THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SUMMER 2008

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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 of its members.*

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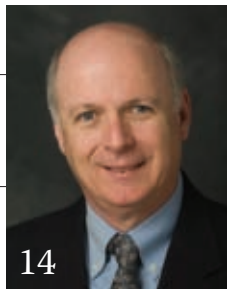
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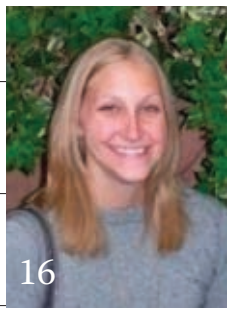
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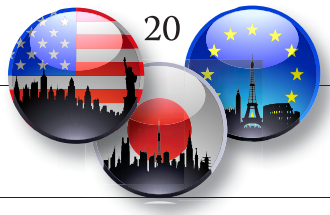
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In My View

Let's Level the Playing Field


Jan M. Bult
PPTA President

IN THIS COLUMN, I WANT TO EMPHASIZE the need for a level playing field. In several countries, our Association members are facing constant challenges by competitors who are operating under different rules. Don't get me wrong, strong competition is now and will be the main driver for innovation, and PPTA supports that. But isn't it reasonable to expect that the same rules should be applied to all? Consider this example from my home country, The Netherlands.

In 1998, the country decided that the entire organization for blood collection and manufacturing in The Netherlands was to be revised. The new approach was (and still is):

- One responsible organization (Sanquin) for blood collection (22 blood banks)
- Oversight and responsibility by the Ministry of Health (MOH)
- Annual budget to be approved by the MOH and submitted to Parliament

This clearly demonstrates that the Ministry of Health is directly involved in this created monopoly, and its sanction recently was confirmed in the published review of the law (*wet inzake Bloedvoorziening*). This direct involvement of the Ministry has created challenges, especially when the manufacturer opts for maximum safety, and the MOH for optimal safety. It must be said that Sanquin is operating for maximum safety, demonstrating its commitment to patients to provide safe products.

Sanquin is responsible for the collection of blood and the provision of cellular components to the hospitals in The Netherlands. Sanquin also owns a fractionation plant in Amsterdam, and has two-thirds ownership of the Central Department for Fractionation of the Red Cross (DCF/CAF) fractionation plant in Brussels. Recently the LFB, a French, state-owned company specializing in plasma-derived therapies, acquired a 25 percent interest in DCF/CAF as well. From a business standpoint, all operations contribute to the financial results and must be reflected in the budget. This budget needs to be approved by the MOH and subsequently submitted to the Parliament.

Since PPTA is not actively involved in blood collection, I will not comment specifically on this business arrangement, except to say that there is a serious potential for unfair competition. I am not stating that it happens, but it sure can. Every manufacturer has to buy the starting material, plasma, at a cost. Through the allocation of costs over the various activities, the price of plasma can be kept at a low level. If that is the case, then the costs of manufacturing can be kept down, and products can be offered at a lower price. A price comparison is one of the ways to find out how the prices of cellular components compare to surrounding countries, and how the costs of final products compare to competitors.

This is not just theory; there is an indicator for what I am describing. In today's market, Sanquin offers 1 gram of IVIG for about 28 euros. The current market price for 1 liter of plasma exceeds $150 U.S. dollars, or 100 euros. If one assumes that there are 4 grams of IG per liter, it is easy to see that the costs per gram already are 25 euros, and the manufacturing has not even started. Though Sanquin is obliged to be not-for-profit, this does not mean that it can afford to lose money. It is acceptable that Sanquin is acting in the market like all other private manufacturers. There is nothing wrong with that, except it should be done with the same rules. For that reason, transparency is required.

To make things more complicated, we need to have a look at Belgium. All manufacturers are obliged to pay annual taxes based on their sales. DCF/CAF and Sanquin have argued that they cannot afford to pay these taxes, and there even is a law that states that companies that are using plasma from voluntary, uncompensated donors are exempted from paying these taxes. Of course PPTA has challenged this discriminatory law, and it seems that a correction is on its way. But for me, the question is: How can the Dutch Ministry of Health (and Parliament) approve a budget for a monopoly organization that refuses to pay its taxes in a neighboring country? This is wrong.

Now is the time to discuss whether it is time for the privatization of fractionation centers owned by national blood collection organizations. There are examples in the world where this has already happened. One good approach would be to ensure that these companies adhere to the same rules that we have to, eliminate potential cross subsidies and compete in the marketplace to stimulate innovation. That is what I call a level playing field. ●

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IPPC 2008 Brings Impressive Cadre of Industry Stakeholders to Warsaw

By Kym Kilbourne

Dr. Karl Freese, European Commission

One of the most rousing quotes of the well-attended two-day International Plasma Protein Congress came from Dr. Karl Freese with the European Commission who said, "***we look at health care policy in terms of an investment in the health and well being of the patient, not in terms of health care cost***."

For many of the 280-plus delegates, who traveled from 31 countries to attend this year's Congress in Warsaw, Poland, the spirit of Dr. Freese's remarks were an important, refreshing take away of the meeting.

The 2008 IPPC featured panels of government, industry and academic experts discussing topics including self-sufficiency, European regulatory policy, quality, plasma availability, contract fractionation, health care policy and new indications with delegates from around the world.

The Congress opened to a standing-room-only crowd during the keynote session where Dr. Norman Relkin an associate professor of Clinical Neurology and Neuroscience at the Weill Cornell Medical College in New York and PPTA Global Board Chairman, Larry Guiheen of Baxter BioScience discussed the use of immunoglobulins in the treatment of Alzheimer's disease and the state of the plasma protein therapeutics industry respectively.

Dr. Relkin's explained the results of research evaluating the use of immune globulin (IG) in the treatment of Alzheimer's, a disease that is the fourth leading cause of death and the primary cause of dementia in individuals over the age of 60, saying, "it may be an effective way of treating one of our most devastating diseases of our time."

In addition to conducting two clinical trials, the second of which he described as

Dr. George Schreiber, WESTAT; Mr. Josh Penrod, PPTA Source; Mr. Martin Bezdekovsky, Fenwal; Mag. Rudolf E. Meixner, Humanplasma Austria.

achieving dramatic results in patients in as little as six months of treatment, Dr. Relkin described the third phase of the study that will evaluate 360 people for 18 months.

In his remarks, Mr. Guiheen discussed the continuing strength of the plasma protein therapeutics industry stating that, "The overall goal is to provide patients with essential therapies so they can continue to lead healthier, productive and fulfilling lives."

He described challenges familiar to the delegates in terms of reimbursement for high-impact therapies, and explained that the focus of PPTA continues to be on providing quality therapies, which start with the donor and the collection centers; innovation; and patient access.

Mr. Guiheen also underscored the need to address restrictive government policies in countries such as China and other discriminatory practices that prevent patients from getting the therapies they need. Citing the lack of treatment for individuals with hemophilia worldwide, he highlighted the global opportunity and that there is a role for both plasma-derived and recombinant therapies in treating patients.

A session on self-sufficiency led to discussion of contract or toll fractionation. PPTA president Jan M. Bult presented the challenges to entering the market. He described the industry's position to ensure that there is transparent decision-making, that the opportunity be given to all manufacturers, and that there is mutual regulatory recognition, underscoring that the focus in today's world should be on global self-sufficiency in the best interest of public health.

Graciously stepping in as a last-minute panelist, Mark Skinner, president of the World Federation of Hemophilia (WFH), echoed earlier speakers by stating that demand around the world for therapies is growing and that plasma-derived and recombinant therapies are both important to meet the demands and fulfill supply needs, reinforcing the need for countries to have a national plan. He praised the "robust safety record [of plasma-derived therapies] over the last 20 years." When looking at investments in fractionation plants around the world, Mr. Skinner stressed that they be evaluated based on, "what is the best,

Dr. Norman Relkin, Weill Cornell Medical College, New York, presented information to IPPC delegates on the use of IG in the treatment of Alzheimer's disease.

Dr. Anneliese Hilger, Paul-Ehrlich-Institut; Dr. Mirella Calcinai, Kedrion S.p.A.; Dr. Reiner Laske, CSL Behring; and Dr. Johannes Blümel, EMEA.

