a long way to go to achieve early diagnosis and adequate treatment for all patients in Germany. Going forward, the FIND-ID leadership will continue to work hard to improve the infrastructure for the treatment of PID patients. This is particularly challenging as clinical immunology is not a field in which physicians can specialize in in Germany.

The FIND-ID network has demonstrated that raising awareness, increasing the diagnosis rate and treatment improvements for a rare disease can be achieved if all relevant stakeholders work together. There is evidence that the number of patients in FIND-ID centers is on the increase. FIND-ID is a testament to what a group of committed and engaged people can accomplish. Thanks to the commitment of the FIND-ID Steering Committee, PID patients in Germany have a much better likelihood of early diagnosis and adequate treatment.

Stefan Grafenhorst, Senior Manager, Germany
Our Mission

QualTex Laboratories is dedicated to supporting global public safety with the timely delivery of high quality testing services for patients, donors, and regulated biological products.

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- Nucleic Acid Testing
- Immunohematology Reference Lab
- Microbiology Testing
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- Supports multiple industries
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- FDA registered
- EU GMP certificate of compliance
- German Health Ministry certification
- ISO9001:2008 certified
- Active research & development
How old are you?
I am 25 years old.

When did you first have symptoms?
I never had any problems as a child. It started when I was 14 or 15 years old. At that time I got very severe pneumonia. After that, I was sick very often.

Did you go frequently to the hospital?
No, I have not been in the hospital very often. But I suffered frequently from infections in my lungs. I had to stay home and take antibiotics. Unfortunately, those never made me feel better. I missed many days in school, which did not make it easier. I never finished my professional education. But I think that was not necessarily related to my disease.

When were you diagnosed with immunodeficiency?
I was diagnosed only recently, around 9 months ago. Shortly before I was diagnosed with bronchiectasis. I thought, now that they know what I’m suffering from, I will finally get better. But I did not. The physicians had to take more tests and then they finally diagnosed the immunodeficiency.

How did you react?
Immunodeficiencies are very rare diseases. I do not blame the physicians that they did not think about it immediately. I had always seen general physicians never any specialists. When I went to that specialist for the first time, he diagnosed me very quickly. That was a big relief.

How often do you have to go to the hospital to get your medication?
Every 2 months I go to the hospital for a check-up. They monitor the evolution of my disease and check for secondary disorders. This exam takes about 45 minutes and is very important for me.

Are other family members affected?
My uncle has not exactly the same disease but he has also some kind of mild immunodeficiency. Apart from that, nobody in my family suffers from immunodeficiencies. Maybe someone from my father’s side but we do not know that for sure.

What do you do for a living?
I am working on my high school diploma and work on the side. I would like to study physics.

Does the disease affect your everyday life?
No, thanks to the medication I am less sick than before. I started taking the medication a couple of months ago. I have not yet experienced a whole winter but I can definitely say that I feel already much better.

What do you know about your medication (Immunoglobulin)?
I have received an explanation about what kind of medication it is. It is made from human plasma and it is very expensive because the manufacturing is very complex. I have also been educated about my disease.

Would you be interested in visiting a plasma collection center to see how the plasma is collected that is used to manufacture your medication?
No, not really. I do not think that is important for me. I do not want to focus too much on my disease. I want to live. I know there is medication that makes me feel better and there are physicians that take care of me. Apart from that I don’t want my disease to take more space in my life than it already does. I admire people that are very engaged for a cause but I would like to distance myself from that.

What would you like to tell other afflicted persons?
It always depends of the degree of your disease. I have a friend; she also suffers from an immunodeficiency but on a more severe level. She is not able to manage easy things. For her, leaving the house alone or taking public transport is impossible. The fact is, you have to pull yourself together. That means you have to renounce a lot of things. But in the end that brings you further. I have to live healthy, that means eating fresh fruit and vegetables, drinking a lot of water. That makes me feel good and improves my body awareness.

