

The Source

The Newsmagazine of the Plasma Protein Therapeutics Industry



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RN Puts Young, Vital Face on Hemophilia
Interview with Professor Rainer Seitz

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"As a first time attendee, the meeting was fantastic!" M. Kevani, KOA Eng. (Iran)

"An interesting, enjoyable and beneficial event" L. Warren, Alpha One Foundation (Ireland)

"I am leaving with a wealth of information. Thank you!" K. Antilla, Immune Deficiency Foundation (United States)

Home

Dear Friends and other plasma protein industry stakeholders,

It is with great pleasure that I welcome you to PPTA's 2008 International Plasma Protein Congress (IPPC) website. This year, for the 14th edition of the congress, we look forward to welcoming you to Warsaw in Poland on 4 and 5 March.

On this website you will be able to find all relevant information relating to the event, from the preliminary conference agenda, congress registration and hotel reservation forms, opportunities for sponsors and exhibitors and details of our supporting organizations. As we get nearer to the event additional details will be added and you should receive regular reminders through email messages.

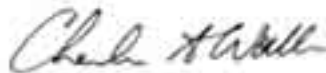
Since 1994 we believe that PPTA's Congresses have provided valuable insights into the issues affecting the plasma protein industry.

IPPC has been the primary international congress for all stakeholders of plasma protein therapies. Building on the experience and success of the past but with a clear eye to the future, the IPPC 2008 offers delegates a unique, two-day programme with speakers of the highest calibre, representing institutions and organizations that provide leadership and direction in several important areas.

Sessions will feature topics such as clinical developments, patient access, self-sufficiency, regulatory considerations, quality (GMP), contract fractionation, plasma collection, health policy and much more. There will be ample time for discussion and questions after the presentations and during the cocktail reception and gala dinner on 4 March which will complement the full and diverse programme.

The interests of patients and physicians are everyone else's number one priority, be they politicians, regulators, scientists and healthcare funding organizations. Our intention is to create a congress for all.

We hope to see you in Warsaw in March 2008.



Charles Waller
Executive Director
PPTA Europe

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THE SOURCE STAFF

Managing Editor: Kara Flynn

Editors: Sybille Beck, Kym Kilbourne

The Source is published four times a year by PPTA, 147 Old Solomons Island Road, Annapolis, MD, 21401. Phone: +1.202.789.3100
 e-mail: thesource@pptaglobal.be

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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 newsmagazine may contain statements of opinion on such issues. These statements are those of the author and do not necessarily reflect the opinion of PPTA or its members.

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The QSEAL Standard

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In my View

Everywhere in the world people require plasma protein therapies to treat their medical conditions, sometimes life-threatening, always serious. The degree of treatment is dependent upon several factors, such as awareness, diagnostics, availability of therapies, and proper reimbursement. In this column I want to focus on the availability of therapies, and, more specifically, to highlight some barriers that are still in place that limit patient access to care. Let me give you some examples:

Japan

In 2003, Japan issued a Blood Law that requires self-sufficiency in 2008. It is well known that this cannot be accomplished and there will be an adjustment regarding the time it is expected to be finalized. Achieving self-sufficiency for finished products is a political goal and has less to do with clinical need. The use of IVIG in Japan has been stable for almost 20 years, a sharp contrast with other developed countries where, as a result of greater awareness and better diagnosis, annual growth numbers are between six and eight percent. When a country's consumption levels remain constant, one can only conclude that this country does not provide the necessary care for many patients with immune deficiencies. New initiatives are needed to help the Japanese authorities understand that early diagnosis and treatment is more cost efficient than waiting for expensive hospitalization costs and loss of labor when patients are not diagnosed. I am certain that in one of the next editions of *The Source* we can report about such an initiative.

China

In 1989, the Chinese government enacted Article 49, which prohibits companies from bringing plasma protein

therapies to China with the exception of albumin. The rationale for this law developed in the 1980s, after the HIV tragedy that affected so many people with hemophilia. In today's world, not only has the industry dramatically changed, but HIV in China by far exceeds any prevalence of HIV in other developed countries. Nevertheless, Article 49 is still in place and prohibits companies from bringing their newest therapies based on current technology to the market.

