

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

WINTER 2011

The Global Economic Crisis Breakdown

Entitlements are
Under Scrutiny

**Prof. Vincent
Discusses Albumin**

Access to Orphan Drugs

**PID Patient Fights
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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.

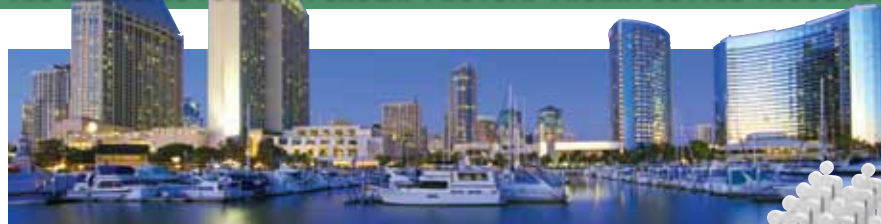
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INSIDE

WINTER 2011

THE MAGAZINE FOR THE PLASMA PROTEIN THERAPEUTICS INDUSTRY



2 IN MY VIEW How PPTA Dealt with Shortages

4 PPTA INTERVIEW Prof. Jean-Louis Vincent, M.D., Ph.D. Professor of Intensive Care, Université Libre de Bruxelles and the Head of the Department of Intensive Care, Erasme University Hospital.

6 Source Business Forum 2011 Twenty Years of IQPP

8 H.R. 2672: "Preserving Access to Orphan Drugs Act of 2011" Congressman Jim Gerlach explains his introduction of the Act.

10 PATIENT ADVOCATES PID Patient Channels Pain of Fighting Illness & Insurance Coverage into Helping Others.

12 What is Discussed Behind Closed Doors? Interaction between the European Medicines Agency (EMA) and the US Food and Drug Administration (FDA).

16 Working for Patient Access to Blood Clotting Factor Patient access is always a concern for Medicaid recipients because Medicaid reimbursement is low when compared to private insurance and Medicare.

18 The Global Financial Crisis and the Plasma Protein Patient Community Troubling indications are emerging that the community of patients needing plasma protein therapies (PPTs) are potentially very vulnerable to its effects.

21 Deficit Reduction Efforts Target Medicare The Super Committee's failure triggers a statutory procedure known as sequestration, resulting in indiscriminate across-the-board cuts.

24 INSIDE PPTA PPTA News from Around the Globe

27 Meet the PPTA Staff Cathy Izzi

28 EVENTS Upcoming Conferences & Symposiums



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

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COVER ART: WEBMINK, JASTAN / ISTOCK, BONOTOM STUDIO





IN MY VIEW

HOW PPTA DEALT WITH SHORTAGES


IN SEPTEMBER 2011 FDA (CDER) organized a public Workshop to address drug shortages in the United States of America. Many speakers voiced their concern about drug shortages, especially in the areas of anesthetics, parenteral food, cancer treatments and antibiotics. Late October, President Obama signed an Executive Order to double the number of FDA staff working on this important issue.

I remember as if it were yesterday the enormous attention we got from patients, treaters, congress and media when there was a shortage of immune globulins in the late 90's. I had just moved to the United States and suddenly I learned firsthand what it is to be in the middle of congressional and media attention. My introduction to the United States was being interviewed by Mike Wallace on *60 Minutes*, being interviewed by the *New York Times* and CNN, and not to forget the 2 Congressional Hearings in 1998. By the way, the second Hearing in September 1998 was relatively short because it was the day that Ken Starr published the "Lewinsky Report" which seemed to be far more important for the House of Representatives than our issues.

During the FDA Workshop we heard several statements that demonstrate the outrage by many participants: "Drug shortage situation is unacceptable.... Cannot believe this is happening in America... Action is needed now..." "Drug shortage is a public health problem and responsibility of everyone in the room..." "The situation calls for real-time, bi-directional communication on supply between manufacturers and FDA/ stakeholders..."

It sounded very similar to the comments that we heard in the late nineties and were the basis for the Advisory Committee on Blood Safety and Availability (ACBSA) to develop their recommendation to develop a program that collects and disseminates standardized information on supply, with involvement of the trade association and preferably on a monthly basis.

PPTA made a statement at the FDA Workshop to explain the history of our data system and its utility to patients, regulators and other stakeholders. Notably, while many workshop participants focused on the problems associated with drug shortages, PPTA's presentation was the only one that described a concrete, time-tested plan of action. Stakeholders like patient groups and FDA (CDER) have repeatedly stated their appreciation for the system developed by PPTA. Like any system that was developed over a decade ago, it is always necessary to look at it and see whether improvements can be made. That speaks for itself. But we should never forget that having data available whenever there are questions about a potential shortage is a fundamental tool to clarify any concern that may exist. Over the years we have learned that and were always able to respond in a responsible manner to stakeholders.

We received many compliments over the years about our system and we are thankful for that. I hope that our system will maintain its importance and can be a model for other sectors of the pharmaceutical industry that are currently facing similar problem. 

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PROF. JEAN-LOUIS VINCENT,

Professor of Intensive Care, Université Libre de Bruxelles and the Head of the Department of Intensive Care, Erasme University Hospital.

PROFESSOR VINCENT IS PRESENTLY SECRETARY GENERAL of the World Federation of Societies of Intensive and Critical Care Medicine. He is a former President of the European Society of Intensive Care Medicine (ESICM), the European Shock Society (ESS) and Past-Chair of the International Sepsis Forum (ISF).

Dr. Vincent is the editor in chief of *Critical Care*, *Current Opinion in Critical Care and ICU Management*. He is a member of the editorial board of about 30 international journals including *Critical Care Medicine* (Senior Editor), *Public Library of Science* (PLoS), *Lancet Infectious Diseases*, *Anesthesiology*, *Intensive Care Medicine*, *Chest*, *Shock* and *Journal of Critical Care*.



randomized some 7,000 critically ill patients to fluid resuscitation with 6 percent HES (130/0.4) or saline (0.9 percent sodium chloride).

The ISICEM program will also cover many other aspects, including the results of the PROWESS-SHOCK study that randomized

1,700 patients with septic shock to receive placebo or activated protein C – unfortunately, the results showed no effect of activated protein C on mortality rates and this drug has now been withdrawn—these disappointing findings are certain to create a lot of discussion.

Research Interests

Dr. Vincent's main fields of research interest and investigation are sepsis, acute circulatory failure (circulatory shock) and its treatment, oxygen transport, haemodynamic monitoring, and vital emergencies and he has a special interest in the ethical aspects of intensive care.

The 32nd International Symposium on Intensive Care and Emergency Medicine (ISICEM) is coming up soon in March 2012. Could you tell us a little more on this year's event looking at the scientific programme and global participation of speakers and participants?

The scientific program again promises to be very full and will include the results of several important, recently completed studies. In the field of intravenous fluids, we will have important data on the use of albumin solutions in septic shock. Further analysis of data from the SAFE trial conducted in 2001-2003 have suggested a beneficial effect of albumin especially in the presence of hypoalbuminaemia, and the results of a meta-analysis of albumin use in patients with sepsis also support an outcome benefit with use of albumin as resuscitation fluid in patients with severe sepsis. The results of the Italian multicentre ALBIOS study, in which patients with severe sepsis or septic shock were randomised to receive either albumin or crystalloids as resuscitation fluid will be presented during the 32nd ISICEM and are awaited with some interest. We will also have the results of several studies on the safety of hydroxyethyl starch (HES) solutions, in particular their effects on kidney function, a really important but unanswered question. The largest of these will be the Chest study that

What is your view on plasma protein therapies in the area of intensive care medicine?

I'll restrict my answer to discussion of albumin. Generally, albumin solutions are considered as costly, but we don't dispense these solutions in large quantities, rather use them judiciously. There are many costly therapies today. More importantly, costs are not really so high if you consider the potential savings associated with even a small reduction in severity of organ failure or length of stay in the ICU. There is now better evidence that albumin supplementation in septic patients with hypoalbuminaemia can be beneficial. Moreover, the relatively poor efficacy of gelatin solutions and the adverse effects of HES on haemostasis and perhaps other aspects, including renal function, raise questions about the true value of alternative colloids. Nevertheless, it's good to be able to have a choice of intravenous solutions; after all, we don't drink the same kind of fluid at breakfast as we do at an evening party! And too much of any type of fluid (even water) can be harmful.

You are the Editor-in-Chief of *Critical Care*, a high quality, peer-reviewed, international clinical medical journal. Could you explain to our readers the advantages of Open Access journals?

I believe Open Access is the future of journal publications—all research should be widely accessible. In the past, potential readers had to buy the journal to get the article; in this new paradigm, the authors have to pay the journal but readers then have free access so that study results are available to all. There are several advantages to this system: First, easy


M.D., PH.D.

access means that new discoveries can be more rapidly disseminated and, therefore, hopefully more rapidly implemented. Second, access is not restricted to those who can afford it or who belong to institutions with subscriptions – everyone, even those in less wealthy regions of the world, can benefit from being able to read (and apply) the very latest experimental and clinical research findings. Authors are sometimes upset that they have to pay 1-2,000 euros to have their paper published, but we just need to change our approach to publishing and start to include these costs in the study's budget. Moreover, most journals will waive these charges if the author is unable to pay, e.g., is from a less-developed country. Ultimately, all journals will move towards open-access. Indeed, the National Institutes of Health (NIH) already requires all NIH-funded investigators to make their final manuscripts freely available to all.