Do you exercise?
Yes, more than ever. Recently I have started to run intensely. I wanted to prove myself that I was physically capable of doing it. It is very stimulating. I would like to run a marathon in the future. ☺️
Thank you for partnering with us for more than 40 years

- An ongoing commitment to donor safety and serving the needs of the plasma industry worldwide
- Reliable, proven partner with close proximity to our customers in all regions
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Visit our new website at www.haemonetics.com to learn more.
The Czech Republic Makes its Mark

by Sonia Balboni

This year, we celebrated the first ever International Quality Plasma Program (IQPP) certifications of plasma collection centers in the Czech Republic. It is also the first time in twelve years, that a plasma center has become IQPP certified outside the United States, Germany or Austria. This milestone signifies the strong contributions of the Czech Republic to the global plasma collection market. More importantly, it indicates the growing depth of the infrastructure supporting source plasma collection.

When UNICAplasma, s.r.o. earned IQPP certification this year, its President Milan Malý stated, “Getting the IQPP certificate is a key step in our pursuit of perfection. It will allow us to demonstrate both to our donors and to the relevant authorities how important quality and safety are to us.”

There is a growing recognition worldwide that reliable availability of final product therapies is difficult to achieve through whole blood donations alone. Collection of source plasma by private companies is therefore becoming more accepted around the world. This results in a greater availability of healthy and more committed donors to supply the needed plasma. Ultimately, this translates into a steady supply of lifesaving plasma protein therapies for patients.

“We are proud to play an important role in the Czech Republic, collecting plasma from Czech donors that can be used to prepare therapies that ultimately save lives. The Czech Republic has long been a strong contributor to the European plasma collection landscape. Europlasma, s.r.o. looks forward to continuing its presence in the country for the long term,” says Rudolf Meixner, CEO of Europlasma Group.

The road to keeping the Czech market open has not been easy in recent years. Local collectors, with the assistance of PPTA, have had significant dialogue with the country’s authorities in an effort to better inform lawmakers, health
officials and other policymakers about the need to keep private collection viable in the country. They made the case, in large part by explaining the public health benefits that result from an infrastructure supporting private collections.

Although navigating the recent changes in Czech policy has created uncertainties, we now have an increased understanding of the issues that collectors commonly face in the country. PPTA also strengthened its affinity with its Czech colleagues, and through this experience our members in the region have become more vocal within the Association.

Much attention has been paid to this country recently, and deservedly so. The bottom line is that the Czech Republic is important to the industry and the many patients who rely on Plasma Protein therapies.

*Sonia Balboni, Manager Source and Standards*

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“*I have been a plasma donor since 2009. I was motivated to donate plasma by my schoolmate, who also recently began donating. I believe that plasma donation is an act of humanity, a gift. In my leisure time I mostly play sports. I also like to spend time going on trips with my friends and family.*”

Lucie Obermannová, Plasma Donor
DCCH Center, Chodov, Czech Republic

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**Country Profile**

| 2011 Population (est.): 10.5 million |
| Area: 78,864 sq. kilometers |
| Cities: Capital: PRAGUE (POP. 1.26 million) |
| Other cities: BRNO (384,000), OSTRAVA (310,000), PLZEN (373,000) |
| Nominal GDP (2011 est.): $272.2 billion |
| GDP per capita (2011 est.): $25,923 |
| Annual GDP growth rate (2011, Czech Statistical Office): 1.7% |

*Source: United States Department of State*

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**Plasma Collection and Manufacturing in the Czech Republic**

Allowed donation frequency: **One donation every two weeks**

Private Collection: **Yes, four companies currently operate, with 11 centers in total**

Public (state)-managed Collection: **Yes**
THE SOURCE INDUSTRY PROFILE COMMITTEE (SIPC) (see p. 20) recognized the need to leverage the value of International Quality Plasma Program (IQPP) certification, as well as, member’s investment in obtaining and maintaining certification. The standards programs provide an ideal platform for raising public awareness on the safety and quality of life sustaining plasma collection and plasma protein therapies. The Committee strongly supported updating the brand image and creating a promotional campaign that would be executed unilaterally in all IQPP collection facilities worldwide.

The brand update includes a new, refreshed IQPP logo, a door decal for centers to display and an IQPP certification badge to post on company websites. A new framed certificate will be sent to all centers for display and consumer oriented version of the standards “What is IQPP and Why is it Important to You?” has been developed. In addition, PPTA will announce new center certifications in the Leadership Briefing and recognize five-year certification milestones on its global website each month beginning in 2013. These milestones will also be promoted to local media and elected officials. Centers are encouraged to host an annual donor appreciation day and consider inviting local media and policymakers.

