Decades of Safety Measures

Treating Rare Diseases with Plasma Protein Therapeutics

PPTA Leadership Interview: Gordon Naylor

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2 IN MY VIEW
Worldwide Economic Crisis May Impact Healthcare

4 It’s The Economy...
The promise to revamp the nation’s health care system is being sidetracked by economic woes.

6 PPTA LEADERSHIP INTERVIEW
Gordon Naylor

8 Decades of Safety Measures
Plasma Protein Therapies Start with Healthy Donors...

13 Treating Rare Diseases with
Plasma Protein Therapeutics

16 Hemophilia in the Philippines:
PROJECT SHARE:
Hope for the Forgotten: Part 2

20 Rare Plasma Related Disorders
Under the Spotlight in Brussels

22 Patient with Primary Immunodefiency Disease says
Thanks to Plasma Donors

25 PPTA France
Round Table Discussion

26 News
Plasma Protein News from around the World

28 MEET THE PPTA STAFF
Katarina Mlcuchova

29 Events
Upcoming Conferences & Symposiums
2009 IS STARTING OUT WITH ENORMOUS CHALLENGES. The whole world faces a critical financial situation where many governments are scrambling to formulate initiatives in an effort to stimulate the economy. Entire sectors have received financial support to avoid wipe-outs and further unemployment. This support is occurring worldwide as leaders in Europe, China, the U.S. and other countries enact legislation to repair their weak economies. The government interventions are meant to help the weak sectors. Nevertheless, many countries openly talk about recession and anticipate a tough year. Unemployment will increase and not everyone will have access to health insurance that covers their medical expenses.

Though I am very sympathetic to support initiatives that ultimately will benefit many, I am also aware of the very vulnerable position of the patient populations we serve. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us. Not a single individual can be blamed or should be penalized for having a genetic condition that puts his or her life in danger. As a society we have a responsibility to help the weakest among us.

Industry is constantly evolving and many new technologies are changing our world. Some of us aren’t old enough to remember life without a desktop PC or when carbon paper was used in typewriters. Newspapers were printed manually with lead type. Developing photographic film took days and faxing documents was rare. Undeniably, industries can only survive if there is enough investment to fund improvements and new technology.

With the enormous financial help in many countries, total government expenditures go up and create other budget pressures. If that is combined with budget shortfalls including reduced revenues because commodities tax-income is lower (oil prices dropping more than $100 per barrel in six months) then some countries may revise their healthcare priorities and reduce available funds to buy the therapies to help their patients. That would be very problematic because that would be unfair to those with chronic illnesses and rare diseases in our society who need help more than ever in these hard economic times.

PPTA has always made patient access to care one of its primary priorities. Patients in need of life-saving therapies are vulnerable and need to maintain an adequate supply of a full range of therapies, supported by reimbursement policies.

Our industry is unique. It produces life-saving therapies where in most cases human plasma is used as starting material. The collection of high quality and safe plasma from committed donors is costly and is a big factor in the manufacturing costs of the therapies. We cannot compromise on the safety and quality of any of our therapies as we all know.

The economy is a pressing problem for societies in general; but even more problematic for the consumers who rely upon our therapies. The member companies of PPTA will step up to the plate and do everything they can to help their patients maintain access to care.
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Seal with confidence... you can bank on it with SEBRA.
By Julie A. Birkofer

A new Administration brings many things to Washington and the American public. President Barack Obama’s widely touted campaign rhetoric was – “Change” and “Yes We Can.” Not even to the 100 day mark yet, President Obama’s promise to revamp the nation’s health care system and provide affordable health insurance coverage to all Americans is being sidetracked by economic woes. The President signed into law in early February an expansion of the State Children’s Health Insurance Program (SCHIP) that will extend coverage to an estimated 4 million more children. Since President Obama was sworn in, the major focus of his Administration has been on the economy and the passage of a two year, $800 billion stimulus package.

President Obama’s inner circle believe that health care reform will not only improve the lives of Americans; but it should also contribute to pulling the nation out of this economic crisis. One of the health priorities of the Obama Administration that is tied to the faltering economy is that there is a direct correlation between increases in the rolls of the uninsured and job loss. In order to address this there are three major provisions in a House stimulus package (which passed on a party line vote by 244-188). As of press time, the Senate is still debating the package.

1. **COBRA extension for older workers.** When Consolidated Omnibus Budget Reconciliation Act (COBRA) coverage runs out, older workers often have difficulty finding new health insurance because of preexisting conditions or steep premiums that make individual coverage unaffordable. The stimulus allows individuals that have been employed for 10 or more years and are over 55 years of age to continue their COBRA coverage until they become Medicare-eligible

2. **Partially subsidized COBRA coverage.** Employers are currently required to offer COBRA continuation health coverage for up to 18 months when you leave your job. But former employees must pay the entire cost of the insurance plus a 2 percent administrative fee. Many workers eligible for COBRA never take advantage of this health coverage through their former employer because the premium cost is too high. The American Recovery and Reinvestment Act says that workers who lost or lose their job between September 1, 2008 and December 31, 2009 as a result of the economic downturn would be eligible to receive a 65 percent subsidy towards their COBRA premium for up to 12 months. The Congressional Budget Office estimates that this package will help more than 8.5 million people keep health care coverage for themselves and their families.

3. **Job Creation.** The stimulus aims to create and maintain steady jobs. Sectors worth a look during your job search that would receive significant funds are: Construction, Education, Public Service and Green jobs such as the production of renewable energy.

The new Administration also has their sites set on a major overhaul of the Medicare Advantage program, which provides subsidies to private insurance plans to provide insurance coverage to beneficiaries. There has been very little discussion on how to pay for any new reforms or changes to the current health care delivery system. Another priority of the Obama Administration will be payment reform, “We’ve got to get away from payment based on volume to payment based on value.”

Julie A. Birkofer is PPTA’s Vice President, North America
PPTA Leadership Interview:

Gordon Naylor

By Joshua Penrod

As Executive Vice President; Plasma, Supply Chain and Information Systems, Gordon Naylor takes overall responsibility for plasma supply to CSL Behring. He is the Chairman of the Plasma Protein Therapeutics Association’s (PPTA) Source Board and sits on several industry committees.

Please tell us a little bit of background about yourself; where you grew up, education, industry experience, family, etc.

I grew up in rural Australia. My education was fully in the public system – I have an Engineering Degree, a Computer Science Diploma and an MBA.

I’ve worked for CSL for most of my career and have seen the company change enormously in that time. For the past 11 years, I’ve worked outside Australia in several countries as the company grew.

My family (I’m married with three children) have enjoyed the expatriate lifestyle – we like traveling and the adventures of exploring new cultures.

My first direct exposure to plasma collection was in 2001 when I managed the integration of ZLB Plasma into the CSL group and then took over leadership of the business in 2002. It’s an absolutely fascinating industry – I’ve learned an enormous amount from the many dedicated people that I’ve worked with over the years. There’s always another angle – a perspective that you haven’t thought of before.

What do you view as the role of plasma collection in the entire industry?

