

# THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

Winter 2010



## Expansion of Medicaid

Under New Health Reform Law to Benefit Millions of Americans

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**Rho (D) Immune Globulin Saves Thousands of Lives Every Year**

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**PPTA Interview: Ileana Carlisle**

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**Donor Compensation: When Did the Controversy Start?**

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*In the interest of encouraging broad and open discussion of issues relating to plasma protein therapies, collection and fractionation,*

*THE SOURCE magazine may contain statements of opinion on such issues.*

*These statements are those of the author and do not necessarily reflect the opinion of PPTA or its members.*

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# IN MY VIEW

## HOW (NOT) TO DO BUSINESS IN JAPAN



Jan M. Bult  
PPTA President  
and CEO

**DOING BUSINESS IN JAPAN IS INTERESTING.** We all know that Japan has a unique cultural heritage, government, and language, which have traditionally placed massive barriers affecting trade between it and other countries. The present situation with Japanese patients who need plasma protein therapies is yet another example of government practices which set in place multiple roadblocks to the importation of off shore therapies. However, when the need is great, as it is for these patients, the hurdles can be overcome. But first, both parties must recognize that their joined efforts will benefit the many patients who depend on these lifesaving therapies to enjoy an acceptable standard of life. This sounds so easy, but is in sharp contrast with today's reality. Japan's Ministry of Health,

Labour and Welfare (MHLW) plays an important role for our industry. Within MHLW are multiple bureaus, which are further divided into divisions that look at specific issues. We deal primarily with the appointed officials of the Blood Products Division. Over the years,

PPTA has worked constructively with succeeding directors of this division and now meets once to twice a year in an Official Dialogue where both parties discuss relevant issues.

One of the hardest obstacles to overcome is dealing with newly appointed individuals who have little experience with the plasma protein therapeutics industry. The frequent rotation of directors for the Blood Products Division necessitates a steep learning curve in which each new director plays catch-up and only becomes experienced with the issues at stake after thorough training. Since the start of PPTA activities in Japan, I have worked with eight directors in 12 years. Each time a new director starts their two-year term, PPTA experiences the same drill: Explain the role of the PPTA and then listen to the story that there

is a Blood Law that needs to be respected. Then we are advised to work with the local Association, Kekkyo, "since we (sic) have so many issues in common." Our members are importing therapies and pushed out through the desire to achieve self sufficiency and therefore, we have another focus than the local Association.

Over the years, the situation has not improved. Globally we see that the volume of plasma for fractionation has increased significantly over the last 10 years. In Japan, it is stable with 1 million liters (+ or - 5 percent) per year. Worldwide, we see a growth in use of immune globulins, while in Japan it has been stable for the last 20 years. The prices of albumin for importing companies are (much) lower than the prices of domestic manufacturers. This is the result of mandatory price reductions every two years. When hospitals in Japan switch to imported therapies for cost-efficiency, the self sufficiency rate goes down. Since this is politically difficult to sell, hospitals are encouraged to reduce their consumption by financial rewards of up to 5 million Yen per year.

Additionally, Japan is the only country in the world where there is a labeling requirement to differentiate between domestic manufacturers "kenketsu" and importing companies "hikenketsu." The last term is a fabricated word in the Japanese language. This presents a negative tone when patients have to sign an informed consent before therapies are administered.

In my recent meeting I was told that Japanese doctors are well trained in medical school on primary immunodeficiency disease (PID). Knowing that there are 250,000 medical doctors in Japan and only 1,100 patients with diagnosed PID, it is clear that most physicians in Japan do not have the experience of treating immunodeficiency disease cases. Therefore, expertise needs to be developed.

Decision making by government officials in health matters is not always evident. A Japanese expert told me recently that children's vaccination programs are far behind programs in other countries. Historically, each official rotates out of office, decisions to adjust regimens are postponed and deferred to successors. As a result, the immunization program in Japan lags behind much of the developed world.

On a macro level, Japan has very good healthcare with high life expectancies. However, a macro approach for individual patients does not work. We need to continue to help Japanese government officials understand the specific needs of rare diseases. Patients need our help. ☺





International Plasma Protein Congress

IPPC

# IPPC TO ADDRESS CUTTING EDGE TOPICS

BY LAURA SAVINI

**E**ACH YEAR, PPTA's International Plasma Protein Congress (IPPC) brings the plasma protein sector together to explore the key issues facing our industry. 2011's wide range of speakers includes industry representatives, regulatory officials and patient representatives, providing delegates with the most cutting-edge expertise available on a number of topics and more.

Europe has dealt with sobering economic conditions as a result of the financial crisis. Governments have been forced to review and reconsider their expenditure, leading to increased pressures on the pharmaceutical sector.

Health Technology Assessment (HTA) - the buzzword of the day in healthcare economics - will also feature prominently with an update on the status of this evaluation technique, its impact on government decisions, and where all this is headed for industry.

The United Kingdom has been the first country in Europe to supervise and control administration of immunoglobulin. Professor Graham Sewell - one of the initiators of the Immunoglobulin Demand Management Plan and its database - will give an overview of the Plan and present key data from the database, with a focus on the implications for immunoglobulin use. This debate

would not be complete without the input of end-users of plasma protein products: the patients. What challenges do patients face in being recognized as legitimate stakeholders? What are their views on HTA? What structural barriers to treatment still exist?

One of the plasma industry's greatest strengths is its standards. Speakers will explore the status of these standards compared to official regulatory measures, and will consider if they are still necessary, and what can be done to improve them.

As always, IPPC 2011 takes a peek at what is happening outside Europe. This year speakers will present the current situation in low-index countries and Russia.

The dynamic debate surrounding plasma collection ethics will also continue in 2011, with an IPPC panel discussion devoted to plasma collection, epidemiological data and more.

Finally, regulatory agencies such as the European Medicines Agency, as well as key European Union Institutions, have reorganized the way they regulate pharmaceuticals.

Want to hear more about how this affects your business? Come to beautiful Lisbon for IPPC 2011! 🌐

LAURA SAVINI is PPTA's Assistant Manager, National Affairs, PPTA Europe



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## INTERNATIONAL PLASMA PROTEIN CONGRESS

### March 15, 2011 | 9:00 - 17:30 hrs

#### SESSION 1: OPENING SESSION | 09:00 - 10:30 hrs

- Experience with the UK Demand Management Plan (C. Sewell, Scunthorpe General Hospital)
- State of the Industry (PPTA Global Chairman)

#### SESSION 2: REGULATORY | 11:00 - 12:30 hrs

Chair: A. Hilger, Paul-Ehrlich-Institut

- Variations regulation: industry experience (D. Brazll, CSL Behring)
- Enterprise to Sanco: implications for patients and industry (S. Rohde, Rohde Public Policy)
- The European Medicines Agency: a European institution in the change of the time (G. Silvester, European Medicines Agency)

#### SESSION 3: THE WORLD WITHOUT INDUSTRY STANDARDS | 14:00 - 15:30 hrs

Chair: J. Blümel, Paul-Ehrlich-Institut

- Industry Standards: their role, implementation and future (J. Bult, PPTA)
- Do Industry Standards matter - a regulator's view (J. Kerr, Paul-Ehrlich-Institut)
- Nice to have or real value - a patient's perspective (J. Prévot, IPOPI)

#### SESSION 4: NEW DEVELOPMENTS AND INTERNATIONAL ASPECTS | 16:00 - 17:30 hrs

Chair: M. Skinner, World Federation Hemophilia

- Supply of plasma derivatives in low development index countries (J. Emmanuel, Consultant)
- Regime of haemophilia care in Russia: how did you get there? (Y. Zhulyov, Russian Haemophilia Society)
- Shifting sands of pathogen safety (T.R. Kreil, Baxter)

### March 16, 2011 | 8:00 - 16:00 hrs

#### SESSION 5: ACCESS TO CARE | 08:00 - 10:30 hrs

- Structural barriers to PID treatment (I. Schöndorf, FIND-ID)
- The important role of PID specialist nurses (A. Warner, International Nursing Group for Immunodeficiencies)
- New research from the IDF on the effectiveness of good diagnoses and treatment of PID (M. Boyle, Immune Deficiency Foundation)
- HTAs/CER comparative (European Commission)
- HTAs and Quality of life (B. O'Mahony, Irish Haemophilia Society)

#### SESSION 6: PLASMA | 11:00 - 12:30 hrs

- Donor motivation: data vs. dogma (A. Farrugia, PPTA)
- Epidemiology in donors in Germany (R. Offergeld, Robert Koch Institut)
- Arguing from different premises - missing the points, missing the opportunities (W. Murphy, Irish Blood Transfusion Service)

#### SESSION 7: PRODUCTS | 14:00 - 16:00 hrs

- Paediatrics to adults treatment: evidence from ESID Registry (S. Ehl, University Medical Center, Freiburg)
- Paediatrics to adults treatment: experience in Germany (tbc)
- Paediatrics to adults treatment: experience in France (tbc)
- Alzheimers - IVIG (N. Relkin, Weill Cornell Medical College, NY)

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# ILEANA CARLISLE

Vice President,  
Plasma Operations,  
Biotest Pharmaceuticals Inc.