KERRY FATULA, executive director with the Western Pennsylvania Chapter of the National Hemophilia Federation, described her experience as a mother of three sons with hemophilia A and the efforts in Pennsylvania to affect changes in the state preferred drug list for factor therapies in the wake of Pennsylvania's need to trim costs. Describing the massive campaign in the state that included newspaper coverage and an outpouring of support, advocates were able to turn the tide and defeat the restrictive PDL policy. Ms. Fatula's knowledge is personal; each of her children is on a different therapy, and all have developed inhibitors, demonstrating why it is so important that every product be accessible to all patients.

long-term solution for the patient." Mr. Skinner stated that few countries can really achieve self-sufficiency with plasma-derived therapies only, that it, "creates rationing for care in the country," and that it is "foolish and counter-productive to prohibit imports to meet patient needs." Recognizing that politics and economics perhaps collide on this issue, he stressed that countries need to have, "political and economic will to make changes over time."

IPPC delegates were able to hear from representatives of the primary immune deficiency, alpha-1 antitrypsin and hemophilia patient communities during a session on established products and new therapies. Dr. Philip Wood, an immunologist with St. James Hospital in Leeds, United Kingdom, discussed why early diagnosis and treatment is so critical in increasing life expectancy of primary immune deficiency patients. His study of immune globulin (IG) therapy concluded that it is effective in prolonging life, improving the quality of life, and reducing infection. He noted that his studies show that not only is IG therapy cost effective, home SCIG may be more cost-effective than hospital administered IVIG.

Dr. Adam Wanner, Scientific Director for the Alpha-1 Foundation, provided details on alpha-1 antitrypsin deficiency (AATD) and common COPD (chronic obstructive pulmonary disease), making note of the prevalence with which the disease is under diagnosed in the U.S.; discussing how the disease causes lung destruction; and describing a model for new therapeutic targets in common COPD. Dr. Wanner presented information on how rare disease research has an impact on the treatment of COPD, which affects 250 million people in the U.S., stating that it has facilitated research, that new discoveries can be extrapolated into COPD at large, and that new therapeutic indications for both AATD and common COPD patients are likely to emerge as a result.

Presenting on new developments with the uses of albumin for therapeutic treatment, Dr. Nathan Davies, with the Institute of Hepatology at University College London, noted that albumin is damaged or decreased in patients with liver disease, a growing problem according to reports on hospital admissions data he cited. The potential use of therapeutic albumin in patients with cirrhosis is unique to albumin and the need for patient groups to advocate for albumin was discussed.

A Congress packed with sessions on regulatory policies in Europe, quality, and availability of plasma for further fractionation allowed delegates to hear first hand from numerous impressive panels of industry, medical and government experts on scientific and technical subjects, including specifics of variations, emerging and existing pathogens, good manufacturing practices, ICH guidelines, plasma collection developments and epidemiology. Given the breadth and depth of the topics presented, a summary of each panel will not be covered; however delegates can access the presentations of all of the 2008 IPPC panelists on the conference website: www.ippc2008.com.

The success of the 2008 IPPC, in terms of participation among key industry stakeholders and the level of expertise shared among the assembled panels, establishes it among the most well-attended and respected plasma protein meetings in Europe, creating a forum for conducting business; forging new collaborations; reacquainting with friends and colleagues; and focusing on the primary issues of the day—striving to secure patient access to therapies; achievements in product quality and safety; improvements in global regulatory harmonization; advocacy to affect change in health care policy; and a continued strong and thriving industry focused on delivering the best possible therapies to patients around the world. ●

Kym Kilbourne *is PPTA's Communications Manager.*

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PPUG Meeting Highlights Value of Collaboration

By Kym Kilbourne

Representatives from seven European patient organizations were joined by industry members and PPTA staff for a spirited discussion of key issues facing consumers. The Plasma Protein Users Group (PPUG) met on Monday, March 3 prior to the opening of the International Plasma Protein Congress (IPPC) in Warsaw, Poland and talked over European Union health policy, industry updates, the World Health Organization's Essential Medicines list and PPUG's strategy moving forward.

PPUG members kicked off the meeting, each having an opportunity to share information with their colleagues on their organization's agendas for the year and significant goals and successes and raised questions and issues with each other that produced lively discussion among attendees.

Plasma collection

Charles Waller, PPTA vice president, Europe, provided an industry update highlighting strides in patient access and explaining how plasma collection in 2007 has increased year over year by 20 percent for the last two years. He added that he expects to see new European Union member states coming into the plasma collection industry. One of the more heavily debated issues among the group was a discussion of the International Federation of Blood Donor Organization's (IFBDO) letter to the European Commission regarding its ill-informed, factually incorrect and potentially offensive stance on compensating plasma donors, specifically with respect to supplying plasma-derived therapies in France. Several PPUG members sent letters to the European Commission refuting the IFBDOs claims.

Johan Prévot, PPTA assistant director of Public Affairs, Europe, discussed the impact of the use of compensated vs. non-compensated donors on market access and that the IFBDO, which is reported to have very few members, has fueled EC discussion on this subject once again. He noted that the issue is political rather than legal, primarily in France and also directly affects patient access. Discussion continued that decisions need to be made based on science and safety, not perception. Members of PPUG further

PPUG members, PPTA staff and industry representatives met in Warsaw to discuss key issues of plasma protein users in Europe.

debated identifying a physician to bring expertise and experience into the argument in favor of patient access, and discussed leveraging their collective influence to produce a joint letter to the EC on this subject, specifically citing, among other things, EMEA's stance that plasmapheresis is safe.

European Union initiatives

PPTA staff also discussed key elements of the 2007 and 2008 European Union (EU) dossiers. Particularly highlighting the meeting on rare diseases in January with Members of the European Parliament (see full story page 12), Mr. Waller expressed that working together is yielding results and achieving traction in political discussion. He reported that the EU is continuing to have more influence in national decision-making. "Coming together is having an impact and the European Commission is much more conscious of the diseases treated with plasma protein therapies," Mr. Waller said.

New manufacturers

The group talked about encouraging new fractionation, but stressed the need to ensure that forays into manufacturing are conducted with a sustained approach. Several examples of state- or government-owned fractionation facilities that were built, but that were never able to produce therapies were discussed. The message that nations should not start these activities without a plan for sustainability was made clear by stakeholders, to ensure that valuable resources are not wasted.

In addition, PPTA consultants from Rhode Public Policy presented information concerning European Health Policy Developments, providing an overview of current policies including the medical device directive, donor compensation, rare diseases, patient information and organ donation and transplantation.

Continued collaboration

The open lines of communication in Europe regarding rare diseases give the industry and patient organizations a major opportunity to move forward. The subject is on the forefront of the minds of the European Commission, which seems poised to act on rare diseases, and represents an excellent opportunity to enter the debate and have some official communication and documentation. PPUG and PPTA will continue to meet and work together on common issues to support greater patient access throughout Europe. ●

Kym Kilbourne *is PPTA's Communications Manager.*

PARTICIPATING PPUG ORGANIZATIONS

World Federation of Hemophilia

International Patient Organisation for Primary Immunodeficiencies (IPOPI)

Irish Haemophilia Society

International Patient Organisation for C1 Inhibitor Deficiencies (HAEI)

GBS/CIDP Foundation International

Alpha Europe

ITP Support Organization

Prof. José-Luis Valverde Honored with Hilfenhaus Award

By Johan Prévot

PROF. JOSÉ-LUIS VALVERDE was honored with the prestigious Hilfenhaus Award at the 2008 International Plasma Protein Congress (IPPC) in Warsaw, Poland, for his outstanding contribution in the European Union's political arena.

Prof. Valverde's efforts to ensure patient access to plasma protein therapies across the European Union (EU) and to increase awareness among policymakers regarding the unique nature and value of these therapies are greatly appreciated by the plasma protein community as a whole.

A member of the European Parliament from 1987 to 1999, Prof. Valverde then became the European Parliament representative on the European Medicines Agency (EMEA) Management Board from 2000 to 2007. Prof. Valverde also is the Editor-in-Chief of the International Journal of Pharmaceuticals Policy and Law, an extremely well received publication at EU level. Throughout his career, Prof. Valverde has been tremendously active in the fields of blood and plasma-derived therapies, and soon became an expert in the subject at the European Parliament.

His views on issues such as self-sufficiency, the need for compensated plasma donations to sustain access for patients, and the important differences between labile blood products and plasma proteins have helped to optimize access to these high-value therapies. Additionally, in 2006 Prof. Valverde published *Blood, Plasma and Plasma Proteins: A Unique Contribution to Modern Healthcare*, and more recently another book focusing on immunodeficiencies. Throughout his political and academic career, Prof. Valverde has been a strong contributor to improving access to plasma protein therapies.

His outstanding efforts were not only recognized with the award itself, but equally through the distinction of being the first politician to receive this prestigious prize.