You have a special interest in the ethical aspects of intensive care, could you share with our readers what this means to you?

Ethics is a huge and fascinating topic, involved in so many different aspects of intensive care medicine including admission/discharge criteria, resource allocation, research principles, end-of-life care, organ transplantation, etc. As such, ethical issues are faced by all intensivists on a regular basis, and we should all have some training in these issues. Importantly, when considering any ethical issue, we need to go back to the four fundamental principles: autonomy; beneficence; non-maleficence; and distributive justice. The fundamental goal of medicine is to restore or maintain health in all its components, i.e., physical, mental and social well-being. Although maintaining life is generally the goal of any form of medical therapy, sometimes well-being is more important and sustaining life in a patient with no chance of meaningful recovery may actually be contrary to ethical principles. Crucially, many ethical conflicts arise from poor communication, and we should all try to develop good relations with our patients and the rest of the caregiving team, so that any ethical issues that may arise can be openly discussed and appropriate decisions made. ☞

MENSO BULT is PPTA Europe's Manager, Global Affairs

A portrait of Menso Bult, a middle-aged man with dark hair, wearing a grey suit and a white shirt. He is resting his chin on his hand, looking thoughtfully at the camera.

▶ When considering any ethical issue, we need to go back to the four fundamental principles: autonomy; beneficence; non-maleficence; and distributive justice. The fundamental goal of medicine is to restore or maintain health in all its components, i.e., physical, mental and social well-being.



SOURCE BUSIN

BY SONIA BALBONI

THIS YEAR'S BUSINESS FORUM in beautiful San Diego was a celebration of the twentieth anniversary of the IQPP Standards Program. Enormous changes have occurred within our industry over the past two decades, and members were ready to share their perspectives on the events that shaped the way we operate today. Attendees were warmly welcomed by Source Board Chair Ileana Carlisle, who gave an overview of the Association's activities that provide value to PPTA Source members. PPTA President Jan Bult then discussed the role of the Association in our changing industry environment. Mr. Bult gave us a glance at the results of a recent survey among members, stakeholders and staff on the current work of the



From Left to Right: Ileana Carlisle (Source Board Chair) Biotest Pharmaceuticals, Toni Atkins, California Assembly member, Joshua Penrod, PPTA Vice President, Source

Association, and explained how leadership is considering what they might mean for the organization's future direction.

Dr. Albert Farrugia (Vice President, Global Access) then treated us to an inspired discussion on donor motivations, first deliberating on the works of philosophers Titmuss and Mauss, and then analogizing Maslow's Hierarchy of Needs to the driving forces of donor motivation. Dr. Farrugia concluded that, while the whole blood and plasma sectors have distinct systems, they can "coexist productively and are both essential if we are to continue saving lives."

Sou-Tei-Gai! That was the take-home message from Mr. Shinji Wada (Grifols), who reflected on the industry twenty years ago, before the standards program or even the Quality Plasma Principles existed. *Sou-Tei-Gai* is a Japanese expression meaning,

"Outside of Assumptions," or "Never Even Dreamed of." Mr. Wada reflected on the commitment that many individuals and companies invested in developing a common set of criteria that every plasma collection operation should live up to, which ultimately resulted in the IQPP Standards Program that we now apply on a daily basis. Mr. Wada noted improvements and the higher level of confidence that stakeholders have now as a result of industry's efforts.

EKASH, PENFOLD / ISTOCK

ESS FORUM 2011

► Twenty Years of IQPP

However, he noted that in our industry new challenges have, in the past, emerged unexpectedly, and they will continue to do so in the future. Thus, he concluded, our therapies will be safer if we “continue to accept challenges as our predecessors did twenty years ago, with their convictions!”

Dr. Jon Knowles (CSL Plasma) gave an insightful presentation on the differing FDA and EMA inspection programs, with thoughts on what we might expect from both agencies in the future. Attendees were also briefed by members on the Association’s activities that provide value to PPTA Source members, including work on the Industry Image Campaign (Dan Gamache, Biotest Pharmaceuticals), a report from Source Board Treasurer Bill Bees

(Cangene Corporation), an overview of accomplishments by the Regulatory Affairs Steering Committee (John McVey, Baxter Biolife Plasma Services), and an informative look at issues confronting European collectors (Dr. Gerold Zerlauth, Baxter Healthcare SA).

Attendees were also greeted by California Assembly member Toni Atkins (D). PPTA honored Assembly member Atkins as State Legislator of the Year, for her sponsorship of the Medi-Cal Clinical Laboratory and Laboratory Services and for her work to promote patient access.

The event culminated in a reception featuring chocolates sporting the IQPP logo, designed by Dinstuhl’s Fine Candies of Memphis, and commissioned by Source Board Member

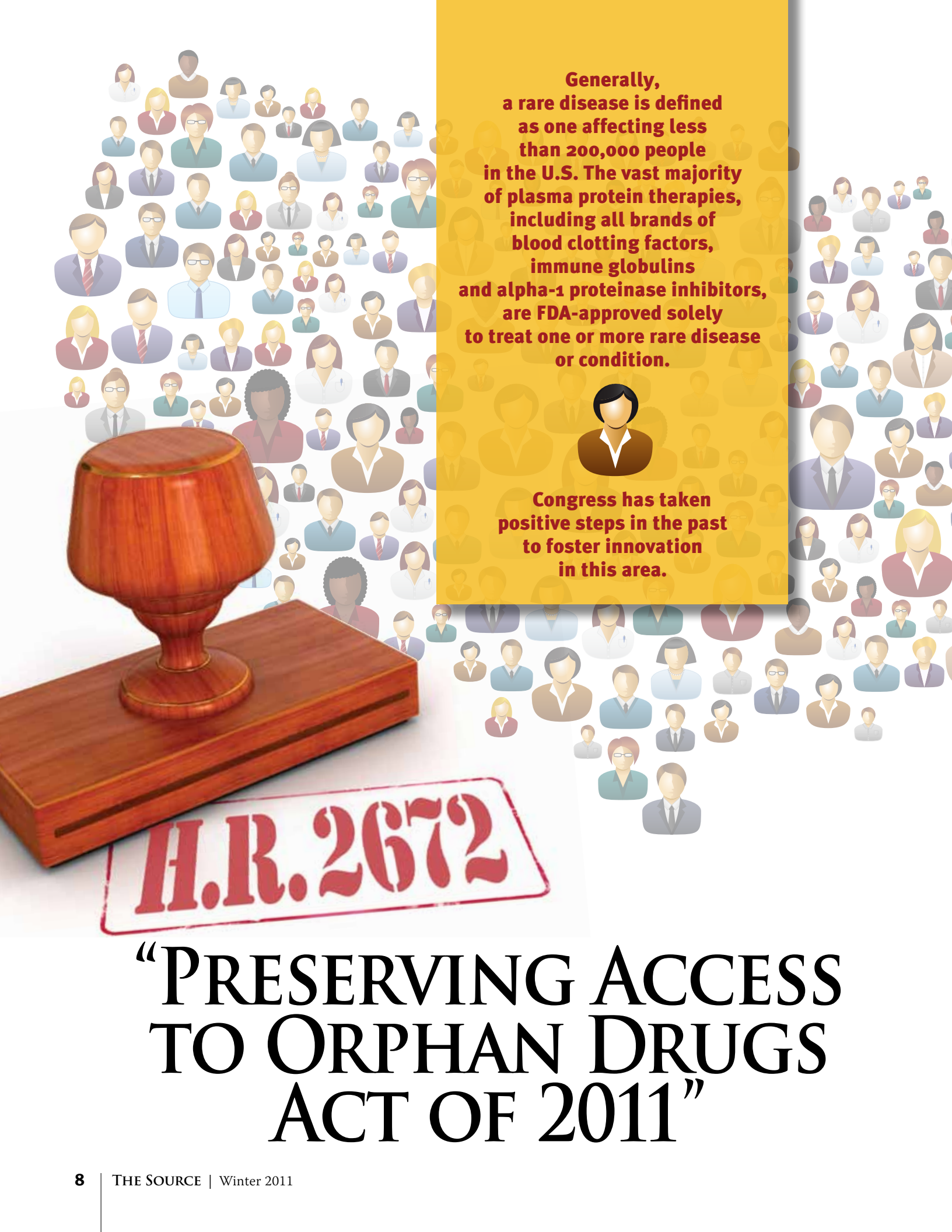
Larry Moss (The Interstate Companies). The PPTA staff thanks members for another successful event, and for their continued participation in the many Source programs that contribute to the overall success of the Association and the industry we represent. 🍫

SONIA BALBONI is PPTA’s Manager, Source & Standards



From left to right: California Assembly member Toni Atkins (D), Joshua Penrod presents Barbara Glantschnig of Baxter AG with an award for her years of commitment and service on the Source Board, and Larry Moss of the Interstate Companies





Generally, a rare disease is defined as one affecting less than 200,000 people in the U.S. The vast majority of plasma protein therapies, including all brands of blood clotting factors, immune globulins and alpha-1 proteinase inhibitors, are FDA-approved solely to treat one or more rare disease or condition.