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**AS VICE PRESIDENT FOR PLASMA OPERATIONS,**

Ileana Carlisle takes overall responsibility for plasma supply at Biotest Pharmaceuticals Inc. She is the Chairwoman of the Plasma Protein Therapeutics Association's Source Board and sits on several industry committees.

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**Please tell us about your background, education, your time in the industry, and so on.**

I am originally from La Habana, Cuba. My family left that country in the early 1960's, shortly after Fidel Castro came to power. We lived in Caracas, Venezuela for four years, and then moved to Southern California in 1964. I grew up in the city of Burbank, located in the San Fernando Valley, and started my college career at the University of California, Northridge. I finished my Bachelor's degree in Biology at Florida International University (FIU) after moving to Florida, and received my Master's of Business Administration in International Business from FIU as well.

After living 30 some years in Florida, I finally call it home. I have a 21 year-old daughter, Stephanie, in college, and I married my soul mate, John Carlisle, in 2007.

I first became aware of plasma collection for manufacturing purposes during college when I worked at a commercial blood bank in the Miami area. We not only collected red cells and serum for diagnostic test kits, but also produced Anti-D plasma for the manufacture of Rho(D) IG. These donors really impressed me—we had a lot of committed ladies that gave plasma regularly so that others would not have to go through the same difficulties they had with their pregnancies. Being Rh negative myself, I was fascinated by their stories and their dedication.

In 1983, I joined North American Biologicals Inc. (later known as Nabi Biopharmaceuticals) and became involved in the distribution of plasma to customers around the world. I had an opportunity to interact with representatives of numerous companies worldwide, our plasma centers, and our donors. Imagine my surprise to find some



of the same Anti-D ladies I had met earlier in my career still donating at one of our Florida centers so many years later.

The mid 1980's were an incredible and difficult period—the advent of AIDS and HIV testing completely changed our industry. It was during these challenging times that I realized I had found my life profession, and 27 years later, I have never looked back.

### **What is your perspective on the role of plasma collection in the entire industry?**

We are part of a very unique business. We produce lifesaving therapies, but yet, we are not pharmaceuticals. The source of our raw material, our donors, is very unique—it takes a very special individual to be willing to donate their precious bodily fluid time, after time, after time. Our patients are unique—they suffer from very rare diseases that most people have never heard of. Their lives depend on our very unique therapies.

But it really is the raw material that sets us apart from any other industry. Our raw material is unique—we collect human plasma, not for transfusion, but to be used for further manufacturing into these therapies. This very special raw material brings with it so many issues—issues that we have been dealing with for many years and continue to deal with in our current environment. Our plasma centers' management and employees deal with these issues on a daily basis. These individuals have a very tough job—they not only work in a highly regulated setting, but also deal directly with the public—our donors. It takes a very special and unique individual to be in this business.

### **You've been in the industry for quite some time; what are some of the changes that you've witnessed and how would you describe the future of the plasma collection process?**

I hate to admit it, but I can vividly remember when plasmapheresis was still a manual procedure, when we had no automation in the plasma centers, when donors' records and the plasma unit documentation were completely paper-based, when there was no Nucleic Acid Testing (NAT), no National Donor Deferral Registry and no

IQPP program. It's hard to believe how far we've come in a relatively short time.

I believe the plasma collection process will continue to progress and improve. Among other things, technology will allow the donation process to become faster and more convenient for our donors. Our collection facilities will continue to evolve to be more professional and appealing, and I believe we will continue to find ways to improve the safety of our plasma and the stability of our donor base.

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**"The mid 1980's were an incredible and difficult period—the advent of AIDS and HIV testing completely changed our industry. It was during these challenging times that I realized I had found my life profession, and 27 years later, I have never looked back."**

— Ileana Carlisle

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### **What types of threats does the industry face? Opportunities?**

*"Our biggest threat may well come from within. If we become complacent, accepting of the status quo, and fail to strive for continuous improvement, we will fail our donors and the patients who receive our products."* As I mentioned when I spoke at this year's Source Business Forum, we are only as strong as our weakest link. We have to keep raising that bar. Our products are the safest they ever have been—and we should be very proud of that. But this didn't come easy. It took several years and a lot of effort from the members of this industry to get to the point where we are today.

Our donors give a significant amount of their time to provide the plasma needed for the therapies we manufacture—they deserve the best customer service and convenience we can make available. The patients that use our products demand and deserve the safest therapies at the highest standards possible. A steady and reliable supply of the highest quality plasma is essential to our industry; we cannot stop raising the bar.

### **Please tell us a little about the Source Board of Directors.**

I have been a part of the Source Board for several years. I have had the privilege of working along side some of the "icons" of our industry. Some of these individuals not only started this business, but actually shaped it into what it is today. The members of the Source Board of Directors are an interesting mix of individuals who genuinely care about our industry. We have an excellent cross representation of fractionators and independents, as well as, U.S. and European companies. Jointly, and with the very capable guidance of PPTA staff, the Board gives a significant amount of time to respond to emerging issues and industry challenges, and continues to champion improvements towards donor safety and plasma collection processes that ultimately result in making products safer and more available to patients everywhere.

### **What would you like the readers of *The Source* to know about plasma collection?**

I have been very fortunate in my career—I am in an industry that truly makes a difference in people's quality of life.

To our donors, I would like you to know how important you are to us and the patients who receive the products made from the plasma you donate. Your plasma donations provide life changing products to all of the patients who depend on them.

To those in our industry, I would like you to know how important you and the work you do are to the quality of the plasma we collect. Your efforts are a key component in delivering safe and efficacious products.

To others, I would encourage you, if you have never been to a plasmapheresis center, to visit one. Take the time to speak to some of our donors. As you learn more about our industry, our products, and the impact these therapies make in the lives of the patients that receive them, I believe you will find ours to be a very interesting story. ☁

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JOSH PERNOD is PPTA's  
Vice President, Source

# EXPANSION OF MEDICAID Under New Health Reform Law TO BENEFIT MILLIONS OF AMERICANS

BY BILL SPEIR

**DEFYING THE EXPERTS AND HISTORY,** The 111th Congress passed a health reform package through a series of legislative maneuvers to avoid a Senate filibuster.

The House first passed the Senate's version of the Patient Protection and Affordable Care Act (Public Law 111-148) and then the two chambers passed the Health Care and Education Reconciliation Act (Public Law 111-152). Together the bills are known as the Affordable Care Act (ACA). The expansion of Medicaid is a vital component in the new health delivery framework that promises to provide health benefits to 32 million more Americans by 2014. It is expected that half of these individuals will be enrolled in Medicaid as a result of changes in the ACA.

Medicaid has caused some states to question whether they can afford their share of the expansion. Florida expects the expansion of Medicaid to cost the state an additional \$1 billion by 2019.

For states that believe the costs of Medicaid expansion are too great, they always have the option of ceasing their Medicaid participation. Last year, a bill was filed in Virginia that would have required the state to cancel its participation in Medicaid if the federal government passed health care reform. At this summer's National Conference of State Legislatures, elected officials from Arizona and Wyoming mentioned their states were considering opting out of Medicaid.

In 2008, total Medicaid expenditures climbed to nearly \$339 billion. For health providers, that is \$339 billion in income. If state legislatures seriously consider dropping out of Medicaid, the hospitals, doctors, and other health providers in the state would show up at the capitols by the busload to protest the elimination of Medicaid. So unless the courts rule the ACA is unconstitutional, or the new Republican wave leads to repeal of the ACA, it looks like Medicaid expansion is upon us.

The states face many challenges in expanding Medicaid and implementing health reform. They will need to enhance their enrollment systems to add the new



recipients. They will need to improve their provider networks to provide recipients with access to care. This will be a great struggle for Medicaid Directors because Medicaid programs already have difficulty providing their recipients with access to care.

States are stuck in the worst budget situation since the 1930s, so there won't be much money to upgrade their information technologies and lure new providers to the program. This means they will need to limit costs elsewhere. This could mean trouble for consumers of plasma protein therapies.

Medicaid recipients that rely on plasma protein therapies can expect that their state Medicaid program will

seek to enroll them in managed care. They will also seek to drive costs down through a variety of cost-containment policies. These policies may have a negative impact on individuals that rely on plasma protein therapies. Utilization controls could involve step-therapy that would cause patients to switch therapies. Greater out-of-pocket costs could lead to patients not having enough money to procure their therapies or infusing less frequently than medically appropriate. PPTA will continue to advocate for patient's needs on this issue. 🌐

BILL SPEIR is PPTA's Assistant Director, State Affairs

## PPTA INTERVIEW

# Valerie J. Harr

Acting Director of the Division of Medical Assistance and Health Services (DMAHS), New Jersey

### **What are the greatest challenges facing your Medicaid program with expected increased caseload as a result of Medicaid expansion under the Affordable Care Act?**

There are still a lot of decisions to be made in New Jersey, such as how the insurance exchange will operate and how to develop policy and implement new eligibility rules for the expansion.

### **What are your expectations for the woodwork effect of new enrollees that are currently eligible?**

New Jersey has historically been, and continues to be, very aggressive in outreach to families who are eligible for our population. The newly eligible group will

be adults without dependent children who are not currently eligible for our program.