Clearly plasma collection (both source and recovered) is integral to the entire industry – to a large extent it’s the raw material that distinguishes this industry from other parts of the bio-therapeutic sector.

I spoke about this in June 2008 at the PPTA Plasma Protein Forum as well. Plasma, like whole blood and cellular components, is a very special raw material that carries with it important emotional and ethical issues in addition to its economic and material value. These add dimensions of complexity that the folks in our plasma collection operations must deal with every single day.

How would you describe the future of plasma collection? Is there room for further innovation?

Our industry (plasma collection) could be seen as mature. The core operational processes have been quite similar for many years, especially since the advent of automated plasmapheresis technology.
I prefer to think of the industry as stable, with both a history and a future of steady innovation. Recent examples include the introduction of new information technology, the creative use of modern building materials and increasingly sophisticated communication with current and prospective donors as part of the local communities in which we operate. In the latter case, the PPTA itself is playing an important role in the form of the Source Industry Image and Credibility Campaign.

How would you describe some threats and some opportunities for the plasma collection industry?

Our biggest challenge – and opportunity – is to keep moving. Our environment changes all the time – we have business dynamics, emerging therapies, shifts in the regulatory landscape etc – we need to pay attention and respond.

Our patients demand the highest standards of care with global access to safe products. A supply of high quality plasma is fundamental to industry success.

Unfortunately, well-intended government regulation can sometimes impede progress – or even lock us into practices that have been superseded or become redundant. Our best defense in the interests of patients is an active and well-considered adoption of best practice as these opportunities emerge.

How would you describe the role and scope of the Source Board of Directors?

I have the good fortune to work with a group of people who are highly engaged and committed to supporting the industry that they represent. Along with PPTA staff (led by Josh Penrod), we meet via conference call or face-to-face almost monthly (sometimes more frequently) to address current and strategic issues as they affect the industry.

The Source Board is unique within PPTA as it represents the interests of our independent members – collectors on both sides of the Atlantic who are not owned by global fractionators – in addition to the global collection interests of the global members.

How has PPTA changed since you joined the Board?

Since you became Chairman?

I have been privileged to serve on the Board for almost seven years. During that time, I think that the Source Division has become more closely integrated with the rest of PPTA – which is consistent with the trend of vertical integration in the industry, while maintaining a distinct and very focused identity.

We have become a little more formal in our management of membership and representation – I think that this has helped with transparency especially for the independent members.

Another shift is that I think that the Source Board is now more international in outlook, rather than focusing solely upon the United States.

All of these trends occurred under the leadership of Joe Rosen of Baxter, who I respect enormously. I think it’s too early to say whether my chairmanship has “moved the needle” – certainly we’re continuing the good work of the past years with a group that is highly engaged and committed to this industry.

What would you like the readers of The Source to know about the industry?

For the folks within plasma collection – your work is important and integral to delivering the medicines that are so important for our patients.

For our other readers – plasma collection has some unique challenges – we appreciate the support that the Source Division receives and welcome you to spend time with us where our therapies begin.

Joshua Penrod is PPTA’s Vice President, Source
Decades of Safe

By Prof. Albert Farrugia, Mary Gustafson and Ilka von Hoegen, Ph.D.

THE PLASMA PROTEIN THERAPEUTICS ASSOCIATION (PPTA) represents the plasma protein therapeutics industry, which manufactures plasma protein therapies by purifying them from human plasma and genetically engineered cell lines. These therapies are used to treat a number of diseases in which the naturally occurring plasma proteins are deficient or present in amounts lower than required by the body. These disorders include deficiencies of the blood clotting mechanism, leading to bleeding disorders such as hemophilia, or the lack of ability to synthesize antibodies, leading to chronic infections. In addition, healthy individuals such as travelers and pregnant women are protected from certain illnesses with the antibodies produced by the plasma protein therapy industry.

This article will explore the “Decades of Safety Measures” that the plasma protein therapeutics industry has put in place over the years to demonstrate its commitment to continuing safety. This industry is very different from traditional pharmaceuticals. Manufacturing plasma protein therapies that are infused or injected by patients results in the formulation of a unique class of biologics that are dissimilar from chemical compounds. PPTA member companies exemplify vigilance and innovation through the various safety measures that have been implemented over the decades. From, vein to vein – from the plasma donor to the patient – today's plasma protein therapies represent a compilation of state-of-the-art collection, viral inactivation and manufacturing protocols that have been fine tuned over the decades.

Blood component therapies and plasma protein therapies start with the same source material—blood and plasma donations from people who are willing to give of themselves to help others. It is important to make sure that donors of both blood and plasma are healthy to donate and that their donations do not harm the eventual recipients of therapies produced from their donations. To help ensure this goal, donors are selected by subjecting them to a series of steps to make certain that they are eligible to donate. Donors must meet certain criteria that include age, minimum weight and normal vital signs; the use of a health history questionnaire that screens for certain health conditions, diseases or behaviors that might indicate infections that would be transmissible to the recipients of therapies manufactured from their donated blood or plasma; and freedom from diseases transmissible by blood as determined by specific testing, eg., tests for hepatitis B and C and Human Immunodeficiency Virus (HIV).

For a potential donor of source plasma, used exclusively for the manufacture of plasma protein therapies, the donor is more closely monitored because the donation frequency and volumes collected exceed those for routine blood donation. Donors of source plasma have an initial and annual physical examination and are monitored for protein levels. In addition, PPTA maintains a voluntary standards program for collectors of source plasma, the International Quality Plasma Program (IQPP), which includes additional criteria and safeguards to ensure the health of donors and the safety of plasma protein therapies produced from source plasma. These standards include having donors be part of the community in which they donate, the qualification and testing of donors, and standards that cover the suitability of the collection facility in which they donate. All of these measures help ensure that plasma used in the manufacture of plasma protein therapies is of the highest quality.

In addition to ensuring the quality of donations, plasma for use in the manufacture of plasma protein therapies undergoes manufacturing processes beyond the selection of the donor and testing (the first and second legs of the tripod, see page 10). The manufacture of plasma protein therapies includes steps that further ensure the safety and purity of plasma protein therapies. Some of these steps are integral to the process of separating and purifying the proteins;
some are added specifically to eliminate pathogens that inadvertently may be in the plasma. These processes are effective for known and unknown pathogens.

Throughout this year, the Source magazine will focus on the safety of plasma protein therapies and the safety measures undertaken through the decades that work to ensure that patients receiving plasma protein therapies receive the highest quality therapies, free from inadvertent risk from pathogens.

**Plasma Protein Therapies Treat Rare, Chronic, Genetic Diseases**

The patients treated with these therapies manufactured from human plasma are generally severely ill or at considerable risk of life-threatening illness. These conditions are rare, and some of them are poorly understood, making these patients vulnerable, and the therapies life-saving.