The Hilfenhaus Award is named for the late Dr. Joachim Hilfenhaus, who was among other things, a dedicated and effective Chairman of PPTA's Viral Safety Working Group. Annually at the IPPC, PPTA presents the Hilfenhaus Award to an individual who the Europe Board recognizes as having made an outstanding contribution to patient access to safe plasma protein therapies. ●

Johan Prévot *is PPTA's assistant director, Public Affairs, Europe.*

Previous Honorees

Prof. Dr. Inger Nielsen: Stockholm

Dr. Florian Horraud: Pasteur Institute, Paris

Prof. Wolfgang Schramm: University Hospital, Munich

Prof. Hans-Hermann Brackman: Haemophilia Center, Bonn

Dr. Helen Chapel: University of Oxford

Dr. Bruce Evatt: Centers for Disease Control, Atlanta

Dr. Wolfhart Kreuz: Johann Wolfgang von Goethe University, Frankfurt amMain

Prof. Vicente Arroyo: Hospital Clinic, University of Barcelona

Prof. Reinhold Schmidt: Hannover Medical School, Germany

Charles Waller, vice president PPTA Europe, Prof. José-Luis Valverde, and Dr. Giovanni Rinaldi, Chairman of PPTA's European Board of Directors at the 2008 IPPC reception in Warsaw, Poland.

PPTA Informs European Parliament Members

By Johan Prévot

The Plasma Protein Users Group (PPUG) met with Members of the European Parliament (MEPs) at the European Parliament in Brussels, Belgium to discuss plasma proteins in the treatment of rare diseases. Hosted by Miroslav Mikolasik (EPP/ED, Slovakia), the lunch meeting was attended by a number of influential MEPs and represented the culmination of work by PPTA and PPUG to engage key European policymakers on important patient and industry issues and establish a useful, long-term dialogue.

Dr. Mikolasik opened the meeting by outlining the European Union's (EU) main responsibilities in the area of plasma protein therapies and rare diseases as a whole. He stated that the European Parliament should encourage Member States to increase levels of diagnosis and treatment, and that closing the gap in standards between Western and Eastern Europe is imperative. Mr. Mikolasik also emphasized the importance of the Commission's proposal on rare diseases, to come later in 2008.

Representing PPUG, David Watters, executive director, IPOPI described a number of plasma protein disorders and discussed how individuals who cope with them are affected in their daily life. Mr. Watters stressed the life-saving nature the available treatments, which allow the majority of those suffering from plasma protein disorders to live happy and productive lives. He highlighted the following critical issues for members of the PPUG:

- Poor diagnosis rates
- Access to therapy & cost benefit factor
- Inequality of diagnosis and treatment in EU Member States
- Awareness and education

During the meeting, PPTA discussed how plasma protein therapies are developed, manufactured and used to save and improve the lives of patients with rare and chronic disorders. PPTA highlighted a number of positive actions for patients and the industry are being taken at the EU level in the areas of patient information, health services, rare diseases and telemedicine. PPTA outlined that further EU actions should be initiated to optimize access to plasma protein therapies, improve treatment levels and tackle disparities in EU Member States, increase awareness, develop patient registries, establish networks of reference centers and recognize the unique nature of plasma protein therapies in national healthcare policies.

Antoni Montserrat of DG SANCO (Health Information Unit) presented the European Commission's viewpoint on rare diseases and outlined a summary of previous EU actions intended to address rare diseases, such as the European Commission Taskforce on Rare Diseases. Mr. Montserrat recognized the need for a Commission Communication on rare diseases, and announced that it will be published in May 2008, following the review of responses received in the open consultation. He previewed some the proposals that DG SANCO want to make such as ensuring that Member States have national rare diseases action plans, creating an EU Working Group of rare diseases to advise the World Health Organization, developing national/regional centres of reference, and establishing EU reference networks.

Antoni Montserrat, Milan Gal'a, Manuela Ripa, Johan Prévot, Dr. Miroslav Mikolasik, Karl Petrovsky, David Watters, Rüdiger Gatermann and Dr. Peter Pustoslemsek.

GIORGIO FOCHESATO / ISTOCK

Following the formal presentations, an interesting and informative dialogue took place, in which all of the MEPs present demonstrated their engagement with the topic. Special emphasis was placed on discussing the upcoming Rare Diseases proposals with Mr. Montserrat, who confirmed that this will be published in May of this year. Adamos Adamou (GUE/NGL, Cyprus) stated that he wants to see what the actual impact of the proposals will be, and expressed doubt over whether previous Commission actions had made any difference. Mr. Montserrat responded that he was convinced that the EU has contributed positively in the past, and will continue to do so. He said that while initiatives on rare diseases may not make the newspapers, patients feel that their day-to-day lives have been improved by a number of EU actions. The consensus was, though, that the Commission must continue to add value to Member State actions and that the Parliament and patient communities would be monitoring them to make sure that they do. Additionally, it was highlighted that plasma protein therapies were interdependent and should be seen as one major public health tool in the treatment of numerous plasma protein disorders which taken as a whole are well over the rare diseases threshold (1:2000 p.) as defined by the European Union.

To close the meeting, Dr. Miroslav Mikolasik concluded with five action points that the EU should recognize:

1. Diagnosis must be improved.
2. Better and more equal treatment is needed.
3. There is a need to reflect the special case of plasma protein therapies in national health care policies.
4. DG SANCO proposals on rare diseases signal 2008 as a vital year for rare diseases.
5. National rare diseases action plans are vital to success in tackling rare diseases.

A number of MEPs, such as Frédérique Ries (ALDE, Belgium), expressed a clear interest in collaborating with PPTA and PPUG further in the future—one key goal that was achieved during the meeting. A conclusions document, endorsed by Dr. Mikolasik, has been distributed to all MEPs who were invited, including those who were not able to attend, to provide a lasting record of the important meeting.

Contact Johan Prévot for more detailed information about the outcomes of the meeting at +32-2-705-5811 or johan@pptaglobal.eu. ●

Johan Prévot *is PPTA's assistant director, Public Affairs, Europe.*

PPTA Leadership

By Kara Flynn

As president of U.S. BioPharmaceuticals for Baxter BioScience (a business of Baxter International), Lawrence P. Guiheen is responsible for sales and marketing of plasma-derived and recombinant biotherapeutics in North America. Mr. Guiheen has been a senior member of the Baxter BioScience management team for more than 10 years, setting the global strategy for the biopharmaceutical business.

Mr. Guiheen joined Baxter Healthcare Corp. in 1978 as a field sales representative in the New York/New Jersey area with responsibility for plasma therapeutics and blood and plasma collection devices. He quickly rose in the organization, holding positions in marketing, sales, and operations in several of Baxter's business units, including: bone marrow and stem cell collection; processing technologies used in the treatment of oncology; diabetes insulin pumps; and homecare services.

In 1995, Mr. Guiheen was promoted to Vice President of Global Marketing for the Fenwal division, gaining worldwide responsibilities for blood, platelet and plasma collection technologies. From this position, he was promoted to Vice President of Marketing for the North America Biotech business, which included the Hyland, Fenwal and Immunotherapy business units. With the Immuno acquisition by Baxter in 1997, Mr. Guiheen was then promoted to President, Hyland Immuno North America.

Mr. Guiheen currently is on the board of the California Healthcare Institute (CHI) and also is the Chairman, Global Board of Directors of the Plasma Protein Therapeutics Association (PPTA). Mr. Guiheen holds a bachelor of science degree in Business Administration from Rutgers University.

What are some of the priorities, challenges and opportunities for patient access to care globally?

In the United States, Canada and Europe, we need to maintain continued access to plasma and recombinant protein therapeutics. Even in these countries, there is the opportunity to reach patients who could benefit from our therapies who have yet to be diagnosed. Patients with primary immune deficiency still are taking five-to-seven years to be diagnosed, and it has been reported that only 50 percent of potential patients have been diagnosed. In Europe, there are dramatic variations in treatment across the countries.

In the developing world, this is more complicated. The right environment for free trade needs to be established. Currently, there are a number of barriers. We need to look at the backbone of our industry, which is the quality of plasma protein therapies, and begin working closely with different regulatory agencies around the world to obtain appropriate safety standards and achieve patient access. Our goal is to ensure that patients receive quality care and access to quality products.

What do you see as the most important task that you will have as PPTA's Chairman?

The reputation of the industry that has been built over the last two decades has created a foundation of quality and safety. This quality starts with the plasma donor. From the start, we focus on donor selection and testing, followed by advanced viral inactivation technologies to protect the patients using our therapies. We want to build on what we already have established and continue to make improvements in terms of donation collection and fractionation.