To complement the brand update, a new logo was also developed for “Donating Plasma”. These websites are designed to reach the general public and potential donors. All of the project materials have been developed in English, German and Czech with the goal of distribution to all IQPP collection centers in Europe and North America by year’s end.

The IQPP branding project lays the foundation for a broader public awareness campaign that will include a major redesign of each of the donating plasma websites, PPTA’s global website and an annual recognition of plasma donation via International Plasma Awareness Week. These combined initiatives enable PPTA and member companies to effectively promote the importance of donating plasma and the Association’s standards programs on a global level. ☞

Lisa LoVullo, Senior Manager, Communications
INNOVATION IN ISSUES MANAGEMENT

THE STATE PATIENT ACCESS

BY BILL SPEIR

STATE MEDICAID AGENCIES ARE UNDER TREMENDOUS PRESSURE TO CONTROL COSTS given the slow fiscal recovery of the “Great Recession.” Education and Medicaid are by far the largest appropriation categories in state budgets. Given that no one wants to cut education spending, Medicaid is often the target for cost savings when states convene their legislatures.

Medicaid officials help state legislators find savings in their programs, and because of the high per recipient cost of Medicaid enrollees with hemophilia, blood clotting factor has been under great scrutiny by Medicaid pharmacy directors in the last few years.

As Medicaid pharmacy directors develop new cost containment strategies, there is a potential that individuals with hemophilia will lose access to their medically appropriate blood clotting factor and specialty pharmacy provider. To ensure patient access to their medically appropriate therapy and pharmacy provider, PPTA realized the need to work with others that share the goal of protecting patient access to blood clotting factors.

As a result, PPTA organized the State Patient Access Coalition (SPAC) which represents the world’s leading manufacturers of clotting factor and the nation’s leading distributors of clotting factor. In addition to PPTA members, SPAC manufacturers include Bayer Healthcare, Novo Nordisk and Pfizer. PPTA also sought out specialty pharmacies since they are the entities that dispense blood-clotting factor to Medicaid recipients, and they are most often the entities directly impacted by Medicaid cost-containment initiatives.

The SPAC’s goal is to inform decision makers in CMS, state Medicaid agencies, and state legislatures about the need for sound policies related to the purchase, dispensing and administration of blood clotting factor therapies. As part of this education, SPAC has developed Patient Access Principles that urges decision-makers to consider the National Hemophilia Foundation’s Medical and Scientific Advisory Council (MASAC) Recommendations 188 and 159 when implementing Medicaid policies that impact patient access to clotting factor.

MASAC Recommendation 159 states, “Clotting factor therapies are neither pharmacologically nor therapeutically equivalent and vary based upon purity, half-life, recovery, method of manufacture, viral removal and inactivation processes, potential immunogenicity, and other attributes. The characteristics of each product and the resultant product choice for an individual patient require a complex decision making process with the ultimate product being agreed upon by the patient and their respective healthcare provider. It is critical that the bleeding disorders community has access to a diverse range of therapies and that prescriptions for specific clotting factor concentrates are respected and reimbursed.”

MASAC 188 establishes standards of service for pharmacy providers of blood clotting factor for home use that must be met to ensure blood clotting factor is dispensed safely.

SPAC began with a conference call in April of 2011. Since that time, SPAC has been very active in addressing the need for patient access to all FDA approved blood clotting factors from all qualified specialty pharmacies. In 2012, we have addressed threats to patient access in a number of states including Alabama, New York, and North Carolina.

Bill Speir, Director, State Affairs

Alabama

On June 1, Alabama Medicaid implemented an emergency rule to limit Medicaid recipients to one brand pharmaceutical per month. It also put out a proposed rule for comment that would make the emergency rule permanent.

SPAC had multiple phone conversations with Alabama Medicaid. During these conversations, PPTA staff stressed the importance of providing Medicaid recipients access to their medically appropriate blood clotting factor.