As these therapies are necessarily derived from biological sources, including human plasma and animal cell-lines, the therapeutic plasma protein products are at risk of contamination by pathogens from the biological source material. These pathogens include viruses, bacteria and prions (abnormal proteins which can self-replicate and cause lethal diseases of the brain). Such contamination still constitutes a rare but observable risk for patients receiving blood transfusions. In addition, biologically-derived therapies such as blood are vulnerable to contamination by emerging pathogens which are still unknown but which can, nevertheless, cause disease. For example, over the past ten years, a virus, previously unknown in the United States but prevalent in Africa and the Middle East, entered the United States and ultimately, contaminated the whole blood supply. This virus, called West Nile Virus (WNV), caused illness, including some fatalities, in people given blood transfusions. On the other side of the Atlantic, some recipients of blood in the United Kingdom were infected with prions transmitting variant Creuzfeldt Jakob Disease (vCJD). In both these instances, the blood-borne infections were previously unknown, and infections were transmitted through blood transfusions before measures to minimize their risk were understood, developed and applied.

Why have the recipients of these therapies, when manufactured in the high-quality facilities of the PPTA member companies, not been infected with a single external pathogen for the past fifteen years? This has not been a fortuitous situation, but has been the result of the commitment, investments and dedication shown by the industry as it has hastened to apply safety measures as risks have become known, and to apply prudent, precautionary and preemptive measures to minimize the effect of unknown and emerging risks. These measures, which have been introduced ahead of and in excess of measures legally required by government agencies, are linked together through the so-called “Safety Tripod” underpinning the safety of products. The combined effect of selecting safe raw material for manufacture, testing this material to exclude contamination and treating this material during manufacture to eliminate pathogens, results in the production of safe, high-quality and efficacious plasma protein therapies.

**Measures Taken to Exclude High-Risk Donors**

Throughout the decades as the epidemiology of infectious pathogens became known, the industry introduced measures to exclude the entry of high-risk donors into the plasma pool used to manufacture plasma protein therapies. For example, in 1982-83 PPTA companies introduced selection measures to exclude donors at high risk of HIV-AIDS before these were mandated by authorities or implemented by the whole blood sector. Once the risk factors associated with other diseases were known, appropriate selection procedures were also introduced. The first plasma borne pathogen which could be tested, hepatitis B virus (HBV), was tested with methods of increasing sensitivity by the industry in the 1970’s, which also introduced testing for HIV and Hepatitis C virus as soon as these were available and ahead of government regulations. The testing for evidence of infections was strengthened with the introduction of nucleic acid testing (NAT) for the actual pathogens in the late 1990’s, again ahead of regulators and leading to considerably enhanced safety of the
manufacturing pool. The possibility that other viruses, such as human parvovirus B19, may cause illness in the recipients of plasma protein therapies, led the industry to introduce this test in 2000, again in the absence of government regulation and similar measures in the whole blood sector.

These measures depend on knowledge of the epidemiology of pathogens and the availability of tests to screen them out. The minimization of risk from unknown and emerging agents, where scientific knowledge has not yet come to the stage of understanding how pathogens are transmitted and tested, depends on the processes which the industry has developed to eliminate pathogens through manufacturing steps which are designed for this purpose. These measures started to become available in the early 1980’s and over the succeeding decade, they were rapidly adopted by the industry so that, by the early 1990’s, the risk of plasma protein therapies from the known blood borne pathogens was immeasurably low. The processes introduced are very robust so that the emergence of WNV did not affect plasma protein therapies. While no biologically-derived product can be declared risk free, the characteristics of pathogen elimination procedures gives us great confidence that future threats can also be avoided.

The products of the plasma protein therapeutics industry have a safety profile which exceeds that of most of the therapies of the other biological sectors and of the products of big pharma. This enviable position is the result of a commitment to product improvements and a state of constant vigilance to ensure currency in safety, quality and efficacy.

The PPTA Pathogen Safety Steering Committee (PSSC)

In 1994 the Virus Safety Working Group (VSWG (Later Pathogen Safety Steering Committee or PSSC) was established as the industry’s scientific expert committee to address virus safety issues of plasma protein therapies on a global basis. The members of this PPTA expert group on pathogen safety are all well seasoned scientists striving to remain accepted members within the scientific community, while at the same time ensuring that within their commercial environment pathogen safety of plasma protein therapies is always addressed according to state-of-the-art developments in technology.

Over the years since its foundation the PSSC has established itself as the globally accepted industry expert group on issues related to the safety of plasma protein therapies. PSSC is responsible for the identification and prioritization of key safety issues. The experts monitor, assess and comment on newly emerging pathogens and are responsible for advancing the scientific credibility of the industry. The most important task for PSSC is the liaison with authorities on scientific issues including the initiation and supervision of task forces dedicated to specific targets, such as WNV for example.

PSSC is recognized by the U.S. Food and Drug Administration (FDA) as a valuable contributor to the discussions within the Blood Products Advisory Committee (BPAC), the Transmissible Spongiform Encephalopathy (TSE) Advisory Committee (TSEAC) and other FDA expert groups. Within the European Union regulatory framework PSSC is the scientific expert group in discussions with the European Medicines Agency’s (EMEA) Biotechnology Working Party and their TSE expert group. Within the World Health Organization (WHO) PSSC contributed to their TSE and NAT standard committees.

PSSC has had many achievements and successes over the years of which the introduction of industry wide standards for HIV, Hepatitis B virus (HBV), Hepatitis C virus (HCV) and Parvovirus B19 have to be regarded as the most significant initiatives to raise the credibility of the industry and assuring patients, physicians and regulators about the margin of safety of these often life-saving medicines.

The Parvovirus B19 standard can serve as an example of how a proactive industry initiative can assure stakeholders and at the same time hold up unnecessary regulatory constrains. The Parvovirus B19 standard was introduced in 2000 in reaction to concern about its impact to the patient community, particularly to pregnant women. The standard set a cut-off limit for Parvovirus B19 in the plasma pool of 10^5 IU/ml and has proven its value since its introduction, by simultaneously avoiding unnecessary loss of the precious starting material, human plasma. It is very unfortunate that despite the abundance of available experience over the years U.S. regulators have recently decided to require a cut-off limit of 10^5 IU/ml. European regulators have refrained from considering a similar approach in the context of the revision of the Note for Guidance on plasma-derived medicinal products (CPMP/BWP/269/95).

Sanitization Agents are Introduced

At the height of the discussion pertaining to the possible transmission of the vCJD agent by plasma protein therapies, the TSE Task force mostly composed of PSSC members undertook a comprehensive investigation of the inactivation of a model TSE agent using Sodium Hydroxide NaOH, the most commonly used sanitization agent. The study, which was published in a peer reviewed journal after its finalization, investigated the influence of concentration, temperature and time on the effectiveness of TSE inactivation by
NaOH. This unique database of inactivation data was also intended to serve as a basis for additional company specific studies of combined cleaning and sanitization. The results were presented to U.S. and European regulators as well as at a number of international scientific events. Most importantly the study was helpful to prevent regulators to request segregation of production lines for European and U.S. plasma by demonstrating the effectiveness of cleaning and sanitization procedures. According to Dr. Harvey Alter, an infectious disease specialist at the National Institutes of Health in Bethesda, Md., solvent-detergent treatment of commercial plasma and its derivatives has established the principle that pathogen reduction of even a single blood clot component is enormously valuable and has simultaneously established the principle of preemptive pathogen reduction.1 "Universal solvent-detergent treatment has rendered the formula that the formerly, most risky of blood transfusion products, plasma and plasma derivatives, will now be the safest," says Dr. Alter. "As blood transfusionists scramble to find a way to stop West Nile virus from whole blood and platelets, how reassured the plasma industry must have been to know they already had this agent pre-emptively covered. Those same measures would protect against Dengue in plasma or any lipid-encapsulated agent that threatens the blood supply."