There is a continued focus around the world on the high costs of health care. The therapies we provide are high-impact therapies offering enormous value in terms of quality of life. Many medicines available today prevent serious health consequences. For example, a patient using a therapy that reduces cholesterol that could lead to a heart attack is using a preventative medicine. On the other hand, plasma protein therapies absolutely have to be taken by patients in order for them to maintain normal and productive lives. We need to work to get this message of the high-impact of our therapies to policymakers in the governments. The fear is that these critical therapies might get included with those less impactful medicines.

Global expansion into countries, where barriers currently exist, such as Japan or China is also important. Right now, the self sufficiency policy of Japan

Interview

"Our goal is to ensure that patients receive quality care and access to quality products."

and Article 49 in China are restricting the availability of therapies to patients who desperately need them.

What are some of the objectives for PPTA's Global Board of Directors this year?

Focus on our regional boards is especially important. In North America and Europe right now, there is an onslaught of issues. In Europe, access and availability of high-quality plasma and the increasing demand for immunoglobulins and other plasma proteins are ongoing challenges. As a Board, we need to carry the message that quality and safety are our primary objectives in the plasma protein therapeutics industry. We focus on quality standards based on fact, not history and assumptions. We need to continue to ensure that this industry continues to improve. Access to care is paramount. We need to remain focused on better diagnosis and assure that patients can get the right treatment for their condition. Reimbursement for these therapies is obviously critical to patient access.

How do you see the future of the plasma industry evolving?

The industry continues to strengthen and grow. I have talked earlier about better diagnosis and assuring access to therapies. Growth will continue to be a driver for this industry. It is still reported that less than 50 percent of the people worldwide with hemophilia receive adequate treatment. The same statistics are true for immune deficiencies and alpha-1 antitrypsin deficiency. So there is plenty of opportunity to grow. The strengthening of the industry has created a growth in investment. Investments in research and development focused on product improvements, as well as new indications for our current therapies and new therapies, will continue.

Investments in plasma collection and expansion of fractionation capacity are being reported to meet the growing demand for our therapies. The long-term view of the industry is robust. We have high-value/high-impact therapies that will continue to grow in demand both in the existing markets and new markets around the world. ●

Kara Flynn *is PPTA's director of Global Communications.*

Larry Guiheen, Baxter BioScience, is Chairman of the PPTA Global Board of Directors. He will focus this year on patient access to therapies worldwide and on quality and safety of plasma protein therapies.

Shelly Mattson

A Young Adult View on Bleeding Disorders

By Kara Flynn

Shelly Mattson shares her experiences with von Willebrand disease with young adults through the National Hemophilia Foundation's National Youth Leadership Council. She is a featured speaker at this year's Plasma Protein Forum in Washington, D.C. in June.

UNTIL SHELLY MATTSON VISITED CAMP BOLD EAGLE in Michigan, through a program organized by the Hemophilia Foundation of Michigan, she didn't feel that she had a way to connect with other young people with hereditary bleeding disorders.

Although the Milwaukee, Wisconsin native's mother and sister have von Willebrand disease, an inherited disorder that affects the blood's ability to clot properly, as Mattson does, she never really actively communicated with others and felt that her family was reluctant to face up to their condition. She has spent the last several years of her life attempting to reach out to others who might need a way to communicate with people in similar circumstances, through her involvement with the National Youth Leadership Council (NYLC), a program of the National Hemophilia Foundation.

Youth leader

NYLC was created to foster the development of young adult leaders ages 18 to 25. NYLC liaisons, such as Mattson, work to implement educational activities among youth affected by bleeding disorders within their local communities and become involved with their local chapters in various ways, including putting together fun activities, field trips and educational programs, or serving as role models for young people in the hemophilia community by providing guidance and support when needed.

For Mattson, who is a public relations major at the University of Wisconsin, La Crosse, the program has provided an opportunity to travel around the country and share her personal experiences with other young adults. "I got involved with NYLC at the Camp Bold Eagle initially, where I learned about the National Hemophilia Foundation's scholarship program, which is organized through the Council, and felt there was a way that I could serve as an ambassador and

communicate with other young people," she said.

It's a tough schedule to maintain, even for someone who doesn't have her condition. "I'm a pretty organized person and have always made it a point to speak to all of my professors at college and let them know about my involvement in NYLC, as well as my personal health issues. They have always been incredibly supportive, and I try to be very pro-active about doing my homework, even if it's accomplished on the road in some cases."

Story teller

Mattson says she is a big people person and enjoys public speaking and networking. Of particular interest, is her work on the advocacy front, where she enjoys speaking with legislators on both the federal and state level about the importance of access to critical therapies for those with bleeding disorders and educating this audience about rare diseases, such as hemophilia. Last year, she interned at the World Federation of Hemophilia in Montreal, Canada and learned how hemophilia is affecting people on a global level.

In June, Mattson will be one of the keynote speakers at PPTA's Plasma Protein Forum, which will be held at the Washington Marriott in Washington, D.C. In her remarks, Mattson hopes to discuss the importance of youth involvement. "We are a generation that has not been faced with some of the issues that impacted persons with bleeding disorders in the past," she says, speaking of the period in the late 1970s to the mid-1980s when many people with hemophilia contracted HIV. "People of my generation are reluctant to get involved. They feel that there is no need to think beyond the present, and what they are missing is that others around the world do not have the standard of care that we do in the U.S."

Advocate for teens

According to Mattson, the need for involvement is why she also gives time to a new program sponsored by Hemophilia Innovations, Transition Ignition, an all-day workshop for teens, who are transitioning from high school to college. In this program, speakers like Mattson attempt to promote a comfort level with hemophilia among teens; work to increase their understanding of the need to take responsibility for their own care; build parental opportunities for guidance; and teach and practice skills that help teens and parents manage hemophilia treatment in schools and social settings.

She says that all of these activities take up a great deal of her time, but feels that the commitment is very important, given some of the issues that her generation faces in the future. "We have to work to tell our story, because if we don't, we risk that people won't care," Mattson says.

For Mattson, who graduated from college in May, she is looking forward to working for a nonprofit organization somewhere on the east coast, where she can continue her focus on advocacy work. "No matter where I land, I hope I will be able to express my views and engage in meaningful issues, as I have been doing with the Council," she said. ●

Kara Flynn *is PPTA's director of Global Communications.*

"No matter where I land, I hope I will be able to express my views and engage in meaningful issues, as I have been doing with the Council."

FROM COAST TO COAST

U.S. Patient Access Advocacy Initiatives

By Ryan Faden and Bill Speir

Looming State Budget Deficits Portend Cuts for Health Care

Florida advocates meet with legislators

Floridians committed to raising awareness for persons with bleeding disorders and primary immunodeficiencies traveled to the state capital in March to advocate on behalf of individuals with rare, chronic, genetic diseases. PPTA staff was delighted to join this energized group and meet with legislators including Rep. Aaron Bean, Chairman of the House Healthcare Council.

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These decision-makers were informed of the need to ensure patient access to the appropriate medical therapy and medical provider. As a bellwether state, Florida is an indicator of what may occur in other states facing budget problems—many states tend to follow the lead of Florida and other states with larger budgets or specialized programs when it comes to managing budget issues.

When the legislature began crafting the 2009 budget, it faced a deficit of $3.4 billion. The Health and Human Services Appropriations Committee chairmen in both the House and Senate were directed by their respective leadership to cut between $750 million to $1 billion from their budgets. In March, the Florida Senate proposed a budget that would eliminate Medically Needy and Medicaid Aged and Disabled (MEDS/AD) as eligibility categories in order to save $300 million.

An individual qualifies for Medicaid as Medically Needy when the cost of their care effectively reduces their income to Medicaid levels. MEDS/AD is an optional Medicaid eligibility category that allows elderly and disabled individuals to receive Medicaid benefits if their income is at or below 88 percent of the federal poverty level. Currently, that level is $10,400 for individuals and $14,000 for a two-person household (see chart on page 19). There are more than 20,000 individuals in these eligibility categories, including 33 with hemophilia.

The patient community, including both Florida chapters of the National Hemophilia Foundation, bombarded the legislature with communications requesting that they refrain from cutting these two eligibility categories. On April 23, 2008, budget conference negotiators from the House and Senate agreed to fund Medicaid services for these individuals with nonrecurring dollars from a state reserve fund, virtually guaranteeing that coverage for persons with bleeding disorders will be on the chopping block again next year.