Alabama Medicaid stated they were defining brand as one National Drug Code (NDC) per month. Due to the fact that factor concentrates are dosed based on the patient’s weight, it is not uncommon for an individual with hemophilia to require two different vial dosages of the same brand product to complete a prescription that is closest to the actual prescribed dose from the physician. Each vial will have a different NDC. This means Alabama Medicaid’s proposal might not even cover a complete prescription for a recipient that requires blood clotting factor.

SPAC’s comment letter pointed out that this would be a dangerous policy and contrary to Alabama Medicaid’s mission to “serve eligible, low-income Alabamians by efficiently and effectively financing medical services in order to ensure patient-centered, quality focused healthcare.” It also stated that the proposed rule was contrary to MASAC recommendations and would likely increase costs for other Medicaid services such as hospitalization. Based on the comment letters they received, Alabama Medicaid decided not to continue the one-brand limit and reverted back to the four-brand limit on August 1.
North Carolina Medicaid is creating a hemophilia specialty pharmacy program as required by the North Carolina budget bill for the current fiscal year. SPAC has attended a number of meetings with North Carolina Medicaid on the proposed program to ensure patients have access to all medically appropriate blood clotting factors and all qualified specialty pharmacies. SPAC made an educational presentation to the North Carolina Medicaid Pharmacy staff in November, explaining the manufacturing process for both recombinant and plasma-derived blood clotting factor as well as the numerous activities necessary to dispense blood clotting factor in accordance with MASAC Recommendation 188.

New York Medicaid proposed implementing a Medicaid reimbursement change for clotting factor based on Actual Acquisition Cost (AAC). The current Medicaid reimbursement benchmark is based on Average Wholesale Price (AWP). The switch to AAC was to begin on August 1, 2011. SPAC, working with NHF, has led an education campaign to ensure that New York Medicaid implements this change in a way that will not impact patient access. SPAC has participated in multiple meetings and conference calls with New York Medicaid on this issue.

The implementation of the AAC has not yet occurred. New York Medicaid appears to be working diligently to make sure patient access to blood clotting factor is not impacted by the reimbursement change. The implementation is currently scheduled for March 1, 2013.
MUCH OF THE ASSOCIATION’S WORK IS ACHIEVED BY ITS STANDING COMMITTEES. Together, with staff, members guide strategy, develop initiatives and respond to flashpoints. Members lend considerable time and expertise to issues across the spectrum of Association activities. In recognition, as well as to inform the membership about the important work being done, we are introducing a new feature in The Source: Committee Spotlight.

**Source Industry Profile Committee (SIPC)**
In 2005, a charter established the Industry Image Task Force charged with the task of creating a campaign to raise awareness of the source plasma collection industry to the general public. Due to differences between plasma collection in the U.S. and Europe, as well as, cultural and language differences the Task Force was divided and the Source Image Task Force (U.S.) was formed in 2008 and the European Source Image Task Force in 2009.

Those groups developed a series of print and digital communications including a donating plasma website in both English and German, videos, brochures and Fact Sheets, and launched an ad word campaign on both Google and Facebook.

In May of this year, the two Task Forces were merged to form the Source Industry Profile Committee (SIPC) with an emphasis on effectively improving awareness of the plasma collection on a global scale. The Source Board approved initiatives to be considered over the next several years: Leveraging IQPP certification for community education, an annual International Plasma Awareness Week to commence in October 2013, redesign the donating plasma websites and launch one in the Czech Republic, commission a White Paper on the ethics of compensated donation.

The group has successfully completed the IQPP project (see p. 16) and will devote the majority of their efforts to planning and executing the first International Plasma Awareness Week, October 13-20, 2013. Look for event details early next year.

The Committee is taking important steps in conducting outreach programs that raise awareness with the public about the quality and safety of plasma collection, plasma protein therapies and the positive impact they have on patients throughout the world.

**Lisa LoVullo, Senior Manager, Communications**

**IQPP Standards Committee (QPPS)**
“PPTA’s standards program provides global leadership for the plasma protein industry’s goal of continuous improvement with a focus on safety and quality from the donor to the patient. The Standards program will be transparent, credible, innovative and responsive to stakeholder and industry needs.”