### **How will your Medicaid program ensure there are enough Medicaid providers for the increased caseload?**

This is a nationwide problem, not unique to New Jersey. Reimbursement to primary care providers will increase under the Affordable Care Act; and a variety of other initiatives to increase provider capacity, such as loan forgiveness programs and recognition of paraprofessionals, are being considered.

### **Among the new enrollees will be adults with chronic care needs, how will your**

### **Medicaid program ensure they have access to their appropriate care?**

New Jersey recently received a federal exchange planning grant. As a result of that funding, we are working with a local university to evaluate the demographics and needs of what will be the newly eligible population. In turn, we will determine the best delivery model. It may be our existing full-risk managed Medicaid program or an alternative delivery model such as medical homes, Accountable Care Organizations (ACO), or Primary Care Case Management (PCCM). 🌐

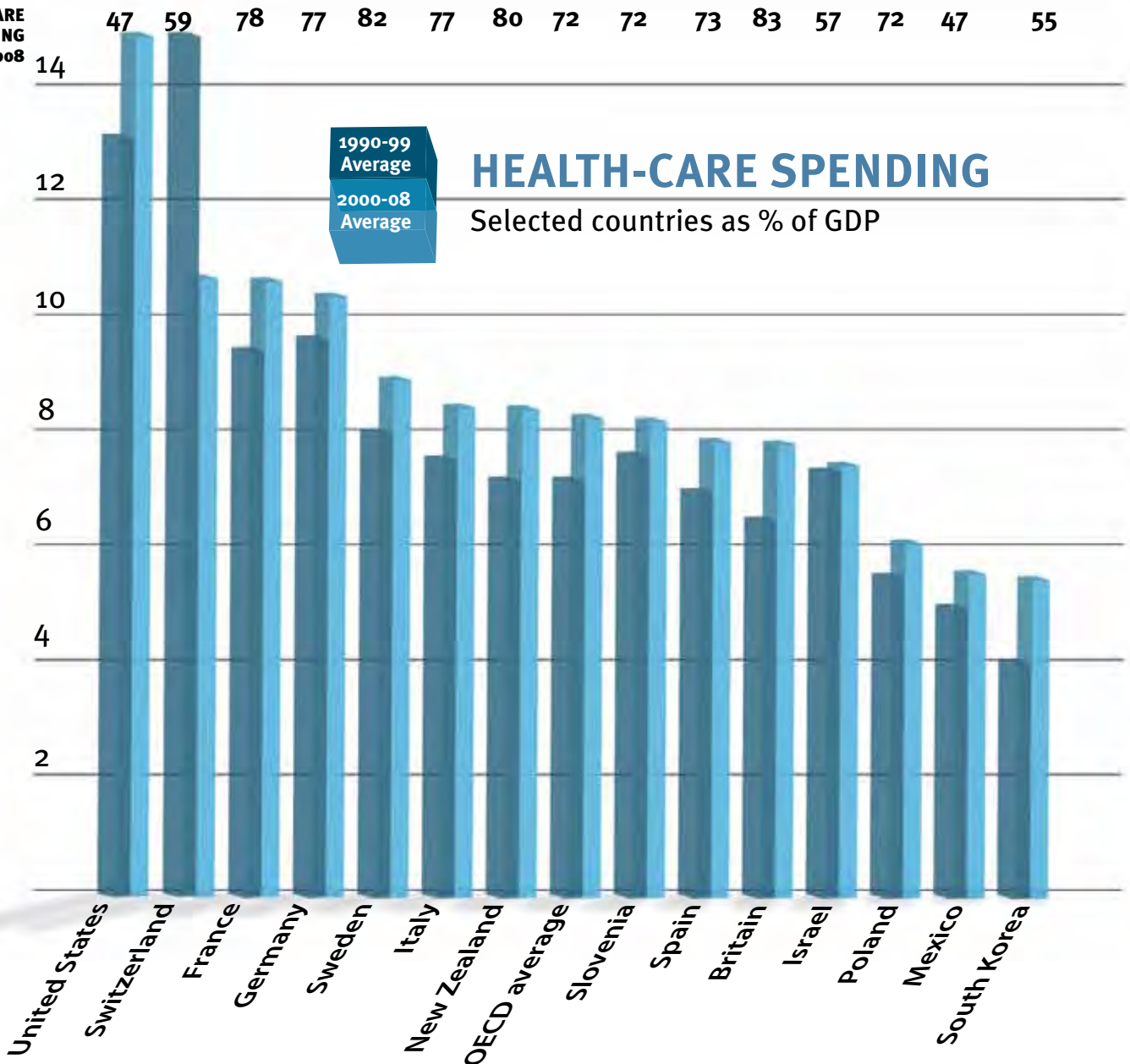


THE SOURCE will periodically feature tactics and strategies governments are adopting with regard to health care spending

# REDUCING HEALTH CARE SPENDING

BY CHARLES WALLER

PUBLIC HEALTH-CARE SPENDING AS % OF TOTAL, 2008





**ACROSS THE WORLD**, governments are having to find new ways to spend less while at the same time dealing with the metronomic increases in inflation. Many of the government strategies are affecting and will affect healthcare in general, pharmaceutical spending in particular and, with it, the plasma protein industry.

This will be the first part of a periodic item in *The Source* where we will provide a roundup of specific tactics and strategies that governments and insurers are adopting that affect or will affect this sector.

Many believe that the impact of the serious financial banking crisis in 2008 and subsequent reverberations are still in their infancy. Governments in Europe and North America, within their own context are looking to save very substantial sums of money and it's going to take a bit more than improving the efficiency of purchasing paper clips.

Inevitably, governments start with their big costs, and healthcare is one of the most expensive items in every country.

(See the Organisation for Economic Co-operation and Development tables, below).

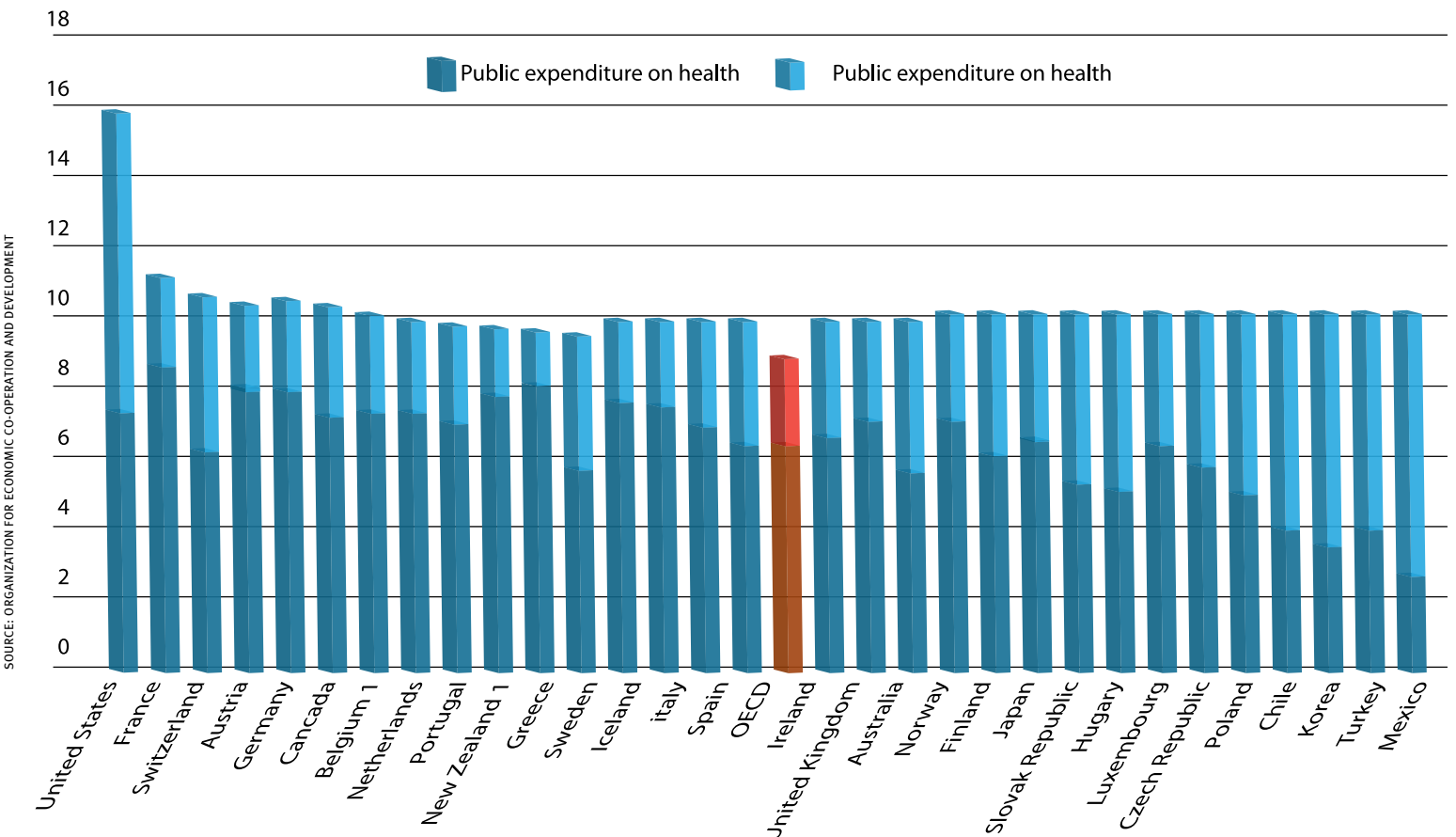
It is also widely recognized that healthcare is late in the economic cycle to show the impact of macroeconomic changes. It takes time to get through the acute crisis phase and to then deal with the consequences. The news from around the world will attempt to capture the latest developments that will affect government policies related to healthcare, including government health spending changes and clinical related policies, e.g. related to dosing. 🌐