California Standards of Service legislation overcomes key hurdle

The Hemophilia Council of California has endorsed legislation on Standards of Service for individuals with hemophilia in the state. The Council's endorsement was pivotal in getting the legislation introduced. In consultation with the Council, PPTA offered public support for the legislation through letters to bill author Sen. Darrel Steinberg and to Sen. Sheila Kuehl, the Chair of the Senate Health Committee, as well as by supporting the bill during the hearing. The legislation passed unanimously out of the Senate Health Committee (11-0). This vote marks another crucial step for the bleeding disorders community as it strives to raise awareness and to promote the importance of high-quality care for consumers. The legislation now has been placed in the "suspense file" (hold) of the Senate Appropriations Committee for consideration. That committee produced a report that indicated a small, but not insignificant, financial impact of the bill. Given the current environment in the California budget—an estimated deficit of $17 billion—any additional outlay could be considered a budget buster. The Hemophilia Council of California continues to lead efforts towards standards of care legislation, and PPTA is pleased to offer its assistance in helping to continue the momentum of this legislation in California.

State budgets in crisis on opposite coasts

These two examples show that advocacy and public policy decision-making are inextricably linked with state budget conditions. In early 2007, the National Conference of State Legislatures made public statements that budgets looked favorable in the short term, but that problems could occur in later years. Less than a year later, the Center for Budget Policy and Priorities now indicates that 27 states will face budget deficits in 2008. This abrupt reversal was caused primarily by the breadth of the sub-prime mortgage crisis and all of the associated impacts on state treasuries, particularly in the form of reduced property tax revenues.

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The Hemophilia Council of California continues to lead efforts towards standards of care legislation, and PPTA is pleased to offer its assistance in helping to continue the momentum of this legislation in California.

Of these 27 states, specific estimates are available for 22 states and the District of Columbia. The combined deficits of these 22 states, plus the District of Columbia, are expected to total at least $39 billion for fiscal year 2009, which begins July 2008 for most states. This total represents approximately 9 percent of these states' general fund budgets. Moreover, the number of states facing budget shortfalls may grow during the year as conditions on the ground continue to evolve.

In states facing budget deficits, the consequences could be severe—for residents as well as the overall economy. Unlike the federal government, states cannot run deficits when the economy falters because of their own individual constitutional requirements. As such, they must cut expenditures, raise taxes, or draw down reserve funds in order to achieve balanced budgets. Even if the U.S. does not fall into an actual recession, actions will have to be taken to close the budget gaps that states are now identifying. The experience of the last recession in 2001 will be instructive in predicting what kinds of actions states may take. These actions can basically take three forms:

1. Cuts in services like health and education;
2. Tax increases; or
3. Cuts in local services or increases in local taxes.

As PPTA reflects on state advocacy so far in 2008 and looks ahead to its efforts in 2009, these budget realities must be kept in mind. When states are in fiscal crisis, it becomes that much more difficult to maintain access to health care services, let alone seek expansions or exceptions from state cost-containment efforts. From the Pacific to the Atlantic coasts, and in between, budget deficits will continue to play an increasing role in patient access. Accordingly, these developments underscore the importance of advocating effectively to differentiate plasma protein therapies from traditional chemical pharmaceuticals and other biologics. Educating policymakers about the vulnerable populations in the consumer community and why they are an important constituency is a shared responsibility among all stakeholders. Complacency is not an option. ●

Ryan Faden *is PPTA's assistant director of State Affairs.*
Bill Speir *is the State Affairs Manager.*

2008 HHS Poverty Guidelines

Persons in Family or Household	48 Contiguous States and D.C.	Alaska	Hawaii
1	$10,400	$13,000	$11,960
2	14,000	17,500	16,100
3	17,600	22,000	20,240
4	21,200	26,500	24,380
5	24,800	31,000	28,520
6	28,400	35,500	32,660
7	32,000	40,000	36,800
8	35,600	44,500	40,940
For each additional person, add	3,600	4,500	4,140

Source: Federal Register, Vol. 73, No. 15, January 23, 2008

Navigating Regulatory Red Tape

Making Changes to Approved Drug Applications: *A Regional Comparison*

By Bridget Elis

THREE OF THE WORLD'S LARGEST PHARMACEUTICAL MARKETS are the United States (U.S.), the European Union (EU), and Japan. Drug manufacturers spend a tremendous amount of time, money and effort ensuring that drug approval applications meet the necessary regulatory requirements for approval in all three regions. Nonetheless, the application process continues post approval with the ongoing reporting requirements necessary for implementing changes to an approved application. The U.S. and the EU have made efforts to reduce regulatory reporting burdens. On the other hand, Japan tends to require more detail for reporting changes and has excessive review times for approval of certain modifications.

U.S. "reinvents" process

In 1996, in response to the President Clinton's objective of "Reinventing Government," the U.S. Food and Drug Administration (FDA) amended 21 CFR 601.12, Changes to an Approved Application, in an effort to streamline the federal government. The revised regulation created a three tiered reporting system, the reporting requirements are now dependent on the nature of the change:

- Major changes require the filing of a prior approval supplement (PAS).
- Moderate changes require a supplement submission at least 30 days prior to distribution of the product (CBE 30).
- Minor changes can be reported in an annual report (AR).

To determine whether a change is major, moderate or minor an analysis of the change, as it relates to the potential for causing an adverse effect on the product, is necessary.[1]

EU renews efforts to minimize onerous reporting

In the EU, any amendment to an approved application is called a variation.[2] In 2003, the European Commission (EC) amended its variation regulations.[3] Similar to FDA's effort to minimize unnecessary reporting, the EC wanted to simplify the reporting procedures and also provide the same regulatory framework for variations in the mutual recognition and the centralized procedures.[4]

Regulations (EC) 1084/2003 and 1085/2003 created the following variation categories: Type IA, Type IB, Type II and Urgent Safety Restrictions.

Type IA variations are minor changes, which only require notification. These are administrative changes or other simple changes that have no affect on the quality, safety, or efficacy of the product and can be implemented within 14 days of notification.

Type IB variations are more scientific minor changes and

LEONA BONAVENTURA, JOAQUIN CROXATTO / ISTOCK

implicit approval is granted within 30 days of notification.[5]

Type IA and Type IB variations are specifically defined in Annex I of the Regulation.

Type II variations are major changes not meeting the criteria of minor variations and line extensions.[7] However, due to an exception that does not allow biologics to report variations as Type I, many Type I variations for plasma-derived therapies must be reported as Type II variations, which results in a longer review period for manufacturers before the change can be implemented.[8]

Recently, the EC determined that the revised regulations did not sufficiently reduce the administrative reporting burden because of the different marketing approvals that exist in Europe, i.e., centralized, decentralized, and national. The EC launched a review project aimed at providing a clearer and more flexible reporting mechanism. The goal of the project was to develop a single regulation that would cover changes to all marketing authorizations for centralized, decentralized/mutual recognition and national approval applications.[9]

Post-approval changes yield lengthy process in Japan

Japan revised its Pharmaceutical Affairs Law in July 2002, which amended the procedure for post approval changes.[10] The review time for post approvals is excessive, compared with the EU and U.S. For example, a partial change such as a change in starting material, a change in control criteria of solvent or a change in process control criteria as quality endpoint of process, would take 12–24 months before approval. More minor changes are denoted as notifications and do not require pre-approval.

Complying with the regulatory requirements for changes to an approved application can be daunting. Compared with the EU and Japan, the U.S. has developed a simple mechanism that alleviated some of the reporting burden for post approval changes. However, until all three regions adopt more analogous reporting requirements for changes to an approved application, drug manufacturers will continue to be plagued by unnecessary reports and lengthy approval periods. ●

Thank you to Mary Gustafson, Takashi Fukui *and* Dr. Reiner Laske *for their assistance in writing this article.*

Bridget Elis, JD, *is PPTA's manager, Regulatory Policy.*

1 62 FR 39891

2 European Commission Volume 2A Procedures for Marketing Authorization

3 Id.

4 GMP News: New Variations Regulations 1084/2003 and 1085/2003

5 EMEA Post-Authorization Evaluation of Medicines for Human Use

6 Laske, Reiner Dr.; Variations—Is there hope for relief? IPPC 2008

7 Official Journal of the European Union 27.6. 2003

8 Laske, Reiner Dr.; Variations—Is there hope for relief? IPPC 2008

9 Bachmann, Peter Dr.; Changes Foreseen in the Variation Regulation in a Future Revision; 2008 PDA/EMEA Joint Conference European GMP: Current Issues and Future Developments

10 Guideline on Matters to be Described in Application for Marketing Approval of Pharmaceuticals, etc. under Revised Pharmaceutical Affairs Law (PFSB/ELD Notification No. 0210001)

Comparison: Changes to Approved Drug Applications

Change Category*	US*	EU Current *	EU Proposed*	Japan
Do and Tell	Annual Report	—	Annual Report	—
Tell and Do (immediate)	CBE Supplement	Type IA (2 week valid.)	Type IA	Notification (does not require pre-approval)
Tell and Wait (review period)	CBE 30 Supplement (30 days)	Type IB (10 valid.) (30 days)	Type IB (by default, 10 valid.) (30days)	—
Tell and Wait (review period)	Prior Approval Supplement (4 months)	Type II Variation (by default, 10 valid.) (3 months)	Type II Variation (10d valid.) (3 months)	Partial Changes (12 – 24 months)

*Information supplied by Dr. Reiner Laske

Persistence, Compassion and Vision

Delin Kong: Voice for the *Hemophilia Home of China*

By Rose Noyes

One of the most famous people in ancient China, Confucius (circa 551-479 BC), was a leader in philosophy and he also created many wise phrases and theories about the law, life, and the government.