Congress has taken positive steps in the past to foster innovation in this area.

H.R. 2672

“PRESERVING ACCESS TO ORPHAN DRUGS ACT OF 2011”

BY CONGRESSMAN JIM GERLACH

Why did you introduce H.R. 2672, the "Preserving Access to Orphan Drugs Act of 2011?"

Patients coping each day with rare and often life-threatening diseases depend on having continued access to the latest, most-innovative treatments. It is critical to ensure that the enactment of the Affordable Care Act does not put these treatments out of reach for patients or discourage investment and research essential to developing new drugs and improving the effectiveness of others used to treat rare diseases.

Generally, a rare disease is defined as one affecting less than 200,000 people in the U.S. The vast majority of plasma protein therapies, including all brands of blood clotting factors, immune globulins and alpha-1 proteinase inhibitors, are FDA-approved solely to treat one or more rare disease or condition. Congress has taken positive steps in the past to foster innovation in this area. With the creation of the Orphan Drug Act (ODA) in 1983, Congress has recognized the importance of investing in the research and development of drugs and therapies intended to serve this small patient population.

Since its enactment, the ODA has successfully supported bringing more than 350 drugs and biological therapies to market. By comparison, fewer than 10 products to treat rare diseases came to the market in the 10 years prior to the ODA enactment. Congress should not turn back the clock to an era when it was much more difficult to get these medicines to the patients whose lives depend on them.

However, there are serious concerns that the Affordable Care Act (ACA) enacted in March of 2010 could jeopardize the progress made since enactment of ODA. The health-care law placed a new annual pharmaceutical fee on the sale of branded drugs. That law exempts orphan drugs from paying the fee, but the exemption

is narrowly defined. H.R. 2672 would clarify or modify that exclusion to apply to all drugs solely indicated to treat one or more rare diseases. This legislation would preserve and build upon the incentives for orphan drug development that Congress has supported for more than two decades.

The common sense legislation I've introduced has the support of fellow Pennsylvania Congressman Jason Altmire of the 4th District, who is an original co-sponsor of the bill. Both Congressman Altmire's district in Southwestern Pennsylvania and my district in Southeastern Pennsylvania are home to a number of innovative biotechnology and biopharmaceutical manufacturers. Every day, the talented researchers and workers at these companies produce therapies for rare diseases. The annual fee in the health care law enacted in 2010 would hamper these efforts and could stifle innovation into orphan therapy development. A number of manufacturers, including CSL Behring, the members of PPTA, and several other biotechnology companies in Pennsylvania have been instrumental in raising awareness about the need for H.R. 2672 and how this proposal would spur innovation.

I'm also pleased that Pennsylvania's two U.S. Senators, Pat Toomey and Robert Casey Jr. along with Senator Wyden from Oregon have introduced an identical, bipartisan bill in the Senate. Further, patient groups support this legislation, including the National Organization for Rare Disorders, the Immune Deficiency Foundation and the Alpha 1 Foundation. All of these groups understand the need to preserve future development of rare disease therapies. I am hopeful that all of these efforts will result in enactment of our legislation, but more importantly continued access to treatment for patients and continued investment in the companies that create jobs and contribute to the vitality of our communities. ☺



H.R. 2672, the "Preserving Access to Orphan Drugs Act of 2011" introduced by Rep. Gerlach in the summer would modify the orphan drug exclusion of a new tax on branded drugs enacted as part of the Affordable Care Act (ACA).

ICONEER, HRONOS7 / ISTOCK, BONOTOM STUDIO

PID Patient Channels Pain of FIGHTING ILLNESS & INSURANCE COVERAGE into Helping Others

UNLIKE MANY PATIENTS who learn they have a primary immune deficiency (PID), Jenise Turner was perfectly healthy until her early 30s. She had three children, no health problems and no family history of a PID that anyone could pinpoint. Then the headaches began, followed by frequent sinus infections, and her health got progressively worse.

Eventually, Jenise was diagnosed with common variable immune deficiency (CVID) after having undergone sinus surgery in 2003 and fighting a massive post-operative infection that led to additional surgery. Jenise then developed MRSA, a type of bacterial staph infection that is extremely difficult to treat.

Throughout this ordeal, Jenise's treating physician, an ear, nose and throat doctor, kept reiterating, "this is not normal; this never happens." She was referred to an immunologist/allergist, who eventually diagnosed her with CVID in the spring of 2004.

At the time, Jenise's IgG levels were only slightly low and she did not qualify for treatment with immune globulins (Ig). For the next two years, Jenise was sick all of the time, underwent four more sinus surgeries, and was treated with oral and intravenous (IV) antibiotics, but her health continued to deteriorate. By 2006, she had been so sick and had missed so much work that she lost her job as a registered nurse working in an oncology office. "I loved what I did and was devastated," Jenise said of losing her job.

Health Declining, Fighting for Coverage

Jenise reached out to her immunologist, who retested her and learned that her IgG levels had dropped into the 500s. However, a new journey was only beginning. Her current insurance provided by her husband's employer denied the Ig treatment for two years, and Jenise's health continued to suffer. She consistently remained on IV antibiotics and was told repeatedly by the health insurance company that Ig treatment was not medically necessary. She ended up getting MRSA again in her sinuses and was referred to an infectious disease specialist who submitted the test results to her insurance company hoping to get the Ig treatment approved. It was not.

"It didn't matter; we had culture reports, operative reports, whatever the reason, it was denied," Jenise said. "The illness had already significantly affected my life, I lost the job I loved and kept getting sicker." Jenise describes the experience as heartbreaking and frustrating.

IDF Connects Patients

Jenise describes one bright note during this time was that she found the Immune Deficiency Foundation (IDF) and started communicating with other patients. What struck her was that some of the people she met through IDF's network, who were not as sick as she was, were able to get insurance coverage for Ig treatment. Jenise describes her connection with IDF patients as a "real eye opener." "It helped me fight even more," she said.

Jenise started a new nursing job in 2006 at a small hospital, but resisted enrolling for her employer-sponsored health insurance, for reasons she characterizes as just feeling powerless and almost convinced that she really didn't need the treatment. She received her last denial from her original health insurance provider in April 2008, and on the last day of eligibility, she applied for health insurance through the hospital where she working. In three weeks, her treatment was approved.

Jenise started using Ig therapy in August of 2008, however given the severity of her frequent illnesses, it took nearly six months for her to begin to feel better and have more energy. "There were so many low lying infections that my body could finally address," she said.

Diagnosis of MBLD

Two years after Jenise started on Ig therapy, she continued to contract various infections. Oral antibiotics were not effective and

again she needed to rely on IV antibiotics. At this point, her immunologist conducted additional testing. While Jenise's IgG levels were good, what they did learn was that she had another, extremely rare immune disorder, Mannan-Binding Lectin Deficiency (MBLD), a complement disorder of the immune system that is concerned with innate immunity. "It prevents the body from recognizing it needs to fight infection," Jenise said. When in combination with another PID, it can cause severe illness.

Currently there is no treatment for MBLD, but Jenise says at least she has more answers, more pieces to the puzzle about her health. She explains how important it is for her to be on prophylactic antibiotic treatment, and when she does become sick, doctors treat her more aggressively.

Life-changing Illness

Jenise says in 2010 she had 92 doctor appointments and missed more work than she attended, which inevitably caused her to leave her job. Being a nurse working in a hospital, she was routinely exposed to infection, and her immunologist finally insisted that she stop working in order to protect her health. "Being a nurse is part of who I am," she said. "I've had a hard time finding myself, it's hard financially and I loved being a nurse. What has helped me cope is becoming involved with the IDF as a volunteer."

Jenise describes her first engagement with IDF as talking with

other people who "get it." "It was life-changing," she says. Jenise was able to attend some of the IDF conferences and recalls the emotions of meeting with some of the people she had connected with online through the IDF forum on its website. Now, Jenise is an active advocate with IDF who regularly talks with new patients, advocates on behalf of PID patients and delivers information about PIDs to doctors around her Wichita, Kansas home. 🐝

KYM H. KILBOURNE is PPTA's Director, Federal Affairs.



WHAT IS DISCUSSED BE

Interaction between the European Medicines Agency

BY ILKA VON HOEGEN

PLASMA PROTEIN THERAPIES are marketed globally, but their approval and the subsequent monitoring of the therapies throughout their life cycles is done by National Competent Authorities, with often different approaches and philosophies towards licensure of medicinal products. It is inevitable that the divergent national approaches result in redundancies and are cumbersome for both, manufacturers as well as regulators.

Regulators recognize the problems and in recent years have commenced to work towards more similarity in their regulatory approaches. In 2003 the European Commission's European Medicines Agency (EMA) and the U. S. Food and Drug Administration (FDA) signed confidentiality arrangements as part of a framework for collaboration between the two agencies. After agreeing on the implementation plan and an extension of the agreement in 2005 the arrangements were extended indefinitely in 2010. Additionally, in 2008, both agencies agreed on an action plan on transatlantic administrative simplification that would benefit both the industry and the regulators by leveraging resources.