CHARLES WALLER is PPTA's Vice President, PPTA Europe

GORDON SAUNDERS / SHUTTERSTOCK

## HEALTH EXPENDITURE AS A SHARE OF GDP, 2008

or latest year available



SOURCE: ORGANIZATION FOR ECONOMIC CO-OPERATION AND DEVELOPMENT

# TRADE ASSOCIATIONS

## THE CONCEPT OF INDUSTRY PARTICIPANTS

coming together to address issues of mutual concern is not a new one, but harkens back to the early days of guilds, unions, and even more informal professional associations. Nevertheless, the very idea creates tension with certain aspects of modern legal systems, most notably in the area of competition law. A trade association, by its very nature, consists of competing companies working together to reach shared objectives. On first impression, this appears to conflict with the goals of antitrust enforcers, who seem bent of preventing cooperation among competitors.

A closer look, however, reveals that this initial impression is misleading. Just as businesses have long recognized the utility of trade associations, so have courts and the legal system. Indeed, both courts of general jurisdiction<sup>1</sup> and specialized antitrust enforcement agencies—in both the United States and Europe<sup>2</sup>—have expressly recognized a broad range of trade association activities as pro-competitive and beneficial.

In addition to generally recognizing the value of industry conferences and policy forums—at which members of a trade can network, discuss best practices, and receive education on the industry state-of-the-art—courts have acknowledged that trade associations are uniquely well-positioned to carry out a variety of functions, including advocacy and standard setting data collection.

## Advocacy/Government Relations

Persuading government officials to take, or not take, a particular action that could impact industry is political conduct, not commercial conduct within the scope of the antitrust laws.<sup>3</sup> Consequently, cooperation in this area is something that both courts and legislators encourage. As a business matter, managing advocacy efforts through a trade association reduces costs by, for example, leveraging the industry's political contacts and collective knowledge of an issue. Government personnel also appreciate a cooperative approach, as it is more respectful of crowded calendars and tends to focus discussions on areas where there is an industry consensus.

## Standard Setting

Courts and regulators have also recognized trade associations' critical role in standard setting. Regulators are often tasked with mandates and oversight responsibilities that far exceed their limited resources. To fill this gap, they not only encourage, but often rely on, voluntary industry self-regulatory efforts. Industry also finds such efforts valuable, as they ensure that those with the greatest knowledge of the trade—the industry participants themselves—will be involved in standards development at the ground floor. This is the model pursued by both PPTA's International Quality Plasma Program (IQPP) and Quality Standards of Excellence, Assurance and Leadership (QSEAL) programs, which involve member company subject matter experts in the development of standards that supplement and exceed the quality and safety requirements imposed by the U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA), and other state- and national-level regulators.

## Data Collection and Reporting

Data collection and reporting is another trade association function with a long history of court and regulator approval. Benchmarking efforts, which permit industry participants to evaluate and improve their performance via comparison to similar enterprises, are perhaps the best and most familiar example of a pro-competitive use of a data program. Data reporting efforts can also provide an invaluable tool to regulators, particularly in industries like biotech and pharmaceuticals, where they provide a useful statistical backdrop against which to evaluate known or emerging safety concerns.

Of course, all of these functions must be carried out in an antitrust-compliant manner, and with appropriate legal safeguards in place. Managing legal risk has always been, and continues to be, a top priority for PPTA. Far from being an obstacle to the Association's objectives, it is just one additional means of ensuring that these objectives are approached in a focused and goal-oriented manner. ☞

JOHN DELACOURT is PPTA's Senior Director, Legal Affairs

<sup>1</sup> *Maple Flooring Mfrs. Ass'n v. United States*, 268 U.S. 563, 582-83 (1925).

<sup>2</sup> *U.S. Dep't of Justice & Fed. Trade Comm'n*, Antitrust Guidelines for Collaborations Among Competitors § 2.1 (2000).

<sup>3</sup> *Eastern R.R. Presidents Conference v. Noerr Motor Freight, Inc.*, 365 U.S. 127 (1961).



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As part of a series, **THE SOURCE** will feature a different specialty immune globulin product in the next several issues of the magazine.

# RHO(D) IMMUNE

## Saves Thousands of Lives

### BEFORE THE INTRODUCTION OF Rho(D)

Immune Globulin (IG) in the late 1960s, the fear of having a “blue baby,” so-named because of the bluish color of infants at birth, among young women planning a family was palpable. Hemolytic disease of the newborn (HDN), as the condition is medically known today, resulted in the loss of thousands of pregnancies worldwide every year to miscarriage, and babies were still born or died often from congestive heart failure as a result of the disease. Besides infant mortality, newborns that did survive often were faced with mental retardation, hearing loss or cystic fibrosis. Before the introduction of Rho(D) IG to treat HDN, 10,000 babies died in the U.S. alone each year, and thousands more worldwide, making it the leading cause of infant mortality.

### What is HDN?

HDN occurs because of a blood incompatibility between the mother and fetus. A body's immune system recognizes substances that are native to the body and the differences between those and what it interprets as foreign substances. When a woman with a Rhesus (Rh)-negative blood type is having a child that is Rh-positive, meaning that the fetus has Rh-positive protein on the membrane of the red blood cells, the mother's immune system identifies that positive protein as foreign and mobilizes against it, creating antibodies to attack the perceived invader. While the condition less frequently affects a first pregnancy, subsequent pregnancies are at serious risk. Once the immune response is triggered, it can never be reversed and causes a life-threatening condition for the fetus in utero because the mother's immune system destroys the fetus' red blood cells.

Rho(D) undergo significant testing and education before being accepted into the immunization program.

### What is the worldwide use of Rho(D) IG immune globulin?

The Marketing Research Bureau global report for 2008 places the market at more than 4 million units sold by 20 global producers, including several nonprofit companies. More than 1 million of those units were sold in the U.S.

### How many women are Rh-negative in the U.S.?

According to the American College of Obstetricians and Gynecologists (ACOG), about 15 percent of the white population and 5-8 percent of the African American and Hispanic populations are Rh-negative. Roughly 22 million women—more than the population of New York state.

### How big was the problem?

Prior to the identification of the Rhesus blood groups in 1940 and, subsequently, the link to Rhesus incompatibility; the recognition in the mid-1950s of HDN; and the introduction in the late 1960s of Rho(D) IG, the prevalence of infant death from blood incompatibility between the mother and child was as high as 46 in every 100,000 births. Today, with proper treatment, it is extremely rare in most developed countries.





# GLOBULIN

## Every Year

### Today's Treatment

To prevent HDN, Rho(D) IG, which first came to market in the U.S. in 1968, is administered to a mother typically once during a pregnancy and again after delivery. It works because the therapy, which contains the anti-Rh antibodies, seeks out the baby's Rh-positive red blood cells in the mother's circulatory system and essentially coats them, preventing the mothers' immune system from recognizing and destroying them. Eventually the anti-Rh antibodies leave the mother's circulatory system, making each pregnancy and birth the first, from an immunological standpoint. Prior to the development of Rho(D) IG a series of exchange transfusions were performed on newborns, a dangerous procedure that essentially replaces the baby's blood.

---

**"This [HDN] was such a feared disease among young women. I think the magnitude of its [Rho(D) immune globulin] success and its importance on American health care in general, and in all vaccine history, has really been underappreciated by the public."**

**– Dr. James Crispen**

*Hematologist and early pioneer, working with Ortho-Clinical Diagnostics, with developing anti-D donors and testing and developing what became RhoGAM, the first Rho(D) IG product.*

---

### Donors Are Pivotal

Many individuals who have worked on this product for years say that one of the most unique and rewarding aspects of developing Rho(D) IG immune globulin, is the strong connection between the plasma donor and the patient. While men exclusively comprised the cohort of early donors and recipients who were essential to the development and testing of the product, most of the initial, committed donors who participated in the special plasma donation program were women who had suffered from Rh incompatibility, had developed the antibody and, in many cases, had lost children to HDN. "They very clearly understood why they were donating," said Tom Wacker, Vice President of Operations for Talecris Plasma Resources. "They were donating to prevent the very experience





The American College of Gynecology and Obstetrics (ACOG) names the development of Rho(D) IG among the top accomplishments in the field in the past 50 years. Pictured here is a mother from Teaneck, New Jersey, who was treated with the first Rho(D) IG product, and her healthy baby born in May 1968.

they had gone through.” Many women donated twice a week for 10, 15, even upwards of 30 years, according to Wacker.

PPTA recognized a long-time Rho(D) IG donor at its Plasma Protein Forum this year. Raymonde Marius, of Winnipeg, Canada, has donated her plasma for 42 years, and having lost a baby to the disease before the therapy was available, she knows what parents go through. “It has been a privilege to be able to do it and be part of the miracle for babies to live,” she says.