"Wheresoever you go, go with all your heart."—Confucius

DELIN KONG HAS TRAVELED 12 HOURS through the night, from Shanghai to Beijing. His 6 feet-plus slim frame is easy to see coming through the revolving lobby doors. He is a tall man, who walks slowly to us, with one leg stiffly unbending. His unlined forehead says he is not elderly, but a man with an affliction that slows his gate. He smiles and says, "hello," with the easy grace of someone accustomed to communicating with westerners of different backgrounds and affiliations. Leaning his cane on the back of a chair, he sets his backpack on a seat and carefully lowers himself into a chair facing us. The background noise of chatting groups in our meeting place is muted by the carpeting in the cavernous marble and chrome first floor of the "ubermodern" western hotel. Outside, arching jets of water from a fountain gently bend in the breeze. Just down the road is the sprawling compound, and ancient buildings of the Forbidden City facing Tiananmen Square and Mao Zedong's tomb. Delin has hemophilia and requires frequent injections of clotting factor. He has journeyed to Beijing to tell us about the patient advocacy group he has helped to build.

"To see what is right and not do it, is want of courage or of principle."—Confucius

Delin Kong and the symbol representing his family name.

Delin Kong has led a life admittedly more difficult than someone without hemophilia. The hardships were exacerbated by many of the Chinese government's health care policies. Delin wants to help fellow "sufferers" in his homeland. Inspired by his experiences and the stories of other patients, Delin helped found the Hemophilia Home of China (HHC) in Shanghai in September 2000, where he currently serves as vice president.

The HHC battles to improve patient access to health care and reliable supply of therapies, as well as reimbursement policies, public awareness and education. Although the organization is not officially supported by the government, the group has solidarity that is unofficially recognized. The HHC is supported by the Hematology Institute of the National Science Academy in Tianjin, which in turn is a national member organization (NMO) of the World Federation of Hemophilia. Current membership in HHC is approximately 2,800 people located in 30 provinces of China. Their goal mirrors sister organization goals around the world—to enhance the quality of life for individuals with hemophilia. Delin tries to engage in ongoing dialogue with health officials, especially about critical issues such as shortages of Factor VIII, but public lobbying is restricted. The group's existence and advocacy efforts are not well known throughout the country, because media access is usually limited, except for occasional reports of hemophilia patients dying because of a lack of clotting factor.

Delin is 46 years old and the 77th descendant of the revered Chinese philosopher Confucius, or as he is known in China, *Kongfuzi*. He has household registration status, or *hukou*, which may be the most important determinant of differential privilege in China, and has perhaps inherited some wisdom from his venerable ancestor. He grew up in north Guang Xi provence, the son of parents who were both managers at the local railway station. At the age of one, abnormal bruising caused his parents to bring him to a hospital in Shanghai, where he was diagnosed with hemophilia. Throughout his younger childhood, Delin doesn't remember his parents severely restricting his activities, except for sports, but they always advised him to "be careful." But even with care and attention, simple childhood experiences, such as loosing a tooth, became critical episodes. As he entered his teen years, his health made it difficult to have the same opportunities as his friends. He couldn't commit to having dinner with other children, because he would never know how well he would feel or if he would have a bleeding episode. At the age of 13, Delin experienced severe knee bleeds that required transfusions of whole blood.

Because of lack of prophylactic therapy, the young Delin was housebound. A gifted storyteller with a love of reading, Delin entertained other children with tales of the *1,001 Arabian Nights* and other fairytales. He was surrounded by good friends, who helped push his wheelchair to the hospital when he needed transfusions. His family relocated to Shanghai to be closer to better treatment, and during his high school years Delin met a doctor who also had hemophilia. Because of the doctor's mentorship, he was able to participate in school sports and better understand his symptoms. Delin excelled academically, and passed all of the achievement tests to qualify him for entrance into a university. However, severe health problems due to painful stomach bleeds caused him to drop out after only a year. His situation was so critical that he was resuscitated several times after his heart stopped. Without a job, Delin remained at home with his parents. He taught himself to read and speak English, with the help of Voice of America broadcasts and English-language textbooks. Curious, Delin followed the stockmarket for fun and became an amateur speculator, helping friends and relatives with their investments. He is now married, with a beautiful three-year-old daughter.

"Maybe the world is not fair enough"
—Delin Kong

Delin Kong is a man of principle and courage. His motivation is to help others like himself to have a pain-free, happy life.

Throughout his life, Delin has experienced the highs and lows of living everyday with hemophilia. Guilt from the financial burden placed on his parents, who paid for his clotting factor, compounded the depression, anxiety and chills he suffered. Welcome relief through 50 percent reimbursement from their railway insurance started later in Delin's life. When asked to describe the most scenic places he has lived, Delin replies, "For a person with hemophilia, I have not much comfortable impressions to the places wherever I went or lived. Nothing could be enjoyed until Factor VIII concentrate and self-infusion could be done, because [then the] nightmare disappeared at a second."

Delin was 29 years of age when he started self-infusing. Other people with hemophilia are sometimes less fortunate. In China today there is a rapidly growing economy and subsequent increase in standard of living. China is a staggeringly huge country, where a decentralized government and emphasis on urban development and manufacturing means modernization in rural areas is not as rapid as in the cities. Natural resources and utilities are strained and local tax bases cannot support anything but limited community programs. Because of this, people with rare diseases such as hemophilia cannot easily find care unless they live in cities like Shanghai, which has a very well developed treatment and reimbursement program. Compounding the problems in finding treatment and skilled health care providers are government policies that ban imports of foreign plasma protein therapies and a health care system that is not standardized throughout the nation. Current acute shortages of Factor VIII can be attributed to a dwindling supply of domestic plasma and increased demand.

"When you have faults, do not fear to abandon them" —Confucius

Delin Kong tells a little joke, "Hemophilia in the Chinese pronunciation is 'blood has disease.' The word also sounds like the word friend. 'Blood friend disease.'"

Unlike this attempt at levity, the reality of living with hemophilia in China today is sometimes very somber. People who are not reimbursed in their localities for their therapies must make hard decisions about how much they can afford, if any. Too many sufferers can only purchase enough clotting factor to combat a sudden, serious bleed. But this is not enough to prevent bleeds or general malaise. Since the ban of imported plasma-derived clotting factors, domestic supplies have had safety issues due to inadequate donor screening and arbitrary health department oversight that does not require consistent standard operating procedures from every company inside China. Mandatory government inspections are being conducted by the domestic plasma product manufacturers. There is no nationwide system that will contact users if a batch of clotting factor is found to have problems after distribution. As we speak, Delin's cell phone rings several times with calls from worried people trying to find a source for their Factor VIII.

Delin's philosophy is simple, "either complain or give strategies to help solve the problem." He believes that people with hemophilia have the right to ask their societies for help specific to their need and economic situation. He looks to the plasma protein therapy standards of the United States and Europe for inspiration. He knows that increased interest in rare diseases by the Chinese health officials will result in more positive outcomes for many. He knows that with adequate treatment the economic burden on society is reduced. He knows that sufficient early therapy will prevent extreme joint damage and radically improve quality of life. Chinese health officials are listening, and government policies are changing.

According to Delin, the National Health Insurance System is being enlarged to cover all people in both rural and urban areas. Factor concentrates are now on the "essential drugs" list. Foreign recombinant Factor VIII has just been approved for import. Domestic manufacturers are asked to prioritize clotting factor production, while the Ministry of Health pledges to allocate more plasma for Factor VIII. Beginning July 1, 2008, plasma must be stored for 90 days (inventory hold) and screened for HIV and hepatitis C, before being processed. However, it is likely that this safety measure will result in a temporary reduction of plasma availability.