The scope of the exchanges between EMA and FDA is on centrally authorized products in the EU (unlike the US, European regulation of therapies includes that of the individual member states as well as the EU). While FDA cannot share personal data or trade secrets, it can share non-public information, such as commercial confidential, investigative, and pre-decisional information. The agencies also share preliminary thinking on guidance development, policies, internal analyses and enforcement actions.


The agencies exchange assessment reports, share pharmacovigilance and inspection information. They also perform joint manufacturing facility inspections. Regular interaction takes place in topic specific groups, the so-called clusters, for example for pediatrics, orphans,



BLITZ, YURA23 / ISTOCK, PHOTO OBJECTS.NET, BONOTOM STUDIO

HIND CLOSED DOORS?

(EMA) and the US Food and Drug Administration (FDA)

A large American flag is positioned on a silver stand in the foreground. Behind the flag is a white, ornate door with gold-colored hardware. The scene is set in a room with light-colored walls and a patterned rug.

pharmacoeconomics, or pharmacovigilance. Recently a blood cluster has been established which will focus on plasma protein therapies. As in the other clusters, experts from both agencies address policy issues, guidance, product development and potential product safety signal. It is the ambitious aim of the exercise to establish streamline approaches to global product development starting with scientific assessments and potential agreement on the design of clinical studies and other particulars.

Are these developments a step into the right direction? Do pharmaceutical manufacturers have a choice? Probably not, the exchange is under way and both agencies have agreed not to disclose what they share.

Information is flowing back and forth throughout the entire life cycle of a product, from the first development phase, when a manufacturer seeks scientific advice through to product approval and product surveillance. FDA and EMA are sharing listings of ongoing EMA marketing authorization application and FDA applications (NDAs/BLAs) during the product evaluation phase giving both agencies the opportunity to follow product development and approval, even if the product is not intended for the market under their jurisdiction.

If a manufacturer intends to market the product in both markets, he needs to avoid inconsistency in the information provided to either side. With different regulatory schemes and reporting requirements, maintaining consistency is not as easy as it might seem.

Manufacturers have the option to seek Parallel Scientific Advice to ensure that both agencies have agreed the development plan for a candidate product, which should in the end facilitate the approval of the product in the US and the EU. The manufacturer submits the necessary documentation with a view to serve the needs of both agencies and waits for a positive opinion. But what if there is no agreement? Then the manufacturer has several options, depending of the nature of the discrepancies. He could approach both agencies individually with a submission tailored to the FDA's or EMA's needs. He could focus only on one market, either EU or US. But then the agencies exchange information that are not

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INTERNATIONAL PLASMA PROTEIN CONGRESS

March 13, 2012 | 9:00 - 17:30 hrs

SESSION 1: KEYNOTE SESSION | 09:00 - 10:30 hrs

Chair: C Waller, PPTA

- Industry Update (P. Perrault, PPTA Global Chairman)
- Evolution of Clinical Use of PPTs 2007-2011 (P. Robert, Marketing Research Bureau)

SESSION 2: FINANCIAL PRESSURES FACING THE INDUSTRY | 11:00 - 12:30 hrs

Chair: J. Birkofer, PPTA

- How is the Pharmaceutical Sector? (TBC)
- SWOT Analysis for the Plasma Protein Industry (R. Waeger, Consultant)
- US State Developments (B. Speir, PPTA)1

SESSION 3: HEALTH TECHNOLOGY ASSESSMENTS AND RARE DISEASES | 14:00 - 15:30 hrs

- HTA's & Chronic Rare Diseases – Compatible? (TBC)
- Clinical and Scientific Evidence (A. Farrugia, PPTA)
- Swedish Experience: the history so far (B. O'Mahony, EHC)

SESSION 4: EU US REGULATORY DEVELOPMENTS: IS THERE A CHANCE FOR HARMONIZATION | 16:00 - 17:30 hrs

Chair:

- EMA: Current Priorities (EMA Representative)
- FDA: Recent Developments (G. Michaud, FDA/ CBER)
- EU US Collaboration and Information Sharing (TBC)

March 14, 2012 | 8:00 - 16:00 hrs

SESSION 5: INTERNATIONAL SNAPSHOTS | 08:00 - 10:30 hrs

Chair: J. M. BULT, PPTA

- LASID: Patients' Role (R. PENA, LASID)
- Fractionation in India and Sub Continent (K.V. Subramaniam, Reliance Life Sciences)
- Iran: Collecting High Quality Plasma for Fractionation (M. Cheraghali, Iranian Blood Transfusion Organization)

SESSION 6: PLASMA: CHALLENGES OF THE FUTURE. WHERE TO GET THE DONORS FROM? | 11:00 - 12:30 hrs

- Demographic Development: The Impact on Safe Blood Supply (M. Greinacher, University of Greifswald)
- The Czech Experience (M. Maly, UNICA Plasma)
- The continuing Evolution of a most Peculiar Medicine. Where next for Labile Blood Products? (W. Murphy, Irish Blood Transfusion Service)

SESSION 7: ESTABLISHED PRODUCTS, NEW INDICATIONS, GREAT FUTURE | 14:00 - 16:00 hrs

Chair: P. Spaeth, University of Bern

- Neurological Immunomodulation Clinical Data (TBC)
- Albumin for Alzheimer Disease (A. Paez, Grifols)

For more information visit our website at

WWW.IPPC.NET

In the current situation providing consistent information is a difficult task, since it is well accepted that the overall philosophy of FDA and EMA are frequently divergent.

disclosed and leave the manufacturer in a situation of greatest uncertainty and risk to lose the investment.


Increased communications and forums for regulatory Policymakers make information related to negative events more easily and quickly transmitted. This often results in making a local event into a global event.

In the current situation providing consistent information is a difficult task, since it is well accepted that the overall philosophy of FDA and EMA are frequently divergent. While EMA has established a catalogue of detailed guidance documents for manufacturers to follow, the FDA has a more flexible approach, making decisions on a case-by-case basis until it gains sufficient experience to develop formal guidance. The EMA Roadmap 2015 foresees to simplify the very complex EU system and the current proposal resembles the FDA approach, thus giving hope for transatlantic administration simplification in the future.


Within the agreements between the agencies there are elements that are designed to precisely address the need to develop common understandings, for example the exchange on guidance development or a practical example, the joint facility manufacturing inspections.

Product surveillance is another area of cooperation.

Regulatory participants exchange findings on new safety signals and discuss interpretation of the data with a view on potential label/SmPC changes. It has to be noted that there are a number of differences between FDA's Risk Evaluation and Mitigation Strategies (REMs) and EMA's Risk Management Plan (RMP) mainly in relation to risk communication, where EMA relies mostly on SPCs while the FDA has Communication plans in place to directly inform patients and health care professionals. Again, a manufacturer providing product to both markets has to accommodate the different expectations of both agencies with a view on the mutual exchange between them.

The globalization of pharmaceutical regulation and distribution is part of today's reality. Manufacturers of pharmaceutical products have to respond to the challenges arising from cooperation between regulatory authorities albeit with different approaches and philosophies. 

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



Save the Date


PPTA's 20TH ANNIVERSARY EVENT

Wednesday, June 20, 2012
6:30 PM

The Pavillion
at the Ronald Reagan Building
Washington, DC

Silent Auction
and Dinner

Special Recognitions
and Reminiscing



PPTA
Plasma Protein Therapeutics Association

WORKING FOR PATIENT ACCESS TO BLOOD CLOTTING FACTOR

BY BILL SPEIR

THE UNITED STATES OF AMERICA provides health care services to low-income individuals through its Medicaid program. The administration of the Medicaid program is a partnership between the federal government and the states.

Basically, the federal government establishes minimum requirements, and the individual states design their program as they see fit, as long as a state program meets the approval of the federal government's Centers for Medicare and Medicaid Services (CMS).

Patient access is always a concern for Medicaid recipients because Medicaid reimbursement is low when compared to private insurance and Medicare. And since providers are not required to participate in Medicaid, finding providers willing to take the low reimbursement is always a concern for Medicaid recipients.

Many individuals with bleeding disorders are Medicaid recipients. Access to blood clotting factor, a plasma protein therapy, is always a concern and any changes to Medicaid reimbursement could result in access problems for those that rely on blood clotting factor for their quality of life.

Since the 1960's, the benchmark for the Medicaid pharmacy reimbursement estimated acquisition cost has been the Average Wholesale Price (AWP), a value based on manufacturer-reported information and compiled by commercial drug pricing compendia. The decision of First DataBank, a major drug pricing compendia to cease publication of AWP in September, 2011 as part of a court settlement was the impetus for state Medicaid programs to find a new benchmark.

In June of 2010, a white paper was developed by The American Medicaid Pharmacy Administrators Association that recommends a change in Medicaid pharmacy reimbursement methodology from AWP to average actual acquisition cost (AAC). The white paper does not address the other piece of outpatient pharmacy reimbursement, a dispensing fee, but it is critical to patient access that the dispensing fee adequately reflects the true cost of dispensing blood clotting factors. If the dispensing fee is established without consideration of the challenges faced in dispensing blood clotting factors, this could result in serious patient access issues.

Key decision-makers are struggling with how to develop an appropriate dispensing fee because some lack the knowledge of how blood clotting factors are delivered and the services necessary for that delivery and medically appropriate administration.