The plasma donated to produce Rho(D) IG is fractionated in a process similar to the manufacture of other immune globulin therapies, although at a much smaller volume, and undergoes the same safety measures as all of the plasma-derived therapies.

### **Success Brings Changes with Recruiting Donors**

Bill Bees, Senior Vice President, Operations, Cangene Corp., explains that in the past it was easier to recruit donors because the women had an antibody resulting from pregnancy and a personal connection to what their donation was preventing. But as the Rho(D) IG successfully prevented HDN, the number of women who experienced it first hand, fortunately, dwindled. Consequently, recruitment into the special plasma donor program has become more difficult, given that many of the committed women donors are now well into their 70s. Bees explained that the industry as a whole is losing a donor base that painfully understands its importance. Today, it requires more education and time in order to recruit donors, because most are required to undergo an immunization process in order to develop the antibody needed for the product.

### **Immunization Program Develops**

As the success of Rho(D) IG spread and helped thousands of women every year, the ability for manufacturers to obtain plasma from women who had pre-existing antibodies diminished, making it necessary to cultivate new donors, men and women, through an immunization program. The requirements for donation are stringent, and the program is heavily regulated by the U.S. Food and Drug Administration (FDA). In addition to being healthy, fully understanding the Rho(D) IG donation program and making the commitment to donate, usually twice a week, women entering the program need to prove that they are physically unable to have children, because the immunization stimulates the production of the antibodies against Rh-positive red blood cells, placing any future pregnancy at risk. Donors submit to a series of tests, and a whole blood profile is performed in order to match with a red cell donor. Once a donor enters the program and is immunized, he or she cannot return to traditional source plasma donation.

After immunization, the donor is tested frequently to evaluate the amount of the antibody their body is producing. It could take as long as six months and multiple immunizations in order to develop

**“The development of Rho(D) IG is a compelling story that clearly demonstrates why plasma donation is irreplaceable.”**

**—Tom Wacker**  
Vice President of Operations for  
Talecris Plasma Resources

an antibody level sufficient to begin donating plasma that contributes to the final product. Donors are then tested on a schedule, roughly every four weeks, and often require additional immunizations in order to maintain the sufficient titer level.

### **Red Blood Cell Donation**

One reason the Rho(D) IG plasma donation program is so highly regulated is that donors are essentially receiving a “mini transfusion of incompatible blood every time they are immunized,” said Ileana Carlisle, Vice President, Plasma Operations with Biotest Pharmaceuticals. Red blood cells are the immunizing agent for the Rho(D) IG negative plasma donor and are another essential component of the therapy's development.

Once a new red blood cell donor is identified, meaning that his or her red cells are typed and matched as closely compatible as possible to the Rho(D) IG donor who will receive them through the immunization process, he or she is tested for all known pathogens similar but not exactly the same as whole blood donors. Once all tests confirm negative for known viruses, such as HIV and hepatitis B and C, the red blood cells are frozen and stored for 12 months, at which point the donor is tested again. Once negative tests for viruses are reconfirmed, the

## **Barriers in Europe**

According to Michaela Rethwilm, an industry consultant who spoke on this subject at the International Plasma Protein Congress (IPPC) this year, Germany is the only country, other than the U.S., with specific guidelines for immunization of plasma donors. Rethwilm believes the guideline works for all other hyperimmune programs except anti-D. Germany has been very successful with hepatitis B virus (HBV) and tetanus programs, however, the requirement for red cell donors and plasma donors to produce Rho(D) IG is extremely high—a barrier she says, but not the main barrier. One company was able to develop a program to meet the strict guideline, however Germany's Transfusion Act permits a donor immunization program if it is limited to serve the German population. The plasma collector that was able to meet the guideline and successfully recruit red cell and plasma donors for the program was not able to get a contract with a fractionator and, therefore, the program never got off the ground. Rethwilm points out that the program in place in the U.S. works, the plasma collectors and fractionators have an FDA licensed product, basically sold worldwide. However, Rho(D) IG plasma must be seen as a global product and the goal is to have more than one supply-region worldwide. Currently, the product is made only from U.S. sourced plasma.

“There is a need to balance risk for the plasma donor, who has no benefit from immunization, against the risk facing pregnant women in the population who are really in need of this product,” Rethwilm said.



red blood cells that have been stored for 12 months can only be used as the immunizing agent for three plasma donors. The donor recipients are tested every three months for the same viral markers to ensure they are nonreactive. After 12 months of testing, if the Rho(D) IG donor is found free from viruses, the red blood cell donor becomes certified or "pedigreed" and their red blood cells are qualified for routine use in the immunization program. However, red blood cells can never be used until they have been stored for 12 months and the donor has been refused. All centers are licensed by regulatory authorities and the program is closely monitored. The medical director is present each time a donor is immunized, and the process of ensuring safe red cell donation is no less rigorous to ensure the safety of the donor and the end recipient of the product.



**Men and women can become Rho(D) IG plasma donors, which requires a level of commitment above that of a typical source plasma donor and is vital to the production of Rho(D) IG. Pictured here are Merry (left) and Tony Aaron (right) who have both voluntarily committed to donate Rho(D) IG plasma.**

before. Once the negative tests are reconfirmed, the donor's cells can be used in limited immunizations (maximum of three recipients who are tested every three months for one year). At the end of this two year period, if the donor and the donor's recipients remain virus-free, the donor becomes certified or "pedigreed" and their red blood cells can be used for routine use in the immunization program. However, any subsequent red blood cell donation must be frozen and go through the 12-month storage and donor retesting. All centers are licensed by regulatory authorities and the program is closely monitored. The medical director is present each time a donor is immunized, and the process of ensuring safe red blood cell donation is no less rigorous to ensure the safety of the donor and the end recipient of the product.

Not every Rho(D) IG -positive person can serve as a donor of red blood cells for immunization. The potential donor is phenotyped, ie., has his or her red blood cells tested for a variety of red blood cell antigens, to make sure that the donor's blood is as compatible as possible, except for the Rho(D) IG factor, with the Rh-negative plasma donor to make sure that anti-Rho(D) IG is the only antibody that forms. Additionally, the red blood cell donor is tested for as many viruses as possible, similar to testing performed for blood and plasma donors. If virus testing is negative, the red blood cells are stored frozen for 12 months. At the end of the 12 months, the donor is tested again for viruses to make sure that no infectious agent was incubating when the red blood cells were drawn a year

On the market now for 42 years, Rho(D) IG, which today only can be manufactured from plasma, has nearly eliminated HDN in developed countries, making it a remarkable product that continues to save lives and prevent severe birth defects across the world every year. ☞

**SPECIAL THANKS** to *Ileana Carlisle, Bill Bees, Tom Wacker, Joe Rosen, Dr. James Crispen, Ortho-Clinical Diagnostics and Michaela Rethwilm.*

*KYM H. KILBOURNE is PPTA's Assistant Director, North America Communications*



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# DONOR COMPENSATION

## When did the Controversy Start?

**THE AMERICAN BLOOD COMMISSION** established in 1973 developed a National Blood Policy, which advocated, amongst other measures, the phasing out of compensated blood donation. The pressure for this measure had been mounting since the demonstration that such donors unquestionably transmitted high rates of hepatitis.

Reportedly, the Commission was strongly influenced by a book by Richard Titmuss. This book, *The Gift Relationship* [Figure 1], has become the bedrock of the case against paid donation. Allegedly, the then-Nixon administration consulted Titmuss on the need for reform in the U.S. blood system.

In most countries, a tacit acceptance of the need to compensate donors in various ways appears to be established, while continued adherence to the “voluntary, non-remunerated principle” is maintained. As the origin of this principle is strongly linked to Titmuss’ work, a discussion of this important milestone in this debate is warranted.

*The Gift Relationship* is essentially a manifesto for the classical welfare state vision of public goods and services and exemplifies these concepts primarily through a comparison of the U.S. and United Kingdom (UK) blood systems in the late 1960s. Titmuss produced his work through the course of a debate with the Institute of Economic Affairs, a UK-based free-market think tank critical of the welfare state and the British National Health Service (NHS) of which Titmuss was a passionate advocate. Titmuss used blood to propose that certain public goods, including health care, should not be part of the market for goods and services. Titmuss dreaded the introduction of market forces in the delivery of government services such as health, and he saw the “commodification” of blood as being the thin edge of the wedge of this development.

Reading *The Gift Relationship* today, Titmuss’ arguments can be summarized as:

1. Commercial supply of paid blood discourages altruistic, voluntary donation, hence leading to supply shortfalls and increasing costs.
2. Paid blood is inherently unsafe as the financial motive makes people in high-risk groups for certain diseases lie about their status in order to get money.
3. Paying donors invariably draws the most economically challenged part of a community into an exploitative relationship.

Despite its undoubted influence and its embrasure by many in the blood banking community, *The Gift Relationship* is not a blood banking book and this is evident in many aspects of the book’s description of the U.S. system as disastrous, while the UK system is lauded. *The Gift Relationship* paints a picture of the U.S. blood system in 1970, which is closer to caricature than reality. Using a fairly draconian definition of voluntary donation, which even excluded most of the donors of the American Red Cross, Titmuss claimed that less than 10 percent of America’s donor population in 1970, was voluntary. In contrast, the National Academy of Sciences estimated that compensated donors at that time constituted 15 percent of the total. Similarly, Titmuss’ claim that blood expiry and waste were much worse in the U.S. system, scantily backed up by data, conflicts with the most concurrent survey of U.S. blood

banking. This showed comparable expiry rates of 12percent in both systems.