"Hemophilia can be managed well, if treatment is available," Delin says. "I have not given up hope to have better quality of life. To have equity in society, we must work hard to educate the public and make the government realize the urgent need for treatment. I read Martin Luther King's speeches in high school and I believe that if people with hemophilia want to 'liberate' themselves, the only way is to have help from society and government."

Delin Kong is a man of principle and courage. His motivation is to help others like himself to have a pain-free, happy life. His teachers in high school told him to expect to be an office worker due to his hemophilia handicap. Delin has proven that with conviction, he can be and can accomplish so much more.

To contact Delin Kong email him at: delin@xueyou.org. The Hemophilia Home of China's website is: www.xueyou.org. ●

Rose Noyes *is a guest writer for The Source.*

MY NAME IS TAKASHI FUKUI. I am PPTA's Director of Government Affairs in the Tokyo, Japan office. I will soon be celebrating five years of employment with PPTA.

My job is challenging as I manage all issues on behalf of PPTA in Japan. There is a significant focus on working with the Japanese Ministry of Health, Labor and Welfare and other government entities, however, over time I am hopeful that we will soon be able to focus on forming relationships with the small patient populations that exist in Japan. This will be difficult, given that there are very few organized patient groups, but I am working toward bridging the gap that currently exists.

In my position, I work frequently with Japan's Ministry of Health, talking several times a day with staff in that agency, several councils and the National Institute of Infectious Diseases. I also interface frequently with both PPTA's Japan Board of Directors and communicate often with foreign government dignitaries in need of industry information on plasma protein therapeutics.

Tell us about your background.

I was born in the western province of Hiroshima, five years after the atomic bombs fell in that city. My family was encouraged to leave the city, since there was a threat of daily bombing in the larger cities. After World War II, this area was devastated, both physically and economically.

After graduating from the Hiroshima University, Faculty of Science, I worked for a Japanese pharmaceutical company as a researcher. After that, I worked for 13 years at Fuji Rebio, a pharmaceutical company that has a plasma fractionation operation and that is a leading Japanese company in diagnostics, producing testing kits for HIV, HCV and other automated antibody tests. In this capacity, I was head of the blood business and was served as an executive member of the Japanese Association of Blood Products. In 2000, Fuji Rebio divided their business units and the pharmaceutical business was sold to Union Chemical of Belgium (UCB). I worked for two years at UCB, where I was employed as the head of marketing operations. Around this time, I learned about PPTA from ZLB Plasma in Bern, Switzerland. Ultimately, my background in the blood business and pharmaceuticals, combined with my knowledge of the Japanese government led me to a position with PPTA, where I am employed today.

I'm married and I have two children—a daughter and a son. In addition, I'm a proud grandfather to a little boy, who is 15 months old.

What is your proudest professional achievement?

I would have to say that establishing a relationship with Japan's Ministry of Health has been one of the most important accomplishments I have made in my time with PPTA. This relationship is integral to the work we are doing on behalf of our Association members. It's taken a great deal of time, but over the years we have achieved a relationship where we can find compromises. If the Ministry does not agree with our opinions, nothing happens, so it is critical that we have opened a door and that there is a willingness to listen.

What is most rewarding about working in this industry?

It is rewarding to work for this industry in the sense that I feel like I am able to make a difference. The Japanese culture is very mistrustful of outsiders. We are an island nation and there is a different mentality in my country than there might be elsewhere.

There are still many misunderstandings about the foreign plasma business among Japanese stakeholders. I believe the best way to achieve understanding is for Japanese patients to educate government officials and the general public and create a good relationship that focuses on patient access to plasma protein therapies.

Although I spend a great deal of my time explaining issues and situations, I sense that there is a growing understanding of our industry, and it's gratifying to know that I might have played a part in that. ●

NEWS

PLASMA PROTEIN NEWS FROM AROUND THE GLOBE

EUROPE

› Responding to the current **strong demand** on the French market, the **French Health Products Safety Agency** (Afssaps) issued a website alert to health professionals that outlined a proposal for the prioritization of indications for **human intravenous immunoglobulins (IVIG)**. According to Afssaps, there is a sharp consumption increase. Afssaps mentions that since 2002, on average a yearly 11 percent consumption increase has been observed. In this context PPTA's organization in France, AMDSA, recommends that the following should be seen as priority indications: primary immunodeficiencies with antibody production deficiency, Kawasaki disease and pediatric and adult immune thrombopenic purpura with visceral hemorrhagic syndrome. The Afssaps also outlines indications that only should be granted in the case of a vital need or a failure of therapeutic alternatives, and indications that should be seen as non-priority. In conclusion, Afssaps expressed its willingness to help health professionals in their daily practice and remind them of the need to implement a moderate usage of IVIG therapies given the current context. To view the full recommendation, go to http://afssaps.sante..fr/htm/alertes/al000.htm and to view the annex to the recommendation, go to http://afssaps.sante.fr/htm/alertes/filalert/md080502.pdf.

› The **French Directory General for Health** (DGS) jointly with the **Directory for Hospitalization and Healthcare Organisation** (DHOS) and the French Health Products Safety Agency (AFSSAPS) have issued a circular relating to the **monitoring of human normal immunoglobulins' supply and management of supply tensions**.

Following the Stakeholders Round Table meeting organized in September 2007 by PPTA's French Group (AMDSA), several stakeholder organizations had jointly been pressing the authorities with AMDSA to release such a circular. The circular is available online at: www.adiph.org/TO/circ140308-ApproImmunoglobulines.pdf. A similar circular for coagulation factors had existed in France since 1998.

› Mrs. **Androula Vassilou** is to take over as **European Union (EU) health commissioner** following the resignation of Markos Kyprianou. Mrs. Vassiliou, a lawyer, was a member of the House of Representatives of Cyprus for two terms until 2006, and vice-president of the European Liberal Democrats and Reform Party from 2001 to 2006. She also chairs the board of trustees of the Cyprus Oncology Center.

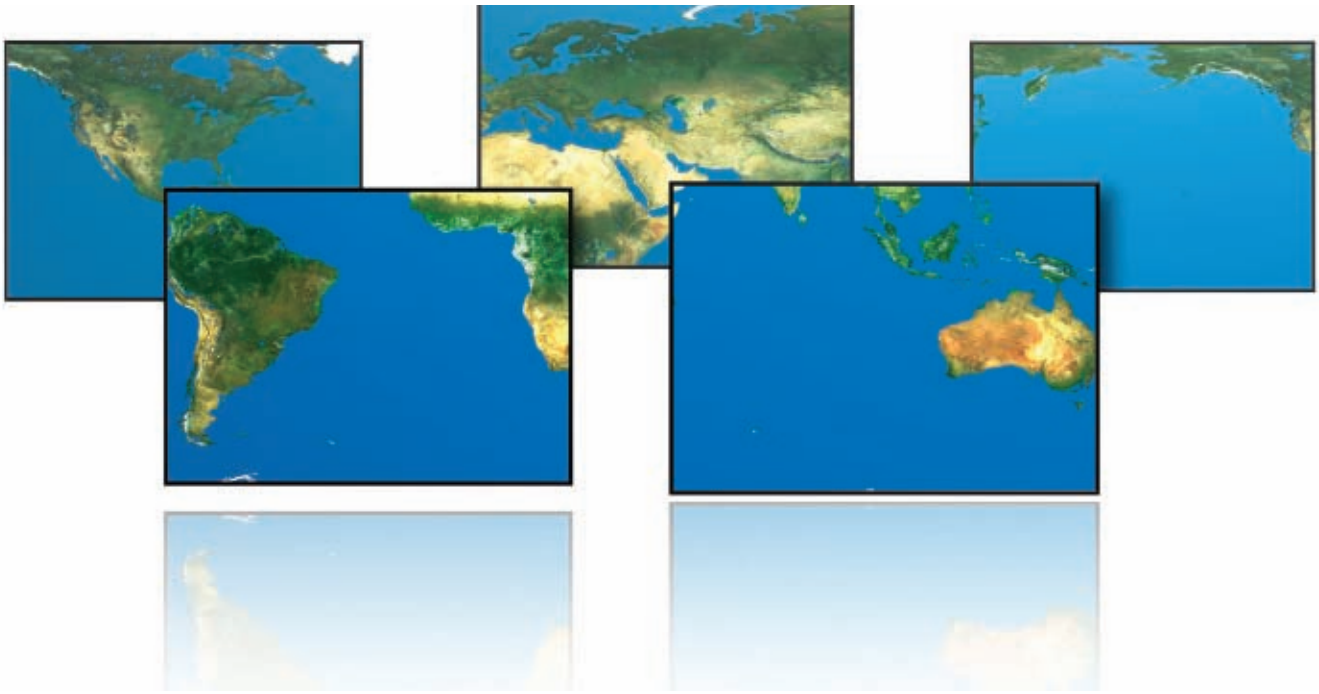
› PPTA's **Belgium** Industry Working Group (BIWG) drafted a statement reinforcing the need to create a **level playing field** in Belgium in order to ensure **patient access** to life-saving and life-enhancing medicines, including plasma-derived medicines. Therefore, PPTA is calling on all stakeholders to work together to remove market distortion that favors one producer over another.