In the past years a number of emerging infectious diseases have challenged manufacturers of plasma protein therapies. In case of an Influenza A virus or H5N1 (bird flu) pandemic it can be expected that 10 to 30 percent of donors would be lost temporarily, which would mean a significant, although, temporary reduction in supply of these life-saving medicinal products. In view of this threat, which has fortunately not yet manifested itself, PPTA launched a Pandemic preparedness website (www.ppta.pandemic.info.us) to inform all involved parties about the newest developments regarding H5N1 virus.

**PPTA Sponsors Round Tables**

In order to establish a globally operating system to monitor and react to emerging infectious diseases, PPTA sponsored two Round Tables in 2004 and 2005 with participation from WHO, FDA, EMEA, patient groups and industry. The discussions at the Round Tables were considered as a helpful tool to shape the understanding of a more precautionary, but reasonable approach to deal with emerging pathogens, for example Severe Acute Respiratory System (SARS).

Another initiative that demonstrat-ed the benefit of a scientific approach beyond its purely scientific recognition is the industry-wide data collection of robustness studies with solvent detergent treatment. This data collection was initiated at a PDA Viral and TSE Safety conference in 2005, where regulators indicated their willingness to reduce the requirements for robustness studies with solvent detergent treatment if a comprehensive data base was available to them. The data base was greatly appreciated by regulators, although an additional caveat was added in that the data should be published in a peer-reviewed journal. The robustness of the data collection should be seen as the first step in an ongoing process, were industry-wide data bases could help to provide regulatory relief.

Over the years PSSC has demonstrated to regulators and stakeholders that the industry has responded vigorously and vigilantly to mitigate virus transmissions through plasma protein therapies. Regulators, scientists, consumer organizations, researchers and treaters have also made significant contributions over the decades towards improved safety. PSSC has established a proactive approach to respond to the challenges of pathogens following the precautionary principle, which is transparently discussed and developed with all involved parties. While in the beginning, PSSC actions were driven by immediate needs for action, PSSC today has increasingly taken the role of a group developing the scientific credibility with stakeholders and assuring the general public about the high margin of safety of the therapies PPTA members manufacture.
Most rare diseases lack visibility because of the small patient populations. For example, most people in the United States had no knowledge of Kawasaki’s syndrome, until it was revealed that actor John Travolta’s late son had contracted the disease as a toddler. The European Commission’s (EC) concerns for rare diseases, however, go beyond mainstream awareness. The EC is concerned that the lack of formal identification of rare diseases in terms of adequate coding and classification imposes impediments to treatment. For example, of the more than 5,000 rare diseases (it is estimated there are between 5,000 and 8,000 diseases that would qualify as “rare”), only 250 currently have an International Classification of Disease (10th version) code. In order to outline the necessary steps for an efficient policy addressing the issue of rare diseases in Europe, the EC adopted, on November 11, 2008, a Communication and a proposal for a Council Recommendation (http://ec.europa.eu/health/ph_threats/non_com/rare_10_en.htm) on rare diseases setting out an overall Community strategy to support Member States in diagnosing, treating and caring for the 36 million EU citizens with rare diseases.

European policymakers held two meetings in 2008 to specifically address poor diagnosis related to some of the above-mentioned diseases and patient access to plasma protein therapies to treat those diseases. Dr. Miroslav Mikolasik, who is a Member of the European Parliament (MEP), chaired a meeting that focused on “plasma protein in the treatment of rare diseases.” Several other MEPs, the European Commission, and representatives of patient, physician, and industry stakeholders attended this meeting. Among Dr. Mikolasik’s recommendations were that “recognition of [the] unique nature [of plasma protein therapies] needs to be taken into account in national health policies to ensure appropriate access to treatment for patients whose life and quality of life depend on these important therapies.”

Building on his previous recommendations, Mikolasik and fellow MEP Jorgo Chatzimarkakis held a second meeting that focused on “improving care for rare plasma disorders.”

The key outcome of this meeting was the issuance of a European Parliament Call for Action. The key outcome of this meeting was the issuance of a European Parliament Call for Action.
for Action is currently being circulated within the European Parliament and will propose key actions to improve care for rare disorders treated by plasma protein therapies.

In response to this issue, the European Commission’s Directory-General for Health and Consumer Protection’s Unit on Health Information has recently created a liaison group with representatives from organizations that represent patients that use plasma protein therapies for their treatment. This group, which is the first liaison created by the European Commission in the context of their rare disease proposals, will hopefully ensure the views and perspectives of patients are given proper consideration in any forthcoming EU legislative and policy actions affecting rare disorders treated by plasma protein therapies.

In contrast to these significant developments in Europe, recent legislative proposals in the United States could further hinder treatment options for patients suffering from rare, chronic, and debilitating diseases, disorders, and medical conditions. H.R. 1, The American Recovery and Reinvestment Act of 2009, which passed the United States House of Representatives on January 29, 2009, includes additional funding for comparative effectiveness research and provides incentives and penalties to spur the widespread adoption of interoperable health information technology systems. The legislation provides $1.1 billion in additional money for comparative effectiveness research -- $400 million to NIH, $400 million in discretionary spending for the Secretary of Health and Human Services (HHS), and $300 million for the Agency for Healthcare Research and Quality (AHRQ).

While there are several problems with the legislative language as currently drafted, an immediate threat to patient access is found in the congressional intent expressed in the report language accompanying the legislation.

In its report, the House Committee on Appropriations suggests that treatment cost should be a component of comparative effectiveness research, which would ultimately be used for coverage determinations. The committee explained that a chief goal of comparative effectiveness research is that “more expensive [drugs] will no longer be prescribed.” Although several federal agencies have been conducting comparative effectiveness research for more than a decade and despite the efforts of the Health Care Financing Administration (now the Centers for Medicare and Medicaid Services (CMS)), cost-effectiveness analysis has generally not been used for coverage policy decisions. Moreover, in January 2006, CMS expressly stated that it does not consider costs in reviewing treatments for the purpose of making National Coverage Determinations (NCDs). CMS contends, however, that its local contractors have the authority to consider cost effectiveness and adopt “least costly alternative policies” in setting local coverage determinations (LCDs).