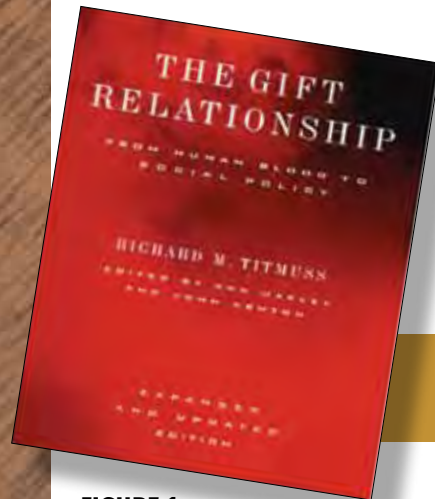
Just as he caricatured the U.S. system, Titmuss idealized the UK system, which was characterized, 17 years after the publication of *The Gift Relationship*, as being severely deficient and, in fact, “a fragmented and disorganized shambles.”

Titmuss’ idealization of voluntary blood donors leads him to propose that the practice provides some kind of societal cement contributing to community solidarity; he continues to contrast the U.S. and UK social environments on this basis. It is difficult to see any evidence for this role for blood donors, who in the best of instances are a very small proportion of the community.

Titmuss extracted much of his conclusions regarding the voluntary donor through a survey he conducted on British blood donors in 1967. Analysis of the raw data in the form of the individual response questionnaires indicates methodological problems including difficulties in understanding and differentiating between questions, lack of information on how the questionnaire analysis was done and, how it led Titmuss to deriving the donor attitudes he proposed in his book.

It is difficult to accept that this influential book has any relevance in today’s era of data driven, evidence-based policy making, or any bearing on the ongoing debate it fuelled. Today we see many blood systems, such as the U.S., where compensated and uncompensated both contribute effectively to maintain-





**FIGURE 1**  
Richard Titmuss  
and his seminal book

ing the supply of essential products. We see many systems where compensated donors are as safe, if not safer, than uncompensated donors. And we see many instances of donors from middle class backgrounds being compensated in ways other than cash. The principal legal measure used to put pressure on paid donation was the introduction by the U.S. Food and Drug Administration (FDA) of a requirement to label blood as paid if it is from monetarily compensated donors. The FDA's regulation also defined "certain benefits that do not constitute

*monetary payment. Those benefits include time off from work, membership in blood assurance programs, and cancellation of non-replacement fees, as long as the benefits are not readily convertible to cash."* But the use of such benefits, readily convertible to cash

[Figure 2] is apparently disregarded. It is time to stop this constant argument, which continues

to threaten supply of essential therapies, and to respectfully consign *The Gift Relationship* to the archives of history. ☺

PROF. ALBERT FARRUGIA is PPTA's Senior Director, Global Access

**mydesert.com**  
October 16, 2009  
**Blood donor wins a new Prius**  
LifeStream sees donations rise

Blood donor Melina Nava (photo) received a white 2010 Toyota Prius at the I-10 Toyota in Indio last week.

The prize was part of the Drive Green with LifeStream promotion, in which blood donors were given a chance to win a car.

LifeStream established a community partnership with I-10 Toyota to promote blood donations and green technology, said Rick Axelrod, president and chief executive officer of LifeStream, based in San Bernardino.

During the last three months, 17,000 people donated, including 5,000 new blood donors, Axelrod said.

"This was a win-win for both of our organizations," he said.

LifeStream has another promotion under way with community partner Southwest Airlines, in which blood donors can win two tickets to travel anywhere in the United States that the airline flies, Axelrod said.

**FIGURE 2** A recent example of an incentive awarded to a blood donor in the U.S., which would have had to have her donation labeled as "paid." Raffles are allowed under FDA guidance in the voluntary-preamble to Trial Rule, 43 FR 2142, (1978).

# FIND ID

## WORKS TO CREATE AWARENESS OF PID IN GERMANY

BY SYBILLE BECK

### NATIONAL ECONOMIC CAPACITY AND THE TREATMENT OF HEMOPHILIA A

are closely correlated, according to a recent study by Stonebraker et al. Whether you look at international units of coagulation factors per capita, or units per patient with hemophilia, Germany is one of the world leaders. So why is it that when it comes to primary immunodeficiencies (PIDs), patients and physicians agree that Germany is far from the top?



**Vicky and Fred Modell  
of the Jeffrey Modell  
Foundation**

Germany has good clinical guidelines for the treatment of PID, a sophisticated health system, the economic means and a good track record in the treatment of other rare diseases. Why then are diagnosis and treatment rates for PID lagging behind other developed countries?

In order to correct this shortcoming, Vicky and Fred Modell of the Jeffrey Modell Foundation together with PPTA, invited the leading PID experts in Germany, the German patient organization DSAI and industry representatives to a meeting in conjunction with the European Society for Immunodeficiencies (ESID) Congress in Den Bosch, The Netherlands in 2008. It became obvious that the underlying

reasons for Germany's low diagnosis rate are multifaceted, but it was time to bring some ideas from the drawing board to reality. Under the direction of Professor Tim Niehues, Helios-Kliniken Krefeld; Professor Volker Wahn, Charité Berlin; and Gabriele Gründl, Chairperson DSAI, the concept of a physician-driven network initiative was developed and FIND-ID was conceived.

The key objectives of FIND-ID are to create awareness for PID, including the warning signs and simple diagnostic tests, among general practitioners (GPs), specialist physicians, ENT-experts, pulmonologists etc., and physicians in regional hospitals, and encourage them to refer their patients to established and specialized PID centers.

\* **For more information on full references**, please contact Sybille Beck at [sybille@pptaglobal.eu](mailto:sybille@pptaglobal.eu)

\*\* **Members of FIND-ID Steering Committee:** Karsten Franke, Gabriele Gründl, Tim Niehues, Reinhold Schmidt, Volker Wahn, Klaus Warnatz, Fred Zepp



# Find-ID - a physician-driven, multi-disciplinary and multi-professional initiative to increase the diagnosis-rate of Primary Immunodeficiencies (PID) in Germany

FIND-ID Group\*



**Summary: FIND-ID**  
**Objective:** increase low diagnosis rate of PID in Germany.  
**Created by** different stakeholders.  
**Activities:** awareness and training activities, generating and providing information and support for caregivers, PID centres and patients.

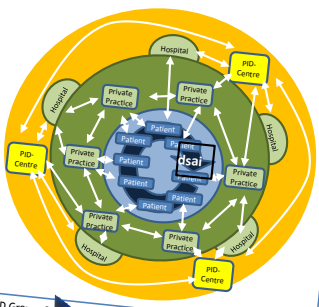
**Methods**  
 Development of the idea of FIND-ID at the ESID congress 2008 by physicians, scientific societies (API, DGfI, DGKJ), patient organisation (dsai), industry (PPTA), PR agency at a JMF initiated meeting\*\*.

**Stepwise approach:**

- analysis of barriers to patient identification and treatment
- questionnaire for stakeholder survey:
  - current situation regarding the care for PID patients (availability of resources, financing)
  - experience with and need for training and information material
  - objectives for FIND-ID
- structured interviews (personal meetings)
- development of a project plan based on the information collected
- metric for effectivity of activities: patients registered in the ESID registry at the model-centre in Frankfurt/Main

\*API (Pediatric Immunology, Arbeitsgemeinschaft Pädiatrische Immunologie), DGfI (Immunologie, Deutsche Gesellschaft für Immunologie), DGKJ (Pädiatrische, Deutsche Gesellschaft für Kinder- und Jugendmedizin e.V.), PPTA (Plasma Protein Association), JMF (Jeffrey Modell Foundation), PR agency (Ball-Com)

**Fig. 1: Network structure of FIND-ID with caregivers and patients: PID centres: sophisticated diagnosis, therapy, support for local hospitals/medical practices, close interaction between all members of the network.**



FIND-ID Group: Steering Committee: \*Wahn, V.1\*, Gründl, G.2,4, Niehues, T.3  
 #Initiator of FIND-ID: Wahn, V.1, Gründl, G.2,4, Niehues, T.3  
 \*Initiator of FIND-ID: Wahn, V.1, Gründl, G.2,4, Niehues, T.3  
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 \*Initiator of FIND-ID: Wahn, V.1, Gründl, G.2,4, Niehues, T.3  
 #Initiator of FIND-ID: Wahn, V.1, Gründl, G.2,4, Niehues, T.3

**Results**  
**Structure of FIND-ID:** steering committee (voting members: PID experts and dsai, non-voting members: JMF, PPTA), advisory board and a medical coordinator;  
**Structure of the whole network:** PID centres, hospitals, medical practices, patients, the patient organisation (Fig. 1), and scientific societies.