In addition to this change, state Medicaid agencies are under tremendous pressure to control costs given the state budget deficits are at historic levels. 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 implement the new Medicaid pharmacy methodology and 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 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. Clotting factor therapies are vital for individuals with bleeding disorders, including hemophilia and von Willebrand Disease.

Current SPAC Members

Accredo Health Group, Inc.	Curascript, Inc.
Baxter Healthcare Corp.	CVS Caremark
Bayer Healthcare	Grifols USA
Biogen Idec	Novo Nordisk
Cangene Corporation	Pfizer
CSL Behring	Walgreens

The SPAC's goal is to educate decision makers in CMS, state Medicaid agencies, and state legislatures on the need for open access to all blood clotting factors. 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.

The SPAC will also respond to threats to patient access to blood clotting factor when they arise. Two examples of this are the developments in New York and Washington.

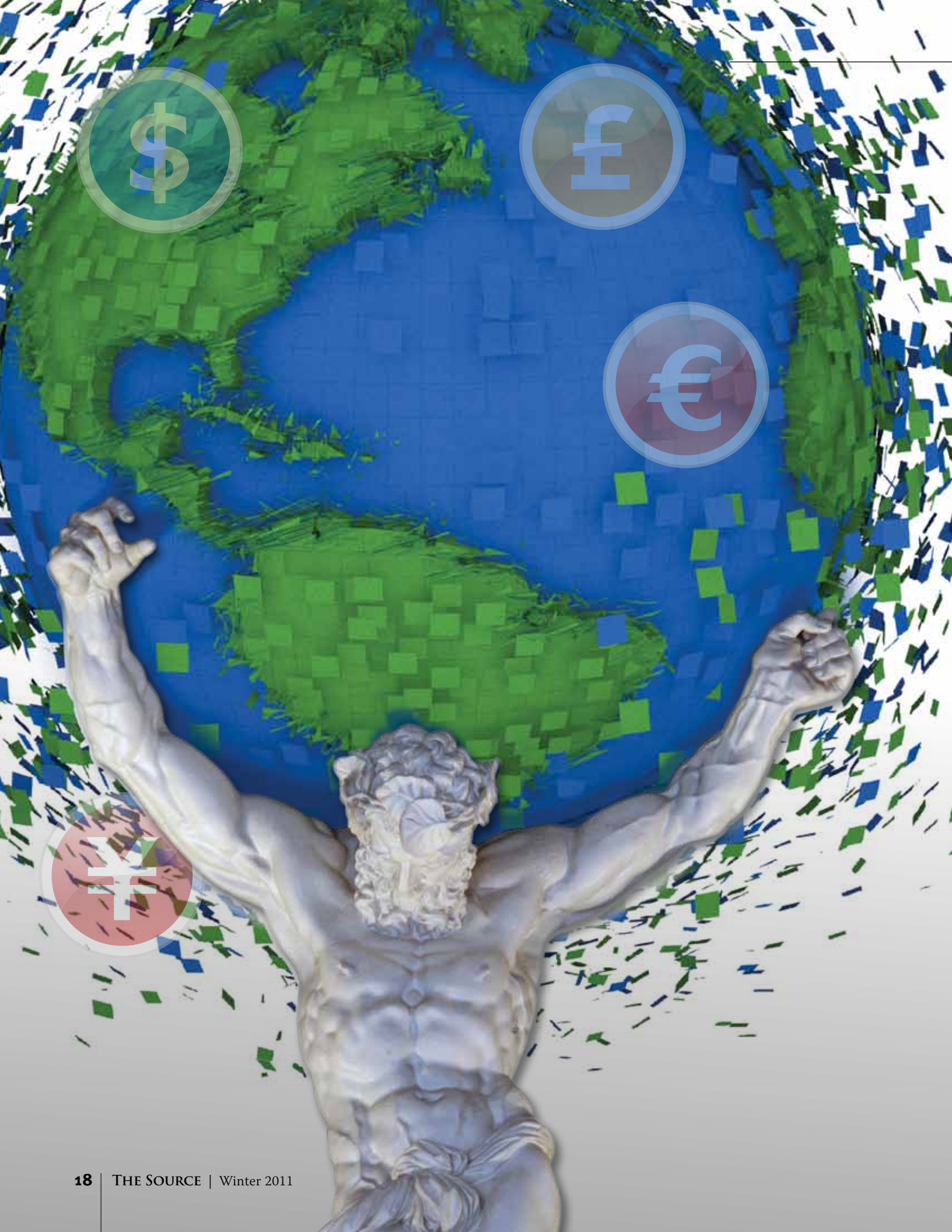
New York has announced it will switch the reimbursement for blood clotting factor to Average Acquisition Cost starting in November of 2011. The SPAC, working with other specialty pharmacies that provide clotting factors and the National Hemophilia Foundation, developed dispensing fee data points for New York Medicaid to consider when developing the dispensing fee that would be paid to specialty pharmacies that provide blood clotting factor to New York Medicaid recipients.

The State of Washington put out a request for external review of a policy that has not yet been formerly proposed according to state rulemaking procedures. The policy would require all individuals that receive health care through public assistance programs, including Medicaid, to have their blood clotting factor supplied by a hemophilia treatment center that is a covered entity under the federal 340B program.

The SPAC responded to the state's request for comment by pointing out we oppose this policy since it limits patient access to qualified blood clotting factor providers which is contrary to MASAC recommendations and federal rules that govern the 340B program. 

BILL SPEIR is PPTA's Director, State Affairs

The State Patient Access Coalition's goal is to educate decision makers in CMS, state Medicaid agencies, and state legislatures on the need for open access to all blood clotting factors.



with contributions from Charles Waller and Bill Speir

THE GLOBAL FINANCIAL CRISIS

and the Plasma Protein Patient Community

AS WE ENTER THE FOURTH YEAR OF THE GLOBAL FINANCIAL CRISIS (GFC), troubling indications are emerging that the community of patients needing plasma protein therapies (PPTs) are potentially very vulnerable to its effects. Authorities worldwide, faced with collapsing economies and mounting budget deficits, are taking the axe to all aspects of health care spending, and this threatens to engulf patient communities with rare diseases.

The cost-effectiveness pressures

In previous issues of The Source we have described the techniques of health technology assessment (HTA), particularly the use of decision analysis to analyze cost-effectiveness. We have described the role of the quality adjusted life year (QALY) in ranking the cost of health care interventions and assisting payers in ranking and prioritizing resources. These techniques have been developed for ranking interventions involving large populations of patients, given drugs which may need extensive clinical trials to demonstrate benefits. In many instances, these benefits may be marginal and the use of a tool to quantify their cost-effectiveness and allow ranking is understandable.

In the case of PPTs for rare diseases involving deficiencies in e.g. coagulation factors, immunoglobulin etc, the benefits for these treatments are entirely clear and indeed, in many countries clinical trials designed to prove their efficacy are considered unethical; for example, trials comparing prophylactic to on-demand treatment for hemophilia are not performed in Sweden as it is considered unacceptable to subject young children to the risk of bleeding. Therefore, the techniques of HTA are not necessarily applicable to PPTs and, sometimes, may impede access to treatment.

In particular, the use of the QALY as a tool in ranking resource allocation needs to be scrutinized critically. Two elements of the estimation of the QALY are, if applied dogmatically, particularly damaging to patients who need chronic treatment for rare disorders. These include the practice of *discounting benefits*, which leads to the minimization of benefits which accrue over long periods of time. An example of this is the prevention of joint bleeds in hemophilia,

AT A GLANCE

Some examples of Government actions

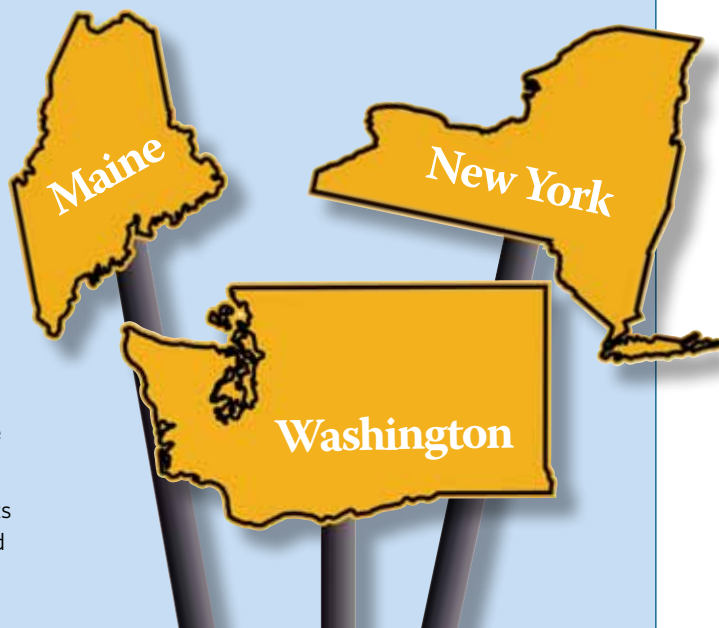
- › France introduces across the board reimbursement price cuts from January 2011 including clotting factors
- › Spain cut pharmaceutical spending by 2.4% in 2010 and introduces reference pricing system with 7.5% cuts to products not in the RPS.
- › Portugal introduces reference pricing system and a new automatic price review system has just been enacted.
- › Turkey introduces reference pricing and in February 2011 implemented a 9.5% discount on all pharmaceuticals.
- › The UK, in 2013, introduces new "value" based pricing in which clinical value in relation to costs and compared to the cheapest product in the same class.
- › Greece announces 30% discounts on generic pharmaceuticals.