The roots of the IQPP Standards Committee date back to the late 1980’s, when the ABRA Quality Assurance Program Committee (later called the ABRA Quality Plasma Program Committee, and eventually the QPP Steering and Coordinating Committees) began to develop the voluntary industry standards program. When the program became international in 2000, the IQPP standards Committee was born. As indicated in its charter, the Committee represents the Association in the areas of Standards development that relate to the source plasma collection industry. Specifically, the Committee is responsible for creating, maintaining, upgrading and optimizing the IQPP Standards Program.

Members of the Committee are on the front lines of plasma collection. Many have operations or quality backgrounds or come from senior management, and all visit their collection centers regularly. Therefore, they know the ins-and-outs of the collection business better than anyone else.

In addition to fulfilling the mission of its charter, the Committee, which is currently chaired by Ileana Carlisle (Biogen), also serves as an expert advisory resource for the Source Board of Directors. The Committee provides its expertise through recommendations to the Board on a variety of subjects, including usage of the National Donor Deferral Registry (NDDR), addressing standards implementation during test kit shortages, and developing new solutions to address common issues faced by collectors in their center operations.

Currently, the Committee is overseeing a task force that is preparing recommendations for revising the standards to address global relevancy. The breadth of the IQPP program is growing, as shown by two recent certifications in the Czech Republic (see related article, p. 14). The Committee is poised to address any new challenges that come its way. ❖

**Sonia Balboni, Manager Source and Standards**
On September 19, PPTA's Regulatory Policy & Compliance Steering Committee (RPSC) and the U.S. Food & Drug Administration's (FDA) Center for Biologics Evaluation & Research (CBER) held their annual Liaison Meeting in Rockville, Maryland. Nearly 40 people gathered to discuss topics of mutual interest, which ranged from final therapy pharmacovigilance, laboratory research, and drug shortages, to donor health, biovigilance, and compliance issues of biological product deviation (BPD) reporting and recalls. New legislation, such as the Food & Drug Administration Safety & Innovation Act (FDASIA), and other CBER initiatives and their effect on the plasma protein therapeutics (PPT) industry also were highlighted. Jay Epstein, MD, Director of CBER's Office of Blood Research & Review, who led the Liaison Meeting with Mary Gustafson, PPTA's Vice President of Global Regulatory Policy, opened his remarks by introducing the group to Peter Marks, MD, PhD, who recently joined CBER as the Center's Deputy Director.

As described in the fall 2012 edition of THE SOURCE, in May 2008, FDA launched the Sentinel Initiative, largely in response to legislation, to create a national, integrated, and electronic Sentinel System that performs “active” surveillance of product safety. The Agency’s pilot Mini-Sentinel exceeded—ahead of schedule—the legislation’s goal of 100 million patients in the system by July 2012. For the
second year in a row, David Martin, MD, MPH, Director of the Division of Epidemiology, represented CBER’s Office of Biostatistics & Epidemiology (OBE) at the Liaison Meeting and updated the group on the progress of Sentinel. While Mini-Sentinel already covers a large segment of the U.S. population, routine surveillance for PPTs, though planned, needs additional work before its institution. Dr. Martin also noted that the “passive” surveillance tool “AERS” now is referred to as “FAERS” (FDA Adverse Events Reporting System).

Later in the Liaison Meeting, PPTA raised an important issue for members, collectors and manufacturers alike: FDAs expectations of members’ paperless documentation systems during inspections. PPTA noted not only the increased ease by which electronic compared to paper records are collected, stored, and presented, but also the challenges that can arise with varying requests from FDA investigators when faced with paperless systems. These requests vary from asking that records be converted to paper for inspection to expecting free access to the company’s computerized systems. As more and more companies have been “going paperless,” the blood community also has flagged this issue as one that requires FDA to define boundaries in its training of investigators and in its Compliance Program Guidance Manuals. PPTA remains engaged on this issue and plans to continue the conversation with CBER by building on the foundation presented at the Liaison Meeting. Another issue in which PPTA will continue to engage CBER is concerning the codes used to report BPD to CBER. Several areas of ambiguity were highlighted at the liaison meeting and PPTA will work further with CBER to consolidate and refine the codes in order to have more accurate and uniform reporting.