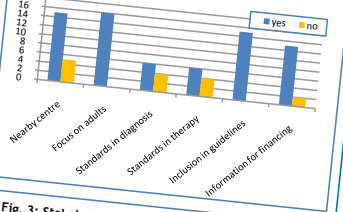
**Values of FIND-ID:**

- focus on the patients' need,
- supporting high quality of care,
- fair/open cooperation of all stakeholders.

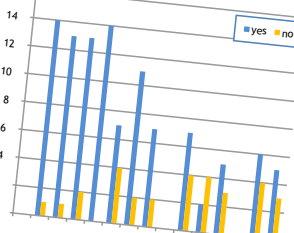
**Stakeholder survey:**  
 N=22, main issues (Fig. 2 and 3):

- weakness: infrastructure (adult patients)
- weakness in co-operation between local caregivers and PID centres;
- need to include PID as differential diagnosis in various medical guidelines
- need for information material for MDs (material for patients available via dsai),
- cost-covering financing of PID centres.

**Fig. 2: Stakeholder survey: Important issues for PID as possible objectives of FIND-ID**



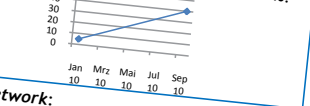
**Fig. 3: Stakeholder survey: Need for awareness activities as focus for FIND-ID**



**Results (continued)**  
**Awareness and education:**

- two workshops for physicians in Frankfurt, Feb. and Sept. 2010
- web-site ([www.find-id.net](http://www.find-id.net)) containing information for MDs, patients, the media, with medical/scientific information, press releases, interviews, download material
- information material for different medical specialities (pneumologist, ENT specialists, gastroenterologists) and teachers (in cooperation with the dsai).
- Patients registered at Frankfurt: Fig. 4.

**Fig. 4: Patients at the Frankfurt centre in the ESID registry as of September 10th, 2010: N=40.**



**Network:**

- presentation of FIND-ID at scientific and patient meetings.
- regular FIND-ID newsletter
- application material: "partner of FIND-ID" for MDs in hospitals and private practices, with quality criteria and certificate
- development of different models for the network, especially for adult patient care
- establishing of contacts to various medical societies

**Service for PID centres:**

- listing of specific laboratory assays (N=470) by PID centre to facilitate search for adequate laboratory
- analysis of different existing models for PID centres

**Outlook - Activities to be implemented:**

- symposia at congress (ESID);
- extension of the web-site (FIND-ID);
- preparation of additional information and training material
- interdisciplinary cooperation

**Discussion**  
 Situation

The classical ordeal of a non-diagnosed PID-patient is an endless journey from GP to GP, expert to expert and hospital to hospital with a history of false and inadequate treatment, before they might be fortunate to receive a proper diagnosis and treatment. So the starting point for FIND-ID is to educate physicians on all these levels to think of PID as a differential diagnosis. Therefore, FIND-ID has developed a website ([www.find-id.net](http://www.find-id.net)), brochures for expert physicians containing algorithms (download on the website—in German only), flyers, a poster developed at the recent congress of the ESID [Fig 1] and Continuing Medical Education (CME) awarded workshops.

#### Adult Patients

In a survey conducted by the Medical Coordinator of FIND-ID among key opinion leaders in Germany, it became evident

that in addition to the general awareness there is a serious deep rooted issue when it comes to the treatment of adult patients. It seems to be a common phenomenon that health systems provide better care for children than for adults. It is, however, particularly surprising that in various countries throughout Europe—irrespective of the prevalence—there is a significant downfall in care when it comes to the treatment of adult PID patients. In Germany, the most evident reasons can be found in the reimbursement system, which only occasionally allows for adult patients to stay in their pediatric department, and in the lack of specialists for PID in the internal medicine community. Clinical immunology is not a field in which physicians can specialize in Germany, therefore, the experts come either from the few hospitals with an excellent PID background or they have to take on a whole new field in addition to their existing field.



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**The key objectives of FIND-ID are to create awareness for PID, including the warning signs and simple diagnostic tests, among general practitioners... and physicians in regional hospitals and encourage them to refer their patients to established and specialized PID centers.**

**Awareness and Structures**

So, in addition to much needed awareness, it is equally paramount for FIND-ID to support the centers to establish

structures that allow the centers to provide adequate diagnosis and treatment for children and adults. The support can range from ensuring that all sources receiving reimbursement for the treatment (compared to the drugs) are exploited over support for the said adult treaters to the definition of quality criteria and patient pathways and, ultimately, new centers. Only this two-tiered approach will bring the success that everyone involved in the project is working for: early diagnosis and adequate treatment for all PID patients in Germany!

Thanks to the engagement of the Steering Committee and the contribution of the industry, FIND-ID will continue to prosper and make a real difference.

*SYBILLE BECK is PPTA's Senior Manager, Source Europe*

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## ▶ ASSOCIATION HOSTS THIRD IN ITS SERIES OF HEALTH REFORM WEBINARS

PPTA's state affairs staff and members of the State Affairs Steering Committee teamed this year to develop a series of webinars on the new federal health reform law, how it may be implemented and how it may affect individuals with rare, chronic diseases that require plasma protein therapies. The third webinar was held on October 23 and covered Medicaid expansion and the individual mandate and how it is being viewed by the courts. All three webinars were a collaboration with members who delivered the content and represented an outreach partnership with national consumer organizations that promoted the webinars to their membership. Strong attendance and



## HEALTH REFORM WEBINAR

Free webinar on how reform may affect people with rare diseases using plasma protein therapies  
Presented by PPTA and its members

feedback from a follow-up survey indicated this information and the webinar format were an excellent way to reach consumers with important details about how reform may affect access to their therapies.

## ▼ PPTA LAUNCHES NEW WEBSITES IN GERMANY AND AUSTRIA TO RAISE AWARENESS ABOUT PLASMA DONATION



PPTA has launched a new educational website in Germany and Austria featuring information on plasma donation and how it helps patients with chronic, life-threatening diseases.

PPTA designed this informative site as part of its Source Industry Image and Credibility Campaign. Die-PlasmaSpende is the only industry-wide website available in the German

language that seeks to explain and clarify the plasma donation process. The new website offers information regarding

the diseases and disorders treated with plasma protein therapies, differences in blood and plasma collection, real-life patient and donor stories, and links to patient group organizations and other online resources. To view the websites please go to the following links:

[www.dieplasmaspende.de](http://www.dieplasmaspende.de) and [www.dieplasmaspende.at](http://www.dieplasmaspende.at).

## ▼ FDA COMMISSIONER DISCUSSES REGULATORY SCIENCE

Margaret Hamburg, M.D., Commissioner of Food and Drugs, October 6, 2010, appeared as luncheon speaker at the National Press Club in Washington, D.C., discussing regulatory science, "an issue that more and more people are recognizing as critical to progress for patients." The Commissioner began her remarks by releasing a U.S. Food and Drug Administration (FDA) White Paper, *Advancing Regulatory Science for Public Health—A Framework for FDA's Regulatory Science Initiative*. FDA defines regulatory science as the science of developing new tools, standards and approaches to assess the safety, efficacy, quality and performance of FDA-regulated products. "This document provides a broad blueprint for progress in the field, as well as our agency's role in promoting this progress," the Commissioner said.



## GLOSSARY OF TERMS

<b>ACA</b>	Affordable Care Act
<b>AIDS</b>	Acquired Immune Deficiency Syndrome
<b>EMA</b>	European Medicines Agency
<b>FDA</b>	U.S. Food and Drug Administration
<b>HIV</b>	Human Immunodeficiency Virus
<b>HTA</b>	Health Technology Assessment
<b>IPPC</b>	International Plasma Protein Congress
<b>IQPP</b>	International Quality Plasma Program
<b>MHLW</b>	Ministry of Health, Labour and Welfare

<b>NAT</b>	Nucleic Acid Testing
<b>NHS</b>	National Health Service
<b>OECD</b>	Organisation for Economic Co-operation and Development
<b>PhRMA</b>	Pharmaceutical Research and Manufacturers of America
<b>PID</b>	Primary Immunodeficiency Disease
<b>QSEAL</b>	Quality Standards of Excellence, Assurance and Leadership



## ► 15 YEARS OF LEADERSHIP

In this edition of *The Source*, we celebrate Jan M. Bult's 15 years of leadership at PPTA. We praise Jan not only for his tenure but for his outstanding achievements in strengthening the plasma protein therapeutics industry and his role as a leader in advocacy. Jan's tireless commitment to making sure that patients worldwide have access to plasma protein therapies is unmatched and he continues to motivate others with his personal commitment. Jan's focus on patient access to life saving plasma protein therapies is inspirational.

As President and CEO of PPTA, Jan's responsibilities today include oversight of all association operations, outreach to stakeholders, acting as industry spokesperson, and the global coordination of all PPTA activities. Jan has held his current position since January 1998. Before that he was the Executive Director of PPTA's European Division (formerly the European Association of the Plasma Products Industry, EAPPI). Prior to joining the Association in 1995, he worked in the pharmaceutical industry



since 1974 for CIBA-GEIGY, Schering AG, Rhone-Poulenc, Mediselect and Biotest. The positions he held were in sales, marketing, R&D, training and business management.