Least costly alternative policies state that a Medicare contractor will not pay the additional cost of a more expensive item if a clinically comparable item costs less. This could be especially problematic for drugs used to treat rare diseases for medically supported off-label indications. According to Dr. Tracy Hampton, a physician-writer with the Journal of the American Medical Association approximately 90 percent of drug use to treat rare diseases is off-label. Utilization of IVIG supports this premise, because as much as 80 percent of it is off-label, according to a study by the HHS Assistant Secretary of Planning and Evaluation.

The House Appropriations Committee report language runs counter to the congressional intent of the initial funding given to AHRQ in the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) to conduct comparative effectiveness research. Specifically, the MMA conference report stated that CMS “may not use data from the research conducted to withhold coverage of a prescription drug, to mandate a national standard, or require a specific approach to quality measurement and reporting.” Likewise, the language accompanying legislation providing increased funding for comparative effectiveness research should at least

"Recognition of [the] unique nature [of plasma protein therapies] needs to be taken into account in national health policies to ensure appropriate access to treatment for patients whose life and quality of life depend on these important therapies.”

—Dr. Miroslav Mikoasik
prohibit CMS from considering cost in determining therapies that
treat rare diseases are not reasonable and necessary for treatment.
While the report language accompanying H.R. 1’s companion bill,
S. 336, is greatly improved, it still seems to give policymakers con-
siderable flexibility in how to use the research information.

Comparative effectiveness research can and will go beyond
doing head to head randomized clinical trials of two or more
drugs, for example. Because of the health IT language included
in H.R. 1 and S. 336, the move toward a rapid-learning health
system to fill the existing knowledge gaps on prescription drugs
is clearly progressing. Very likely, the $400 million in funds to be
transferred from AHRQ to NIH for comparative effectiveness
research will be used toward establishing a national rapid learning
system consisting of electronic health record databases.

According to Lynn Etheredge, an independent consultant working
on healthcare and social policy issues, the databases in such a system
“could be organized by enrolled populations (private health plans,
VA, Medicare, Medicaid), providers (multispecialty clinics, academic
health centers, specialist registries), conditions (disease registries),
technologies (drug safety and efficacy studies, outcomes research), geographic
areas (the Framingham Heart Study),
age cohorts (the National Children’s
Study), minority populations (human genome studies), and other
ways.” When the widespread adoption of health IT will become a
reality in the next decade, physicians will then be able to search these
various databases for similar patient profiles to instantly determine
which course of treatment could be most effective.

Without question, once implemented, a national rapid-learn-
ing network will ultimately result in a Medicare reimbursement
system “based on evidence-based protocols.” Data from compara-
tive clinical effectiveness research will be an important compo-
nent of this network. While the benefits of this information is
immeasurable, policymakers must be mindful that each patient
is unique so clinical responses do vary, even between what may
appear as similar patient profiles. Patients suffering from rare,
chronic, and debilitating diseases, disorders, and medical condi-
tions, such as those treated with plasma protein therapies, have
worked tirelessly with their physician to establish a treatment
plan best suited for their individual needs. Any new information
gleaned through studies and data analysis should not affect the
ability of these patients to access their preferred therapies that
have proven to be clinically effective in their treatment.

Johan Prevot is PPTA Europe’s Director, Health Policy and
John E. Greissing, Esq. is PPTA North America’s
Director, Federal Affairs

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Hemophilia in the Philippines

PROJECT SHARE: HOPE FOR THE FORGOTTEN

PART II
Lila looks at me with a dog’s limpid brown guileless eyes. A beautiful, healthy Boxer mix, resident guardian of the Manila Columban Fathers she lounges next to the ground floor stairs on the cool gray veined white marble floor. A rooster’s call rips the afternoon stillness, just as countless others call throughout the city, both day and night. Lila is lucky, she is a well-cared for pet, not a soon to be served on a dinner table delicacy. The rooster isn’t so lucky, he and countless others tied outside kitchen doors are destined for cockfights. The Philippines is a modern enigma: ambitious modernization programs and crude entertainment exist side by side. I wait for Laurie Kelley, lecturer and founder of Save One Life and Project Share, and Father Donald Kill, Philippine coordinator for Project Share and well loved humanitarian. We are leaving Manila today for Cebu City, to the South in Visayas. Joining us at the airport is Andrea Trinidad-Echavez, a journalist and advocate for People With Hemophilia, who along with her daughter Star, has von Willebrand Disease.

After take-off, Manila and its soot-blackened buildings, colorful jeepneys, child beggars, and stoic, long suffering people quickly recedes into the surrounding velvet green countryside. The events, experiences and lessons of the last week tumble through my mind as the Boeing 737 speeds between the rising columns of pearly cumulus at 7,000 feet in the humid tropical air. I cannot reconcile the comeliness and creativity of the Philippine people with the aloof, self-serving behavior of many of the elite who govern them. I grieve for the hemophiliacs and their families whom I have had the privilege of meeting and I applaud the individuals I have met who stand tall as caregivers and advisors.

The past week’s meetings with patients, support groups and hospital staff were the fertile strata for information sharing. I finally came closer to understanding the meaning of living with hemophilia in an almost total absence of clotting factor concentrates, prohibitively expensive tests, and sky high hospital costs. The stories were shocking because they would not happen in the United States or other prosperous countries. Jeffrey is a slender 25 year old psychology student with severe hemophilia. He wants to give succor to other hemophilia sufferers. He is the only surviving son of five sons; all born with hemophilia. He turns away with a haunted expression while his mother tells how his brothers died- intracranial bleeding, car accident, operation….. Elmer is a man on crutches with an infectious smile. He proudly tells

Above: Looking for Jeffrey’s four brothers interred in a Dumaguete cemetery in Manila
Left: Busy street in Manila, The Philippines
us how he is the first Filipino to survive an amputation procedure when his leg swellings due to bleeds became gangrenous. It is startling to realize that here is a person who is thankful for losing his leg and not bleeding to death. The clotting factor for his procedure was donated by Project Share……. At his home Randolf raises his trouser leg to show us the deep canyon in his thigh where a festering pseudo-tumor (growth-like swelling caused from repeated bleeds) was excised. Even though he is gainfully employed as an illustrator for the Philippine air force he admits to self pity and bitterness. He lives with pain from his condition and the ignorance and prejudice of others.…… Olive cradles her limp toddler son, his head wrapped with bandages. He has an intracranial bleed, and she is afraid he will die. Laurie’s Project Share has just sent vials of Factor IX. Olive’s baby will survive this episode…… In one patient meeting I watch a handsome young man with long hair that he wears covering the left side of his face. I think his hairstyle a teenage affectation, until I see that the eye underneath is milky white……. Nida sits with me after a meeting in Cebu with HAP-C (Hemophilia Association of the Philippines- Cebu) She is the mother of Arvin, who died last year aged 35 years. Waves of emotion- guilt, loss, loneliness, pain- wash over her face as her story unfolds. Arvin was admitted to a hospital with a shoulder bleed. He seemed to be stabilized, even though his temperature during the night rose to 104 degrees. The only factor VIII in the hospital pharmacy was given to a motorcycle accident victim, also with hemophilia. Nida’s son died the next day of an intracranial bleed.