During their meeting each fall, PPTA and CBER open a constructive dialogue on topics that are important to the PPT industry and the patients we serve. The group shares views and concerns and works toward meaningful solutions. Members are encouraged to share with their respective RPSC representatives any ideas that they may have for topics at future Liaison Meetings.
SONIA BALBONI
Manager, Source and Standards

How long have you served at PPTA?
I joined the Association in June 2010.

What do you focus on in your role as Manager, Source and Standards?
I work with the IQPP and QSEAL Standards Committees to develop and manage the implementation of voluntary industry standards relating to the collection of source plasma and the manufacture of final product therapies. I also deal with several complex issues relating to the Source division, including developments with the National Donor Deferral Registry (NDDR), and flashpoints.

Tell us about your background.
I hold a master’s degree in foreign trade and international business management, and an undergraduate degree in international affairs and Latin American studies. Prior to joining PPTA, I served as Director, Standards, for the Association for the Advancement of Medical Instrumentation (AAMI). In that position, I worked on the development of national and international standards, including devices for transfusion/infusion/injection, and biological evaluation and sterilization of medical devices. I have also held positions in Latin American regulatory affairs, market access and the computer and telecommunications industries.

I love to travel. To that end I have lived, studied or worked in Spain, Ecuador and Italy, as well as various regions in the United States. I speak fluent Spanish and conversational Italian and Portuguese. I have tried learning Chinese, but that unfortunately has been a “work in progress” for many years! My heritage is mainly Italian, Swedish, German and Norwegian. I am married with two boys, ages 3 and 4, and live in Maryland, which is also the state where I grew up.

What is your proudest professional achievement?
I am most proud of my work helping committee members to reach consensus on a broad range of issues. It is always gratifying to me to see individuals with divergent view points come up with an approach that satisfies everyone.

What is most rewarding about working in this industry?
Above all, I feel an immense satisfaction knowing that at the end of each day I have contributed, if even in a small way, to saving the lives of patients who use our industry’s therapies. I am inspired by our incredible patients, donors and colleagues here at the PPTA and within the industry.

Plasma is an amazing substance! I look forward to the coming years, as we continue to make new discoveries for its uses and its life-saving qualities.

Johan Reinhoudtt, has been named Executive Director, PPTA Europe. Johan has extensive and diverse hands-on experience (clinical, commercial, consulting, operations and scientific), with demonstrated success in executive leadership roles and experience in start-up, small, medium and complex large size organizational environments. He has lived and worked in the Netherlands, France, Germany and the United States. A graduate of the University of Leiden, the Netherlands where he earned a B.Sc. in Anesthesiology, Johan is certified in General Management by the INSEAD-CEDEP business school in Fontainebleau, France and by the Institute of Social Sciences in Utrecht, the Netherlands.

“I am passionate about rare diseases and feel great about the opportunity to lead PPTA’s activities in Europe,” Johan said in accepting the position.
## EVENTS
### UPCOMING CONFERENCES & SYMPOSIAUS

## 2013

### February 3 – 8
International Winter Symposium in Intensive Care Medicine
Grindelwald, Switzerland

### March 5 – 6
**International Plasma Protein Congress (IPPC)**
*Dublin, Ireland*

### March 19 – 22
33rd International Symposium on Intensive Care and Emergency Medicine
*Brussels, Belgium*

### April 22 – 25
19th Annual International Society for Cellular Therapy Meeting
*Auckland, New Zealand*

### April 23 – 24
IPFA/PEI 20th International Workshop on Surveillance and Screening of Blood Borne Pathogens
*Helsinki, Finland*

### April 24
Frontiers in Critical Care
*Amsterdam, the Netherlands*

### May 20 – 24
**HEMATOLOGY 2013**
- IV International Workshop on Hemophilia
  *La Habana, Cuba*

### May 23 – 26
8th C-1 Inhibitor Deficiency Workshop
*Budapest, Hungary*

### June 2 – 5
23rd Regional Congress of the ISBT,
*Amsterdam, The Netherlands*

### June 10 – 14
6th Annual Emerging Technologies in the OR and Great Fluid Debate
*Lake Buena Vista, Florida*

### June 11-12
**Plasma Protein Forum**
*Reston, Virginia*

### June 29 – July 7
XXIV International Society for Thrombosis and Haemostasis Congress
*Amsterdam, the Netherlands*
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