STATES TO WATCH

MAINE is developing a request for proposals for a single specialty pharmacy to provide specialty pharmacy products to Medicaid recipients. Since blood clotting factor, IG and alpha-1 proteinase inhibitors are specialty pharmacy products, this could result in a change in pharmacy provider for individuals that use these products. It could also result in an interruption in the continuity of care for these individuals.

NEW YORK is proposing a change in their Medicaid reimbursement for clotting factor to one based on average acquisition cost. This change could result in a number of the specialty pharmacies leaving the market. This would disrupt care for individuals with bleeding disorders in New York.

WASHINGTON is considering a policy that would require recipients in their public health programs to have their clotting factor supplied by a 340B Hemophilia Treatment Center.



Lumping all the different immunoglobulin products into one group and then deciding to reimburse the cheapest is scientifically unjustifiable and medically dangerous for patients who react and tolerate different products differently.

which leads to greatly decreased medical costs when treatment is started early. Another example is the prevention of long term lung damage from immunoglobulin deficiency. All these treatments avoid long term disease sequelae and unquestionably decrease medical costs, yet this benefit is minimized, and often totally disappears, in conventional QALY estimates.


The other element which proves problematic in the calculation of the QALY is the use of *utility estimates*, which are derived from questioning individuals such as patients and the general public about their perceived benefits from interventions. For example, such individuals may be asked to rank their preference for prophylactic treatment for hemophilia compared to on-demand treatment. It is well understood that patients with chronic disorders tend to be cautious when faced with a change of treatment. This caution translates into a relatively modest benefit in the cost of a QALY for e.g., prophylaxis compared to on-demand treatment, despite the significant clinical benefits which are acquired.

These difficulties with using the QALY are partly the reason why cost-effectiveness analysis has not been applied to many plasma protein

deficiencies and treatments. However, with payers facing increasing costs in a deteriorating economic climate, some authorities are proposing to use these techniques to prioritize interventions and “ration” care. The danger to patients, exposed to the possibility of being “ranked” below what payers are willing to reimburse, is clear.

Similar or different – pressures on choice

Other cost-containment pressures are evident when payers attempt to group all therapies – e.g., all Factor VIII concentrates, all immunoglobulin products, all albumin solutions – into one category as “similar” or “generic” drugs. Payment is then restricted to the cheapest product in the group. The concept of similarity/genericity is well accepted in mainstream pharmaceutical drugs which are the products of the chemical synthesis of small molecules. This makes such drugs from different manufacturers very similar and comparable in a medical setting. PPTs are made from biological sources including human plasma and mammalian cell lines and are very complex molecules whose overall product composition is very dependent on the manufacturing processes which vary from producer to producer. Hence,



for example, lumping all the different immunoglobulin products into one group and then deciding to reimburse the cheapest is scientifically unjustifiable and medically dangerous for patients who react and tolerate different products differently. Deciding that PPTs such as albumin are classifiable as generic drugs, as was attempted in an economically challenged European country recently, fails to recognize the differences between products which may well affect patient care.

The way forward

So, as a historical figure once said “What is to be done?” It behooves all those responsible for the treatment of patients with rare disorders, including those dependent on PPTs, to advocate for very careful application of these measures described in this article. If measures such as cost-effectiveness and biosimilarity are really about the best use of the health care dollar, then recognition is needed for the special place of rare and chronic disorders. Otherwise patients will be harmed, and quite likely health care costs will increase. And that is something which everybody seeks to avoid. ☞

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EVERETT CROSLAND
is *PPTA's Manager,
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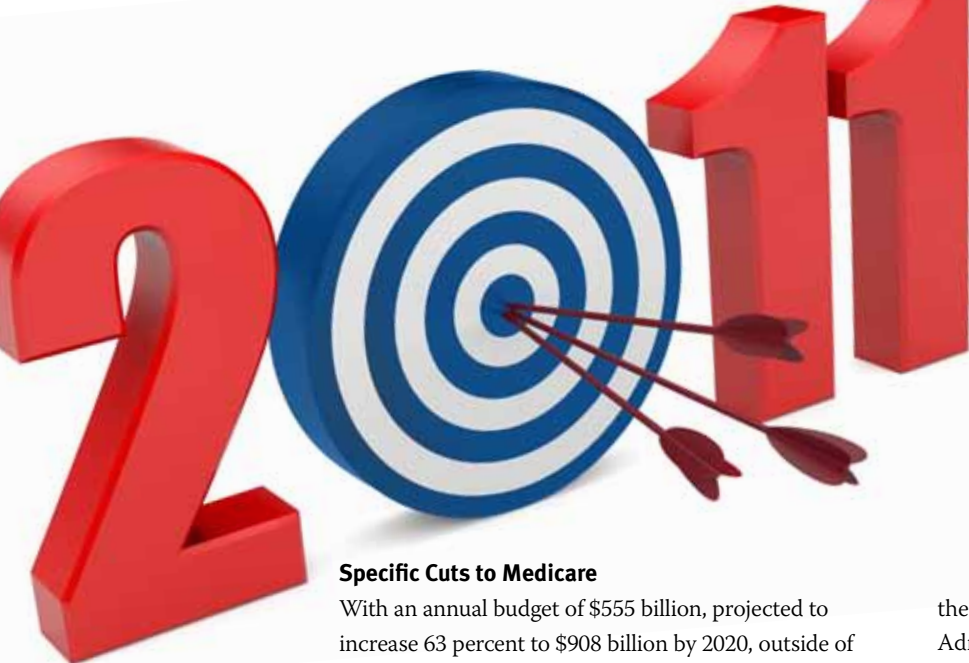
BILL SPEIR is *PPTA's
Director, State Affairs*

DEFICIT REDUCTION EFFORTS TARGET MEDICARE

BY EVERETT CROSLAND

ON NOVEMBER 21, 2011, the members of the Congressional Joint Select Committee on Deficit Reduction—commonly known as the Super Committee—announced that it had failed to reach an agreement to cut the federal deficit by the statutorily required minimum \$1.2 trillion. The Super Committee's failure triggers a statutory procedure known as sequestration, resulting in indiscriminate across-the-board cuts to the federal budget equaling \$1.2 trillion beginning January 1, 2013.

Largest among the many government programs facing across-the-board reductions are defense and Medicare, with cuts to Medicare capped at 2 percent of total Medicare spending. However, even with the 2 percent cap, Medicare sequestration would result in cuts of as much as \$123 billion over 10 years. Motivated by the blunt force of sequestration, it is likely that Congress will take up the charge of the failed Super Committee and seek targeted savings. While the magnitude and indiscriminate nature of sequestration is daunting, it pales in comparison to the likely prospect that Congress, unrestricted by statutory limits, will focus much of its deficit reduction attention on finding savings in Medicare.



Specific Cuts to Medicare

With an annual budget of \$555 billion, projected to increase 63 percent to \$908 billion by 2020, outside of defense spending, Medicare is indisputably the largest target for cost cutting. Recognizing this, industry leaders, consumer organization advocates, and other special interest groups have provided myriad proposals for solving America's deficit problems. Unfortunately, many of these proposals have included substantial cuts to Medicare.

Among these possible proposals a choice few stand out as especially concerning for the plasma protein therapeutic community for their potential to restrict patient access and undermine plasma protein therapy innovation, including

- › cutting average sales price prescription drug reimbursement in the physician office;
- › increasing cost-sharing on the part of Medicare beneficiaries;
- › shifting products from Medicare Part B to Medicare Part D; and
- › least costly alternative determinations.

In addition to cuts to Medicare, proposals to expand the deep discounts of the Health Resources and Services Administration (HRSA) 340B program to the inpatient setting and to aggressively cap long-term Medicaid spending are among other policies under discussion. The combination of cuts to Medicare, Medicaid limits, and the expansion to 340B would severely impact patient access to safe and effective treatment, interrupt patients' site of care, and increase beneficiary costs.

Medicare a Long-Term Target For Deficit Reduction

Despite the potential that the proposed cuts to Medicare will be detrimental to the health of patients and the healthcare system at large, it is likely that the Super Committee process was merely the first stage in a series of deficit reduction activities predominantly focused on Medicare.

With the failure of the Super Committee, the second stage of deficit reduction is under way as the law delaying cuts to physician reimbursement through adjustment to the sustainable growth rate (SGR) is set to expire at the end of 2011. In general, the Medicare SGR is a method to ensure

October 1, 2011

The spending caps passed as part of the BCA being to take effect with the start of Fiscal Year 2012

October 14, 2011

Deadline for Congressional Committees to submit deficit reduction recommendations to the Super Committee

December 23, 2011

Congress must vote to pass or deny the Super Committee's legislation. No amendments or filibuster are allowed

August 2, 2011

Creating the Super Committee, the BCA is passed in the 11th hour, narrowly avoiding government default

August 16, 2011

Members of the Super Committee are appointed

2011

November 23, 2011

Deadline for the Super Committee to provide Congress with legislation that reduces the deficit by \$1.2 trillion - **Failed**

Deficit Reduction



that the yearly increase in the expense per Medicare beneficiary does not exceed the growth in Gross Domestic Product (GDP).