We hold each other and weep uncontrollably. Repeated bleeds treated with ice and faith decay into ulcerous wounds. Uprooted rural families desperately claw a livelihood in the urban cities to be closer to what little medical help is available. Neglect, prejudice, ignorance, injustice, inhumanity… Why is the situation in the Philippines so dire?

There is no measurable government support for most bleeders...

The Philippines is classified as a medium income nation, but the unequal distribution of wealth belies that fact. According to the WHO improvement in the health status of Filipinos is unsatisfactory compared to other nations in the region. Infant
and maternal mortality is still high, chronic and degenerative diseases pose an increasing burden to the health care system. Half of the population earns an average income of just over $1,000 USD per month. Health insurance for an average wage earner would be 1/10 of that income. Public hospitals such as Philippine General Hospital in Manila do not charge for beds or care, but everything else must be paid for. Medicines and other supplies are so expensive that some critically ill median income individuals choose to stay home and die rather than impose mountains of bills on their loved ones.

The healthcare system is increasingly stressed and chronically underfunded. Local politicians sometimes delay pharmacies release of critical medication until election time to garner popular votes. Legislation to help the poor with healthcare is poorly applied and sometimes ignored with little or no prosecution of law breakers. President Gloria Macapagal-Arroyo’s well-publicized health care initiatives such as “medical tourism” attract wealthy foreigners and ignore indigent Filipinos. Corruption and cronyism are so rampant that a survey of Asian businessmen called the Philippines “the most corrupt nation in the region.” The United Nations Development Programme office in Manila estimated that “in 2004 about 13 percent of the government’s annual budget was lost to corruption.” The health care budget is abysmally inadequate and strains to correct the problems of undernourished children, parasitic diseases, malaria, and a host of diseases and conditions endemic to tropical southeast Asia as well as those that afflict western nations. Rare diseases like hemophilia are ignored. All blood assays for VWD must be shipped to labs in other countries. Although people (both men and women) with VWD should number in the thousands, only about 20 known cases have been diagnosed.

No factor concentrates are manufactured in the Philippines and the government does not buy any to distribute to the poor.

Who will help?

In the absence of government support hemotologists and other medical professionals and parents came together to start HAPLOS (Hemophilia Association of the Philippines, for Love and Service). The members of HAPLOS and sister organization HAP-C, headquartered in Cebu, strive to help Filipinos with bleeding disorders lead a more normal life. Sadly, for the majority of patients if cryo or fresh frozen plasma (FFP) are not available or too expensive, many bleeding episodes can only be controlled with ice, massage, and love. Laurie Kelley’s Project Share distributes donated factor concentrates to those who cannot afford them and sister organization Save One Life matches sponsors with patients in the Philippines and from around the world to help with tuition and basic necessities. Together, these organizations and individuals like Father Kill are valiantly making a difference.

Rose Noyes is a guest writer for The Source
WITH RARE DISEASES AT THE TOP OF THE EU INSTITUTIONS HEALTH POLICY AGENDA, European patients have high expectations of what the EU can do for them as we enter 2009. It is in this light that a meeting on “Improving Care for Rare Plasma Related Disorders” was held in the European Parliament on December 2, 2008, hosted by two prominent Members of the European Parliament (MEPs), Dr. Jorgo Chatzimarkakis (Liberals) and Dr. Miroslav Mikolasik (Christian Democrats). The debate was related to rare plasma related disorders, a group of rare chronic life-threatening disorders including amongst others hemophilia, primary immunodeficiencies and alpha-1 antitrypsin deficiencies, which are getting increasing attention from EU policy-makers.

Both host MEPs have been keen campaigners for patients with rare plasma disorders in the past. Miroslav Mikolasik had previously hosted a lunch in January 2008 at the European Parliament on the value of plasma protein therapies in the treatment of rare diseases (see The Source, Summer 2008) and has been a strong supporter of the hemophilia community. Both MEPs therefore were especially enthusiastic to support the patients affected by these life-threatening disorders by bringing the debate to the European Parliament at this time where rare diseases are foremost on the EU health agenda. The appeal of the subject was also proven by the attendance of other MEPs including Adamos Adamou and influential health MEP Frieda Brepoels as well as Nick Fahy, Head of Unit Health Information at the European Commission, a member of the permanent representation of the Czech Republic, which currently holds the EU Presidency and numerous other stakeholders includ-
Nick Fahy, European Commission, presented the European Commission’s proposal for a Council Recommendation on rare diseases with great flair and enthusiasm. The public consultation which preceded the publication of this dossier received a record number of responses, highlighting the number of stakeholders who were highly anticipating the proposed recommendations. Mr. Fahy stressed the priority areas identified in the Commission proposals and summarized the proposed courses of action that the EU and its Member States should take to improve the situation for patients as follows:

- Member States should all introduce a dedicated Rare Diseases Action Plan.
- Creating a coordination of existing reference networks, which valuably support patients with Rare Diseases and the healthcare professionals diagnosing and treating them.
- Provision of funding for Rare Disease research, supporting orphan drugs and backing non-governmental actions that support patients.

Representing the patients’ voice, Brian O’Mahony, Irish Haemophilia Society, spoke on behalf of patients affected by rare plasma disorders. Mr. O’Mahony who knows only too well the issues facing people with rare plasma disorders, as he has haemophilia himself, was pleased to inform participants that he, along with Mr. David Watters from the International Patient Organisation for Primary Immunodeficiencies (IPOPI) had met with Health Commissioner Androulla Vassiliou to agree on a consultation mechanism between patients affected by a plasma related disorder and the Commission to ensure their views are taken into account in EU Institutions related policies. Mr. O’Mahony identified the main priorities for patients with a rare plasma related disorder as being the need for good accurate data on these diseases to improve awareness of the conditions and diagnosis rates; Reference Centres for treatment of plasma protein disorders; consensus treatment protocols; patient involvement in policy process; access to high quality and effective plasma protein therapies and a clear differentiation between plasma and blood in EU legislation.

The Professor encouraged Member States to provide high levels of treatment by economic incentive; provision of treatment makes a benefit of € 60,000 per year of “full” quality of life as the person can work and contribute to society. Patient representatives vehemently backed these statements from the patient’s point of view.

Following on from the presentations, a lively debate took place which testified to the importance of the topic and the high level of interest from the attendants. The consensus was that the European Union has a significant role to play, echoing the statement of Commissioner Vassiliou that the area of Rare Diseases represents the field of healthcare where it is most obvious that the European institutions can add value.

One of the major outcomes of the meeting was an agreement to launch a “Call for Action” specifically outlining the necessary actions and steps forward for rare plasma related disorders identified during the meeting. Amongst the actions identified are the proposal to create an MEP Interest Group on Plasma Related Disorders and the need to consider carefully the intrinsic differences between plasma, plasma protein therapies and other biologicals such as blood derived products in upcoming EU legislation. The Call for Action is currently being circulated in the European Parliament by the host MEPs for endorsement by additional MEPs and will be shortly released officially.