Although Jan travels frequently around the world and astonishes his staff on a regular basis with his stamina and unrelenting vigor while working hard on behalf of others, he still finds time to relax by scuba diving, cycling throughout the world, collecting model trains and taking on home improvement projects. Anyone who comes in contact with Jan, who is an FAA-licensed pilot, leaves with more knowledge about airplanes and flying in general. In some cases,

we learn more than we bargained for with regard to airline safety, but we appreciate and view things very differently as a result when we are passengers on an airplane. Jan and his wife Rose are the loving parents of 5 children and 5 grandchildren. When they are in the U.S., they reside in Millersville, MD and when they are in The Netherlands, they stay in Etten Leur, a small city where Vincent Van Gogh grew up as a child.

Please join us in congratulating Jan on the occasion of his 15 year anniversary. His 15 years of experience at the Association has proved essential to the great level of achievements made at PPTA since he joined the team.

## ▼ MEMBERS PARTICIPATE IN SEPTEMBER'S PLASMA PROTEIN THERAPIES MONTH ACTIVITIES IN MINNESOTA

Two PPTA members hosted activities at plasma collection centers in Minnesota as part of the Plasma Protein Therapies Month recognition from the Governor's office, which was intended to raise awareness for the importance of plasma donation and for the therapies that treat rare, chronic conditions.

CSL Plasma held an event at its Duluth center that recognized donors and patients, and the contribution the center makes to the community and to lifesaving therapies. Invited guests included local, state and federal elected officials, and the event was covered by the local ABC-TV affiliate.

Talecris Plasma Resources hosted a grand opening ceremony

at their West St. Paul center that included a ribbon-cutting, facility tour and the opportunity to meet with patients and donors.

These events represent positive awareness for the industry, and Plasma Protein Therapies Month is a joint initiative of the State Affairs Steering Committee and the Source Industry Image and Credibility Campaign.

**Mary and John Gerszewski (pictured center below, Mary in orange shirt and brown vest and John in a black and white checked polo shirt) are long time donors who met at the CSL Plasma center in Duluth and married a couple of years ago. Pictured here with the center staff, the couple shared their perspectives on the importance of donating.**



# STAFF

## JOSH PENROD

**MY NAME IS JOSH PENROD.** I joined PPTA in June of 2003 as Manager, Regulatory Policy. In 2005, I was promoted to Director, PPTA Source and then, a year later, to Vice President. I work primarily with my colleagues on PPTA Staff and the Source Board of Directors to develop industry positions and policies for plasma collectors in the U.S. and Europe. There are a huge variety of issues that engage us, including government policymaking, standards development and administration, remuneration and ethics, industry image and credibility, responses to the industry's needs in the regulatory sphere, and so on. I also assist with our industry's efforts in the international trade arena and am very involved in the industry's standards program.

**Tell us about your background.**

I'm originally from Johnstown, Pennsylvania, in the western part of that state. I have a BA in philosophy and history, a Juris Doctor, a Master of Laws in International Law, and a Master of Public Health. I'm about to complete my Master of Business Administration, and I'm looking to continue my formal education in a doctoral program in Washington, D.C. As for how I came to PPTA, I had several different experiences through my graduate education, ranging from appellate legal practice at a private law firm focusing on Constitutional and intellectual property issues, to regulatory enforcement at the U.S. Environmental Protection Agency, health policy and risk policy think tanks affiliated with universities, and as a legal consultant for the World

Health Organization during the final round of negotiations for the Framework Convention on Tobacco Control. At the same time, I was interning in the Scientific and Regulatory Affairs Division at the Pharmaceutical Research and Manufacturers of America (PhRMA) and interviewed with PPTA. I've been here ever since! As far as hobbies and interests, beyond school, I'm a voracious reader—I read around 150 books per year, in many different subjects (though I favor early medieval English history, modern mysteries, and social and political philosophy)—and, when time allows, hunting and fishing. I lift weights to stay in some kind of shape, and I love to

write as well. I'm a bit of a film buff, and I also enjoy target shooting and archery.

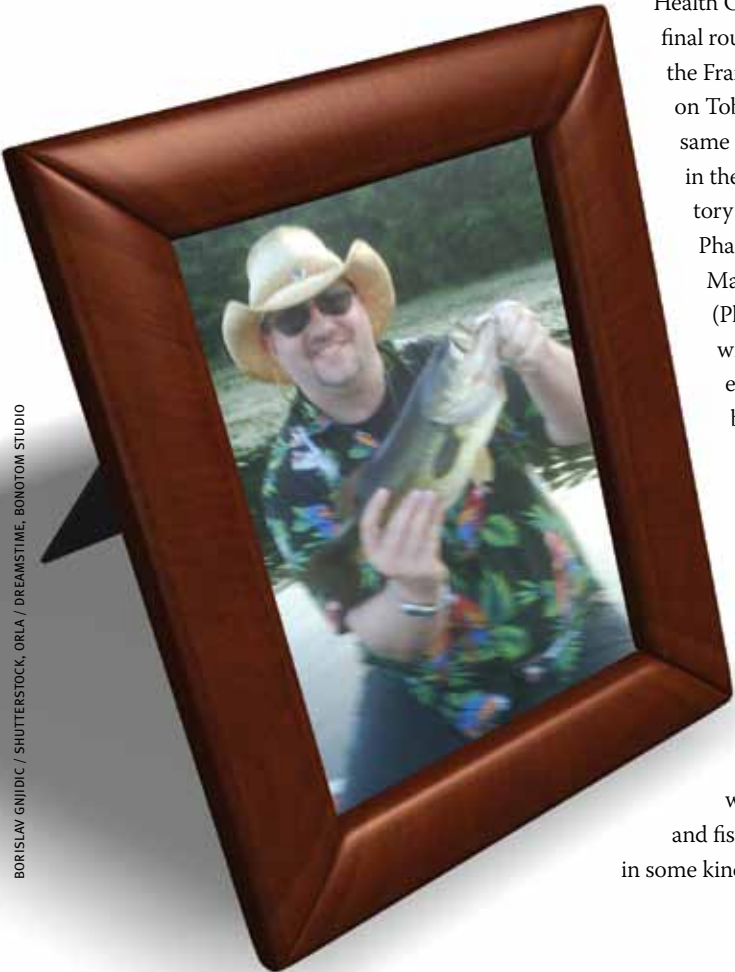
**What is your proudest professional achievement at PPTA?**

One is having a rewarding and effective relationship with the Source Board of Directors. This is the foundational part of being able to achieve anything on behalf of the industry, given the wide range of issues that we face. Being able to draw on the experience of that group, along with my colleagues at PPTA, is the key to the many things that we've done over the past several years: a hugely successful industry image and credibility campaign, a complete overhaul of the International Quality Plasma Program (IQPP), the addition of a new standard, the successful culmination of several regulatory issues, and many others.

The other is being able to work with a very talented group of committed professionals at PPTA. We have a tremendous array of expertise and talent in the PPTA organization and I'm fortunate to be able to have such resources that can be drawn on at any time in both the U.S. and in Europe.

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

I really enjoy meeting the people in the industry. This includes working with the Board of Directors, of course, and the groups of industry experts comprising our many working groups, but I also really enjoy meeting the folks who work in plasma collection all over the world. There are a lot of individuals out there who are dedicated to the industry, collecting plasma and saving the lives of thousands of people around the world. I learn something new from each place I visit and every person I meet, and that enriches my life far more than I ever would have imagined prior to my time at PPTA. ☺



BORISLAV GNJIDIC / SHUTTERSTOCK, ORLA / DREAMSTIME, BONOTOM STUDIO



# EVENTS

UPCOMING  
CONFERENCES  
& SYMPOSIUMS

## 2011

**February 2 – 4** 4<sup>th</sup> Annual Congress of the European Association for Haemophilia and Allied Disorders  
*Geneva, Switzerland*

**March 13 – 17** 6<sup>th</sup> World Congress on Paediatric Critical Care  
*Sydney, Australia*

**March 15 – 16** **International Plasma Protein Congress**  
*Lisbon, Portugal*

**March 22 – 25** 31<sup>th</sup> Symposium on Intensive Care and Emergency Medicine  
*Brussels, Belgium*

**March 31 – April 1** 12<sup>th</sup> WFH Musculoskeletal Congress  
*Cairo, Egypt*

**May 10 – 11** The 2<sup>nd</sup> International Congress on Transfusion Medicine-Plasma Industry  
*Tehran, Iran*

**June 9 – 12** 16<sup>th</sup> Congress of the European Hematology Association  
*London, United Kingdom*

**June 14 – 15** **Plasma Protein Forum**  
*Reston, Virginia, United States*

**June 18 – 22** XXI<sup>st</sup> International Congress of the International Society of Blood Transfusion (ISBT)  
*Lisbon, Portugal*

**September 22 – 23** The Seventh WFH Global Forum on the Safety and Supply of Treatment Products for Bleeding Disorders  
*Montreal, Canada*

**October 7 – 9** European Haemophilia Consortium Conference  
*Budapest, Hungary*

**October 22 – 25** AABB Annual Meeting  
*San Diego, California, United States*

**November 20 – 23** XXII Regional Congress of the ISBT, Asia  
*Taipei, Taiwan*

## 2012

**July 7 – 12** XXXII<sup>nd</sup> International Congress of the ISBT  
*Mexico City, Mexico*

Lisbon, Portugal



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