Potential for Steep Cuts to Physician Payments


If Congress does not act to avoid a negative SGR adjustment to physician fees before January 1, 2012, physicians will face a 27.4 percent cut in their reimbursement for services they provide to Medicare patients. Given the potential for widespread patient access issues that would arise as a result of such a drastic cut, it is likely that Congress will attempt to resolve the issue by offsetting the cost of maintaining physician fees with cuts to other parts of Medicare. However, this is no easy task. It is projected that a ten year fix to physician reimbursement would cost as much \$289.7 billion over 10 years, making the more likely solution a one or two year fix, which would cost \$21 billion and \$38 billion respectively. Regardless, it is expected that Medicare could ultimately be targeted through SGR negotiations prior the end of the year.

While the first and second stages of the deficit reduction process present an alarming prospect for the plasma protein therapeutics community, policy experts are anticipating the process to play out in a third stage over the course of 2012.

Additional Deficit Reduction Factors

In the final two months of 2012, Congress will face the expiration of tax cuts enacted in 2001 and 2003 under President Bush, and extended in 2010 by President Obama. Realizing the bargaining opportunities that will be provided by the expiration of tax cuts and the impending sequestration, some members of Congress have suggested the replacement of the sequestration provisions with targeted deficit reduction measures, thus setting the stage for more negotiations that would likely take place at the end of the campaign season in November 2012.

Conclusion

PPTA recognizes that reducing the deficit is challenging and essential to stimulating job growth, however, indiscriminately cutting Medicare reimbursement cannot be the solution. Indeed, there are areas for reform and opportunities for savings in the Medicare program, but these reforms and savings must be realized through a measured and rational process that recognizes the unique needs of plasma protein therapy patients and the plasma protein industry. 

EVERETT CROSLAND is PPTA's Manager, Federal Affairs

2012

January 15, 2012

Final deadline for Super Committee legislation to pass Congress. Failure to pass legislation by this date will result in an automatic increase in the debt ceiling, and a triggering of sequestration to begin in 2013

January 31, 2012

The Super Committee is terminated

November 6, 2012

Election Day

January 2, 2013

If Super Committee legislation fails to pass, across-the-board cuts to government spending go into effect

2013

December 31, 2012

Bush Tax Cuts expire

Timeline

INSIDE PPTA

PPTA NEWS FROM
AROUND THE GLOBE

Save the Date!

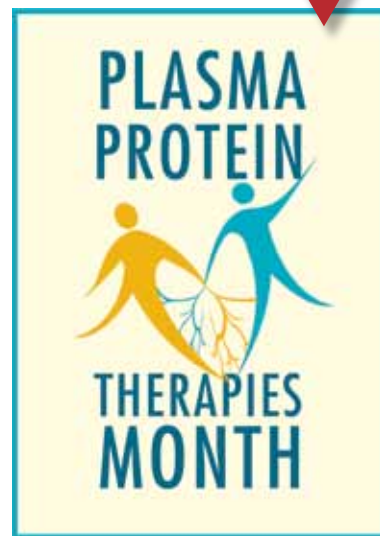
PPTA CELEBRATES 20TH ANNIVERSARY WITH EVENT IN WASHINGTON, D.C.



PPTA is planning a celebration of the Association's 20th anniversary recognizing decades of saving and improving lives on Wednesday, June 20, 2012 at The Pavilion at the Ronald Reagan Building in Washington, D.C. During the gala, a silent auction, dinner and special recognitions will be held. The event venue will be adjacent to the Plasma Protein Forum meeting site at the J.W. Marriott Hotel and will be held the evening prior to the Forum. Reservations, program sponsorships and more information will be available in early 2012 or contact PPTA to learn how you can make the event spectacular!

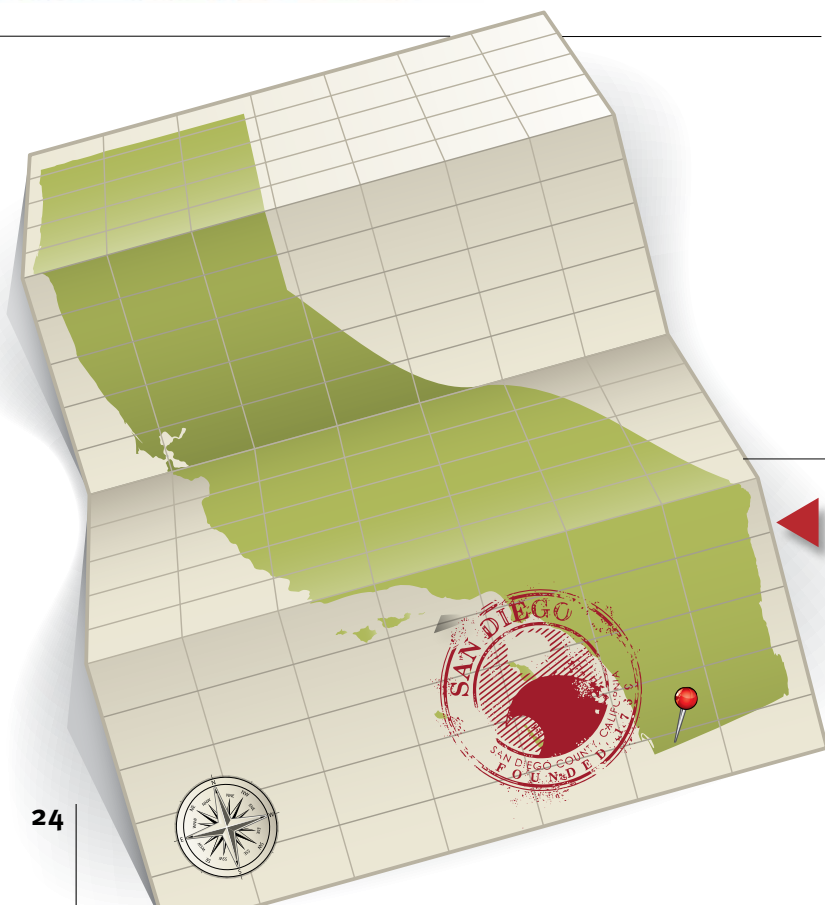
MEMBERS PARTICIPATE IN PLASMA PROTEIN THERAPIES MONTH EVENTS IN CALIFORNIA AND FLORIDA

Two PPTA members hosted activities at plasma collection centers in California and Florida as part of the Plasma Protein Therapies Month recognition, which raised awareness for the importance of plasma donation and for the therapies that treat rare, chronic conditions. Advanced BioSciences held an event at its Reseda, California center that recognized donors and patients, and the contribution the center makes to the community and lifesaving therapies. Invited guests included local officials and the event was covered by community newspapers. Grifols hosted three open houses at its centers in Atlantic Beach, Pensacola, and Tallahassee that included ribbon cutting ceremonies, facility tours and the opportunity to meet with patients and donors. Plasma Protein Therapies Month is a joint initiative of the State Affairs Steering Committee and the Source Industry Image and Credibility Campaign.



PPTA HOSTS SUCCESSFUL SOURCE BUSINESS FORUM IN SAN DIEGO, CALIFORNIA

PPTA held a well-attended and highly successful 2011 Source Business Forum in conjunction with the annual meeting of AABB, in San Diego, California, on Sunday, October 23, 2011. See page 6 for the Forum Report.



PPTA COMMENTS ON PDUFA REAUTHORIZATION

In response to the reopening of the comment period for the **Prescription Drug User Fee Act (PDUFA)**, PPTA submitted comments to the **U.S. Food and Drug Administration (FDA)** on October 28, 2011, that reiterated the Association's April 2010 public meeting testimony and its May 2010 written comments. PPTA also urged FDA to allow products to be orphan designated without a showing of clinical superiority in circumstances where the first to market product's orphan exclusivity has expired or was never granted. Eliminating the clinical superiority requirement in these limited circumstances would provide an opportunity for orphan designation for plasma protein therapies, which, as a result of the unique pharmacokinetics and pharmacodynamics exhibited per patient, are often challenged to show clinical superiority on a population-wide basis, and thus face a high, frequently insurmountable, barrier to orphan designation.

► **European Parliament program** PPTA European Health Policy Steering Committee (HPSC) met with several Members of the



European Parliament and health attachés from the Permanent Representation of Germany, Italy and Spain. The objective of these meetings was to establish a first contact with key stakeholders for the upcoming revision of several health-related European legislations. In fact, legislations such as the Clinical Trials Directive, the Pharmacovigilance Directive and the Transparency Directive, setting the framework for the reimbursement of pharmaceuticals in Europe, will be revised in the next 12 months by both the European Parliament and Member States.