“It is vital that the EU and its Member States listen to all relevant voices and I think that today’s meeting represents an excellent opportunity to do this”.

—Dr. Jorgo Chatzimarkakis in his opening statements
Chasing and Conquering Illness
As a child, Judy suffered from asthma and allergies, which extended into adulthood and were treated with inhaled steroids. In her 40s, she started getting more and more sinus infections that were increasingly difficult to treat. In 2004, her IgG (immunoglobulin G) levels were tested and were within the acceptable range. However, by February 2005, Judy became extremely sick with the flu, which was the beginning of a year-and-a-half long battle with debilitating illnesses that eventually led to two sinus surgeries, a battle with the antibiotic-resistant staph infection MRSA, the need for a PICC (percutaneous intravenous central catheter) line for intravenous antibiotics and a stint on short-term disability from her job as an information technology project technician. Finally the infections started to clear up in September 2006, but she continued to “feel really lousy.” It was at that point that Judy sought a second opinion for the sinus infections that had been plaguing her. She consulted a new Ear, Nose and Throat specialist at Johns Hopkins University Hospital in Baltimore, Maryland and, after a full work up of her blood revealed that her IgG levels were low and that she exhibited a poor immune response to vaccines, she was officially diagnosed with CVID.

Now Judy infuses 23 CCs (cubic centimeters) of immunoglobulin (Ig) twice a week on Sundays and Wednesdays at home, having been trained by a nurse practitioner at her immunologist practice. Once she started taking the therapy, “It was incredible.”

“I’m not who I was, before I got sick,” says Judy, describing her former ability to bounce back easily from “weekend warrior” activities like painting and assembling complicated closet organizers. “But, I’m able to hold a job and I’m able to be a mother to my daughter and a contributing member of society.”

“Thanks” Plasma Donors
Judy also is quick to thank plasma donors who make it possible for her to lead a healthier, productive life. “With this donation, this gift from people giving plasma – they are doing for me what I can’t do on my own,” she says. “This is a huge gift whether [plasma donors] realize it or not.” Judy says there is no way to describe how she felt before she started her Ig therapy, explaining how she would fall asleep during rock concerts she attended with her teenage daughter. “I was barely functioning as a parent and now I’m a mom again. I owe my life to the donors.”

Judy’s daughter Cara remembers that when her mother was sick, she had so much more responsibility than her teenage friends had to face. The honor student describes being overwhelmed by helping her mother manage the household. Judy adds that during the most severe time of her illnesses, she was “present but not accounted for” and is proud of Cara for, “doing the right thing.”

From Nursing to IT and Back
Having earned a Bachelor of Science degree in nursing and being licensed as...
a registered nurse, Judy worked in a critical care setting for many years, before taking a 10-year break from work to concentrate on raising her children—now ages 23 and 18. Rather than going back into nursing when she reentered the workforce, Judy pursued her interest and aptitude for computers and technology, working for the local school district. Knowing the school district’s budget was strained, Judy spent the spring of 2007 completing her nurse refresher course work, only to be laid off from her IT position just days after receiving her active Delaware nursing license in the mail. The timing was particularly fortunate, minimizing the amount of time Judy had to Cobra her state benefits to ensure that she was able to begin her twice weekly Ig treatments. Judy describes her Cobra benefits as, “the most important monthly payment I made.”

Today, she works as a nurse auditor at Thomas Jefferson University Hospital and has an even keener knowledge of the cost of healthcare in this country. Judy feels that she is “very, very lucky” to have good insurance coverage through the hospital.

**Giving Back**

More recently, as a volunteer for the Immune Deficiency Foundation (IDF), Judy has organized an IDF support group for individuals and family members who live with primary immunodeficiency diseases (PIDD). The group draws attendees from four states and had 17 individuals at the last meeting—the group’s second—which featured Dr. Stephen McGeady, an associate professor with Thomas Jefferson University who also treats pediatric patients with immunodeficiencies at A. I. duPont Children’s Hospital in Delaware. The first meeting helped attendees navigate the complicated maze of private and public insurance coverage for their chronic disease—a growing challenge for the community.

Judy describes health insurance coverage as the biggest and most stressful issue facing families coping with PIDD, “The product is available, but it has to do with reimbursement issues and getting insurance to pay.” Many individuals with chronic illnesses like primary immunodeficiency diseases risk hitting the maximum lifetime benefit of their health insurance. “The problem is that you are backed into a wall and need the medicine to survive or to have any quality of life, and there are no easy solutions out there right now if you don’t get insurance with a high lifetime cap or meet criteria for public assistance,” she says. The situation scares Judy because she will need the therapy forever. “I’m very, very lucky insurance has stood by me – so many people don’t have that.”

Kym H. Kilbourne is PPTA’s Assistant Director, North America Communications

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Opposite: Managing her primary immunodeficiency disease with Ig therapy gives Judy Kozulak the opportunity to enjoy a spring day with her daughter, Cara, riding Segues in New York’s Central Park.

Right: Judy takes on Tour de France champion Lance Armstrong at the famous Madam Tussauds wax museum in New York.
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**PPTA France Round Table Discussion Yields Significant Developments**

By Samantha Christey

**PPTA Staff and PPTA France,** organized in Paris its third Round Table discussion on November 21, 2008. The objective of these annual meetings is to create a unique platform for dialogue for French plasma protein community stakeholders to share information, discuss ways to improve access to care for patients with rare plasma protein deficiency disorders and to develop common actions.

The first Round Table was launched in 2006 and successfully gathered for the first time many participants including representatives from patient groups, physician groups, hospital pharmacists and industry.

The most recent Round Table was opened up to include additional patient group participants, including the Willebrand Centre of Reference of France. Attendees agreed on a common action which secured therapeutic continuity by ensuring the diversification of available plasma protein therapies in France.

Many of the debates focused on “patient access to care.” The discussions were, for the first time, moderated by a medical correspondent from Le Figaro, a Paris-based newspaper. The lively panel discussion focused on access to treatments, the non-substitution of plasma derived products, and on the need to improve patient access to plasma protein therapies by integrating projects included in the Rare Diseases National plan and in the revised Law of Bioethics.

Thanks to the 2008 Round Table, participants agreed on a set of common actions with a position statement that will encourage French authorities to implement the newly published “Circulaire on Immunoglobulins.” In addition, attendees agreed to adhere to common actions which will reflect the special case of plasma protein therapies in the Rare Diseases National Plan and in the revised Law of Bioethics which will be voted on in 2010.

PPTA staff will continue to organize Round Tables to bring forward the unique nature of plasma-derived therapies in order to place it squarely on the political agenda.

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Samantha Christey is PPTA Europe’s Manager, National Affairs
EUROPE

The European Commission adopted a Communication and a proposal for a Council Recommendation on rare diseases setting out an overall Community strategy to support European Union (EU) Member States in diagnosing, treating and caring for the 36 million EU citizens with rare diseases. For more information on the Communication, the proposal, the Impact Assessment, and various media resources related to the launch of the Communication, please go to this link: http://ec.europa.eu/health/ph_threats/non_com/rare_10_en.htm

The European Commission has adopted a package of proposed new measures for:

- Protecting the legal supply chain against counterfeited medicines;
- Information to patients on prescription-only medicinal products; and
- Strengthening pharmacovigilance to reduce the adverse effects of medicines.