ASIA

› **Japan**'s Ministry of Health, Labor and Welfare (MHLW) created a **pandemic preparedness enhancement office** in its Health Service Bureau.It will comprise 29 experts, including eight from the private sector, and is charged with handling information collection and control, defense against infection, surveillance, R&D, vaccine stockpiling policy and legal policy.

› PPTA learned that in **Japan** the new reimbursement price for **recombinant albumin** is **30 percent higher** than the highest price in the market and about 60 percent higher than imported albumin. PPTA has contacted the U.S. Department of Commerce to ask for clarification during the Market-Oriented Sector-Selective (MOSS) Talks to determine why domestic companies are treated differently than importing companies.

› Recently, the MHLW Blood Council approved the **2008 Blood Collection Plan and Demand and Supply Plan for plasma-derived medicinal products**. The volume of plasma for fractionation is 1 million liters, and is sold to the manufacturers for 13,010 Yen ($130.85) per liter. To cover the need for albumin, Japan would need 1.5 million liters of plasma.



U.S.

› PPTA held its annual **Legislative Day** or "Fly-In" on Wednesday, May 15. Consumer advocates, member company representatives and PPTA staff banded together for more than 60 congressional meetings to educate policymakers on the unique and complex characteristics of plasma protein therapies. Members of Congress were asked to support the **Medicare IVIG Access Act** (S. 2990 and H.R. 2914) , **Increase Lifetime Insurance Caps** (S.2706) and the **340B Program Improvement and Integrity Act of 2007** (S. 1376 and H.R. 2606). In addition, Members of Congress were urged to co-sponsor **H.R. 1282, the Medigap Access Improvement Act of 2007**. Other topics of discussion with House and Senate offices included the current **Medicaid Drug Rebate, Follow-on Biologics and Prescription Drug Pedigree**.

› The **California Board of Pharmacy** announced a decision to **extend the implementation of the pedigree law** until January 1, 2011. PPTA submitted two letters to the Board urging a delay and emphasizing the potential disruption to patient access to plasma protein therapies if an extension was not granted. The Board will be issuing a written decision outlining its reasoning for granting the delay. Most significantly, the Board expressed concern that insisting on the January 1, 2009 could put the public safety at risk and could threaten access to life-saving medicines.

› PPTA staff organized a **Patients' Day** at the **Florida** Legislature in Tallahassee on March 19. This is the first time that PPTA, in coordination with local consumer group representatives, has held such an event at the state level. Patients representing individuals with bleeding disorders and primary immunodeficiency diseases were empowered to share their personal stories with legislators and, in their own words, convey the importance of **access to plasma protein therapies**. The meetings were held with some of the most prominent Members of the Florida Legislature including Representative **Aaron Bean**, Chairman of the House Health Council.

› Building upon the success of the event in Florida, on April 23, PPTA staff organized a similar **Quality of Care patient day in Minnesota**, which also included representatives of the bleeding disorders, immune deficiency and alpha-1 communities. Meetings focused on Quality of Care legislation that will be under consideration again during the 2009 legislative session.

› PPTA staff participated in the **Ohio Bleeding Disorders Council's Legislative Day** in Columbus, Ohio in April. More than 40 individuals from the patient community and industry broke into groups to meet with legislative offices. The messages conveyed included the need for an increase in the age for dependent coverage in insurance contracts, increased funding for the Hemophilia Insurance Premium Program and the need for a Hemophilia Advisory Board in Ohio.

› The **IVIG** community, led by the Immune Deficiency Foundation (IDF), received additional support from Senator John Kerry (D-MA), who introduced S.2990, The Medicare IVIG Access Action on May 7. The legislation is similar to the House bill that focuses on **Medicare beneficiaries' access to IVIG**. Senator Kerry is expected to push for the inclusion of this bill in the Senate Finance Committee's Medicare package.

Washington, D.C. Mayor Adrian M. Fenty recognized PPTA and the upcoming Plasma Protein Forum with a letter welcoming stakeholders and industry to the city.

Welcome

Plasma Protein Therapeutics Association

Forum

June 16, 2008

As Mayor of the District of Columbia, I am pleased to extend a warm welcome to the Plasma Protein Therapeutics Association, on the occasion of your Forum.

This event provides an opportunity to discuss issues of concern as they relate to plasma protein therapeutics. As you take this time to reflect on your past accomplishments, I welcome you to our city and I invite you to experience our night life, eat in our restaurants, and visit our monuments, museums and diverse neighborhoods. We look forward to your continued support to help enhance the quality of life of others.

On behalf of the residents of the District of Columbia, you have my best wishes for a productive and enjoyable event.

Adrian M. Fenty
Mayor, District of Columbia

› PPTA staff attended the **National Hemophilia Foundation Washington Days** conference March 5-7. The conference brought together chapter leaders and consumers from 41 states. Key legislative issues discussed included legislation to expand **access to Medigap policies**, raising lifetime maximums, achieving passage of the Genetic Information Non-Discrimination Act, and quality of care legislation.

› In late April, U.S. House of Representatives, Congressman Jim Matheson (D-UT) and Steve Buyer (R-IN) introduced the **Safeguarding America's Pharmaceuticals Act of 2008**. The legislation provides for a national uniform standard for the numerical serialization of drugs and biologics that preempts any and all current state laws. The bill gives the Secretary of Health and Human Services (HHS) the authority to identify high-risk drugs and biologics that are likely to be counterfeited or diverted and require manufacturers to implement standard numerical identifiers and track and trace technologies with input from manufacturers, distributors and others in the supply chain. PPTA met with the authors of this legislation in early May to communicate the Association's basic principles for the establishment of prescription drug pedigrees. ●

EVENTS

UPCOMING CONFERENCES& SYMPOSIUMS

2008

June 17–18	PPTA Plasma Protein Forum 2008 *Washington, D.C., USA*
June 19–22	13th International Congress on Infectious Diseases *Kuala Lumpur, Malaysia*
June 22–26	44th Drug Information Association Annual Meeting *Boston, USA*
September 6–9	32nd Annual Meeting American Association of Tissue Banks *Chicago, USA*
September 12–14	European Haemophilia Consortium (EHC) Annual Meeting *Dublin, Ireland*
September 16–19	41st Annual Meeting of the German Society for Transfusion Medicine and Immunehaematology (DGTI) *Düsseldorf, Germany*
October 5	PPTA Source Business Forum *Montreal, Canada* PPTA members only
October 4–7	AABB Annual Meeting and TXPO *Montreal, Canada*
October 7–11	11th International Conference in Thalassaemia and Haemoglobinopathies *Singapore*
October 16–19	XIIIth Meeting of the European Society for Immunodeficiencies (ESID) *'s-Hertogenbosch, The Netherlands*

2009

March 3–4	International Plasma Protein Congress *Paris, France*
March 23–25	21st Annual EuroMeeting of the Drug Information Association *Berlin, Germany*
May 28–30	57th Annual Congress of the Japan Society of Transfusion Medicine and Cell Therapy *Saitama, Japan*
June 2–3	PPTA Plasma Protein Forum 2009 *Washington, D.C., USA*
June 4–7	14th Congress of the European Hematology Association *Berlin, Germany*
October 24–27	AABB Annual Meeting *New Orleans, USA*

2010

June 15–16	PPTA Plasma Protein Forum 2010 *Reston, VA, USA*
June 26–July 1	XXXIst International Congress of the ISBT *Berlin, Germany*
October 7–10	XIVth Meeting of the European Society for Immunodeficiencies *Istanbul, Turkey*

INTERNATIONAL PLASMA PROTEIN CONGRESS

3 & 4 March 2009
Marriott Rive Gauche Hotel
Paris, France

SAVE THE DATE