► PPTA attended the 62nd meeting of the **World Health Organization (WHO)** Expert Committee on Biological Standardization (ECBS). The WHO Blood Regulators Network presented the draft "Assessment Criteria for National Blood Regulatory Systems" which was developed upon request from WHO and the International Conference of Drug Regulatory Authorities (ICDRA). The document aims at the development of an assessment tool to assist capacity building of national regulatory authorities for blood and blood products and to sustain development of the World Health Assembly (WHA) Resolution 63.12 on availability, quality and safety of blood products. The document should aid convergence of blood standards on a global level, however it is not meant as a harmonization tool. During the meeting the ECBS endorsed the proposals to establish the 1st WHO international standard for Hepatitis E Virus (HEV) Ribonucleic Acid (RNA) and to establish the 1st WHO international reference panel for Hepatitis E Virus genotypes. It was noted that in developing countries, HEV is the major cause of acute hepatitis, transmitted by the faecal oral route or contaminated drinking water.

► **PPTA Deutschland**, for the first time since its founding was requested to nominate a representative and a substitute for the National Advisory Committee "Blood". The PPTA Deutschland Board of Directors nominated Dr. Albrecht Gröner, CSL Behring, to be the

main representative and Dr. Matthias Germer, Biotest, to be the substitute. The committee has a long standing history and its votes have influenced the landscape for blood and plasma products and all related aspects, including voluntary and unpaid donations and look back procedures just to name two.

► **PPTA Netherlands met with the Ministry of Health** to share PPTA's view on the recently published ConQuaestor report.



The Ministry confirmed that the ConQuaestor report was the final step to prepare a response from the Dutch Minister of Health to the Parliament. According to the Ministry it is obvious that the current outcomes regarding the position of the local manufacturer needs to be evaluated and are subject of discussion in the Parliament. The impact of the report is not yet clear, though it is clear that changes in the current Law on Blood Supply may be the result of the ConQuaestor report.

► **The FIND-ID network**, supported by PPTA, hosted its first symposium at the 45th Workshop for Ear Nose Throat (ENT) specialists in Mannheim, Germany. 140 ENT experts attended the symposium entitled "Sinusitis - yet again? News about Immunodeficiencies!" The four presentations addressed one or more key symptoms that an ENT expert would frequently see in his daily life and which is a typical hint towards immunodeficiency. The feedback was enthusiastic and many physicians took the opportunity to visit the FIND-ID booth afterwards to get more information about



Primary Immunodeficiencies (PID) and FIND-ID itself. They confirmed what PID experts and industry have been stating for a long time, i.e. that a lot of patients are sent to ENT experts only after general practitioners and pediatricians are at the end of their wisdom with patients who have an above-average number of sinusitis or otitis each year, hence fulfilling one or more of the classical warning signs for PID. Encouraged by this success, FIND-ID will continue to focus on the education of experts in medical areas where traditionally un- or misdiagnosed patients are found.



INSIDE PPTA

PPTA RUNS ADS SUPPORTING LEGISLATION

During the first week in November, PPTA ran online ads on Politico.com and Roll Call.com in support of House and Senate legislation that would modify the orphan drug exclusion from the annual pharmaceutical fee

enacted as part of the Affordable Care Act last year. H.R. 2672 and S.1423 would exempt all therapies that are solely indicated by the Food and Drug Administration to treat one or more rare disease or condition. The ads urged members to cosponsor the bipartisan legislation to protect access to rare disease therapies.

PROTECT ACCESS TO RARE DISEASE THERAPIES

Congress must ensure that the needs of rare disease patient populations continue to be met and that incentives for orphan drug development continue to support bringing life-saving medicines to patients.

The Plasma Protein Therapeutics Association and its members urge you to co-sponsor the bipartisan H.R. 2672 and S. 1423, the "Preserving Access to Orphan Drugs Act of 2011"

Learn more at www.pptaglobal.org/rarediseasetherapies



GLOSSARY OF TERMS

ABRA American Blood Resources Association

ALBIOS Albumin Italian Outcome Sepsis Study

CBER Center for Biologics Evaluation and Research

CDRA Conference of Drug Regulatory Authorities

EMA European Medicines Agency

ENT Ear, Nose and Throat

FDA U.S. Food and Drug Administration

GFC Global Financial Crisis

HEV Hepatitis E Virus

HRSA Health Resources and Services Administration

HTA Health Technology Assessment

MBLD Mannan Binding Lectin Deficiency

MRSA Methicillin-resistant Staphylococcus aureus

PDUFA Prescription Drug User Fee Act

PID Primary Immune Deficiency

QALY Quality-adjusted life year

RNA Ribonucleic Acid

SAFE Saline versus Albumin Fluid Evaluation

SGR Sustainable growth rate

SPAC State Patient Access Coalition

WHO World Health Organization

STAFF

CATHY IZZI

How long have you served at PPTA?

I have been supporting industry and staff since 1982, with my humble beginnings in service of the American Blood Resources Association.

What do you focus on in your role as PPTA's Director, Member Services/Office Manager?

My role as metamorphosed into more of an internal operations function over the years. I administer the benefits, payroll, recruiting services and office administrative services, such as our document management system, training initiatives, office policy and procedures, office supplies, etc. My external member services role includes, in part, lead logistics coordinator for the Plasma Protein Forum, production of the Source Essentials quarterly e-newsletter, and working with Vice President, Josh Penrod in support of PPTA Source.

Tell us about your background.

I initially studied dance at the University of Maryland and was a member of small modern dance company in Bowie, Maryland. In early 1982, I had open heart surgery through the Heart Program at the National Heart, Lung and Blood Institute at the National Institutes of Health (NIH) in Bethesda. I mention this because, as I came to work at the Association, I was involved in a Blood Study relating to transfusion-associated hepatitis. Because of that and that NIH had samples of my blood and that of my blood donors in 1984; I was invited to participate in a study to evaluate "a new test which hopefully will detect carriers of the agent which causes AIDS." Just an interesting correlation and the rest is history.

I have a wonderfully supportive husband of 28 years, and a very talented and loving son of 21 years. My husband Greg and I, along with other family, have a jazz band which gives us a creative outlet.

What is your proudest professional achievement?

After nearly 30 years between ABRA and PPTA, it is difficult to pin point a specific achievement. I would have to say that simply I am most proud of my perseverance, patience and adaptability over the years.

What is most rewarding about working in this industry?

It's all about the people: The people we work with, the people who challenge us, and the people whose lives are improved and saved with the unique therapies our members produce. ☺



EVENTS

UPCOMING
CONFERENCES
& SYMPOSIUMS

2012

March 8 – 11	Second ASID Congress of the African Society for Immunodeficiencies <i>Hammamet, Tunisia</i>
March 13 – 14	International Plasma Protein Congress (IPPC) <i>Madrid, Spain</i>
March 20 – 23	32nd International Symposium on Intensive Care and Emergency Medicine <i>Brussels, Belgium</i>
June 16 – 20	European Academy of Allergy and Clinical Immunology Congress 2012 <i>Geneva, Switzerland</i>
June 21 – 22	Plasma Protein Forum <i>Washington, D.C., United States</i>
July 7 – 12	XXXII International Congress of the ISBT <i>Cancun, Mexico</i>
July 8 – 12	World Federation of Hemophilia, World Congress <i>Paris, France</i>
October 3 – 6	XV Biennial Meeting of the European Society for Immunodeficiencies (ESID) <i>Florence, Italy</i>
October 6 – 9	AABB Annual Meeting <i>Boston, Massachusetts</i>
October 13 – 17	The European Society of Intensive Care Medicine Annual Congress <i>Lisbon, Portugal</i>
October 26 – 28	European Hemophilia Consortium Conference, <i>Prague, Czech Republic</i>
November 8 – 10	National Hemophilia Foundation 64th Annual Meeting <i>Orlando, FL, United States</i>

THINKSTOCK



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Two new multi-dye tests with real-time virus discrimination

cobas® TaqScreen MPX Test, v2.0 (CE-IVD)*

Five critical viral targets detected:

- HIV-1 Group M
- HIV-1 Group O
- HIV-2
- HCV
- HBV
- Pool up to 96 donors
- Immediate HIV, HCV and HBV virus discrimination in a reactive donation

cobas® TaqScreen DPX Test**

Two viral targets detected:

- Parvovirus B19 (B19V) - quantitative
- HAV - qualitative
- Complete genotype coverage
- B19V genotypes 1, 2 and 3
- HAV genotypes I, II and III
- Pool up to 480 donors
- B19V and HAV performance meets current regulatory requirements for plasma for further manufacture

The most comprehensive NAT assay menu for blood and plasma screening

Testing is performed on the automated **cobas s** 201 system featuring fully integrated pooling and data management software to track from sample input to reactive pool resolution.

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<http://molecular.roche.com>

*Not available in the US

**The duplex test for B19V and HAV has been filed with the FDA under a Master File. It is available to US laboratories that meet specific FDA requirements.



cobas®

Life needs answers

FEWER PARTS, FASTER SWAP-OUT, LESS TESTING.



4.0 times the volume.

Multiply your productivity with the 4.0 m² Planova™ virus removal filter. We quadrupled the effective membrane size of our 1.0 m² filter without changing the length.

One 4.0 m² filter can replace four 1.0 m² filters:

- Simplify with fewer valves & joints needed in equipment
- Save operation time with quicker filter swap-out
- Cut costs by reducing frequency of integrity testing

From lab research to process scale, Planova 15N, 20N, and 35N filters give you validated scalability – 0.001 m², 0.01 m², 0.12 m², 0.3 m², 1.0 m², 4.0 m² – for efficient development and rapid time to market.



Planova 20N Filter line.

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