The proposals and the accompanying communication on "Safe, Innovative and Accessible Medicines: a Renewed Vision for the Pharmaceutical Sector" has been published on the Commission’s website: http://ec.europa.eu/enterprise/pharmaceuticals/phamacos/pharmpack_en.htm. It is expected that the proposals will be presented to the European Parliament in early 2009, followed by consideration of the Health and Social Affairs Committee of the Council of Europe. If no significant concerns are raised during this procedure, the proposals will then be adopted. There will be no public consultation period.

A PPTA delegation met with the Director General (DG) of the Dutch Ministry of Health. PPTA expressed its serious concern about the distribution of official documents that suggested that therapies manufactured by PPTA members were less
safe than domestically manufactured therapies. The DG stated that this was not the intention, and that rewording would occur. It was also mentioned that the Ministry had questions about transparency with the business operations of Sanquin, and that an investigation to compare prices for blood components with surrounding countries has started.

The European Commission released a Public consultation on revisions of GMP Annex 14 on “Manufacture of medicinal products derived from human blood or plasma.” The annex has been revised in the light of Directive 2002/98/EC and relevant implementing directives that set standards of quality and safety for the collection, testing, processing, storage and distribution of human blood and blood components. These directives apply to the collection and testing of blood for all uses, including the manufacture of medicinal products.

In a meeting on the use of freeze-dried plasma (FDP) held at the National Institutes of Health, the U.S. Food and Drug Administration (FDA) signaled strict regulatory requirements for plasma preparations, which are in the pipeline from various sponsors including PPTA member companies. While this may affect access in the short term, it should give PPTA member companies that are manufacturing procoagulant and volume expanding therapies an opportunity to compete vigorously with non-evidence-based blood bank products, which appear to face substantial hurdles in acquiring regulatory approval.

The State Children’s Health Insurance Program (SCHIP) was approved by the House of Representatives with a 289-139 vote that would extend SCHIP for four-and-a-half years and expand coverage from 7 million to 11 million children at a projected cost of $33 billion. The huge expansion to the program will be paid for through a 61-cent tax on cigarettes and other tobacco products. It is important to note that an increase to the percentage in the Medicaid drug rebate program paid to states by manufacturers was not included as a budgetary offset in the bill. The Senate approved the bill on January 29, 2009.

The U.S. Food and Drug Administration (FDA) has announced a Draft Guidance for Industry on Standards for Securing the Drug Supply Chain - Standardized Numerical Identification for Prescription Drug Packages. In addition to commenting on this guidance, FDA is also requesting responses from interested stakeholders to questions posed in the Federal Register related to the draft guidance. The draft guidance can be found at the following link: http://www.fda.gov/oc/guidance/drugsupplychain.html. The Federal Register can be found at: http://edocket.access.gpo.gov/2009/pdf/E9-833.pdf.
My name is Katarina Mlcuchova. I am PPTA Europe's Assistant. I have worked for nearly a year at PPTA.

My position as an Assistant covers all sorts of activities within PPTA Europe. Mostly I take care of administrative tasks, such as incoming and outgoing mail, preparing briefing packages for meetings as well as meeting logistics. In addition to these responsibilities, I organize monthly staff meetings in the Brussels office. Finally, I assist my colleagues with PPTA’s Dutch Working Group by maintaining correspondence with the members of the group and attending their meetings.

Tell us about your background.
I come from Slovakia where I attended school until I was 18. In school, I studied economics, accounting and office management. After that, I went to London where I spent time studying English and performing all sorts of small jobs, such as working at a hotel in the reception area. At the age of 20, I moved to the Netherlands where I first worked as an au-pair, and studied the Dutch language. After this, I enrolled in college where I studied social work and psychology. For a short period of time I worked in the Netherlands as a social worker for people who are dealing with extensive drug abuse and/or psychological and psychiatric problems. As much as I have enjoyed working in this field, I followed my heart and ended up moving to Brussels with my boyfriend, who works at the European Commission. This eventually led me to a job at PPTA.

What is your proudest professional achievement?
Since this industry is whole new world to me, my goal last year was to try to familiarize myself with plasma protein therapies and to gather as much information available to help me to do my job more efficiently. Although I have only been with PPTA for nearly one year, I would say I am proud of the work my colleagues at PPTA do to make the lives of the patients easier and more enjoyable. It makes me very proud to be a part of it.

What is most rewarding about working in this industry?
Completing work to the satisfaction of my colleagues at the Association and knowing that I am making their professional lives a little bit less stressful and easy is rewarding. Of utmost importance is also working with the members of PPTA and other stakeholders and providing them with excellent service.
UPCOMING CONFERENCES & SYMPOSIUMS

2009

**March 23–25**
21st Annual EuroMeeting of the Drug Information Association
*Berlin, Germany*

**March 24–27**
29th International Symposium on Intensive Care and Emergency Medicine (ISICEM)
*Brussels, Belgium*

**May 18–22**
VIII Latin-American Meeting in Hematology, Immunology and Transfusion Medicine
*Havana, Cuba*

**May 28–30**
57th Annual Congress of the Japan Society of Transfusion Medicine and Cell Therapy
*Saitama, Japan*

**June 3–4**
PPTA Plasma Protein Forum
*Washington, D.C., USA*

**June 4–7**
14th Congress of the European Hematology Association
*Berlin, Germany*

**July 11–17**
XXII Congress of the International Society on Thrombosis and Haemostasis (ISTH)
*Boston, MA, USA*

**September 15–18**
42nd Annual Meeting of the German Society for Transfusion Medicine and Immunohaematology (DGTI)
*Rostock, Germany*

**September 24–25**
Sixth Global Forum on the Safety and Supply of Treatment Products for Bleeding Disorders
*Montreal, Canada*

**October 24–27**
AABB Annual Meeting
*New Orleans, USA*

**October 25**
PPTA Source Business Forum
*New Orleans, USA*
PPTA members only

**October 29–31**
61st Annual Meeting of the National Hemophilia Foundation (NFH)
*San Francisco, CA*

2010

**May 22–25**
56th Annual Meeting of the Scientific and Standardization Committee of the ISTH
*Cairo, Egypt*

**June 15–16**
Plasma Protein Forum
*Reston, VA, USA*

**June 26–July 1**
XXXIst International Congress of the ISBT
*Berlin, Germany*

**July 10–14**
Hemophilia World Congress
*Buenos Aires, Argentina*

**September 11–13**
Annual General Meeting of the European Hemophilia Consortium
*Vilnius, Lithuania*

**October 7–10**
XIVth Meeting of the European Society for Immunodeficiencies
*Istanbul, Turkey*

**October 9–12**
AABB Annual Meeting
*Orlando, USA*

**October 10**
Source Business Forum
*Orlando, USA*
PPTA members only
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