Chinese authorities have expressed their concerns about the quality and safety of domestically manufactured therapies. As a result of that, double viral inactivation and a 90-day inventory hold have been introduced for domestic manufacturers. Almost immediately, an impact could be seen on the market and fewer therapies became available. Chinese hemophilia patients have vocalized concerns about the reduced availability of therapies.

PPTA is working to find ways to have Article 49 removed.

France

For years the French authorities have discriminated against importers of plasma protein therapies. When a product is made from plasma from compensated donors, the marketing authorization is valid for five years. When a product is produced from plasma collected from donors who have not been reimbursed for their inconvenience and costs, it is only valid for two years. This is a clear case of regulatory discrimination. Though France is in principle an open market, one can imagine that having different marketing authorization periods undermines the credibility of imported products and, as such, influences clinical choice.



Jan M. Bult / President, PPTA

Tenders

Though it can be understood why some countries decide to use a tender to obtain certain quantities of therapies, there are many downsides to it as well. The first is that when tender switches from one company to another one, all patients need to switch their therapy, and in most cases this is an undesired outcome. For companies, it is difficult to bid on high volumes when there is no guarantee that this tender will repeat itself the next year, and it is important to have long-term commitments, for stability and predictability in operations.

Sometimes companies face competition on tenders from companies that are quasi-subsidized by governments, and very low prices result. It is okay and fair that prices are under pressure in an open market, however, it is unfair if low prices are coming from companies that are having the benefit of local governmental support. This will impact patient access to care, because when price is the only driver for clinical choice, how sure can one be that the right therapy is chosen and available for a longer period of time?

The challenges are many, and PPTA is putting forth great effort to affect solutions.

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Source Plasma Collection in the USA

By Jan M. Bult

Source plasma comes from donors who donate their plasma through a process called plasmapheresis. This technology allows donors to donate up to twice a week in more than 300 centers in the United States. Each plasma donation center needs to meet stringent U.S. Food and Drug Administration (FDA) requirements and is regularly inspected. If the plasma collected in that center is used or manufactured in other countries, additional inspections will occur to ensure compliance with the regulations of that other country. New centers are being built and some are awaiting FDA approval; we can anticipate a higher number of centers in the next year. Currently, there are 306 International Quality Plasma Program (IQPP) certified plasma collection centers in the United States.



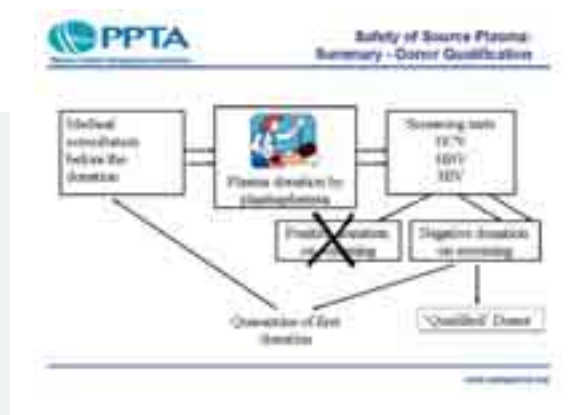
PPTA's predecessor, the American Blood Resources Association (ABRA), developed a set of voluntary standards to further the quality and safety of the plasma. These standards are part of the IQPP, some of which are described in more detail below.

It is very important that plasma is collected from healthy donors who are free from diseases. Before a donor can be accepted, the name of the "applicant" is checked against the National Donor Deferral Registry (NDDR). This nationwide registry contains the names of all individuals who have been found positive in the past for HIV, Hepatitis C and Hepatitis B. This check ensures that no one can

become a donor again once identified as positive for any of these three viruses.

To ensure donor health, it is important that donors do not donate more than the allowed frequency. Common practice at many centers is to use a system where a donor's fingernail is first stained and then checked under an ultraviolet light prior to donation. The stain is not visible in normal daylight. Only when these checks are completed is a donor permitted to continue the process.

The Qualified Donor Standard is a cornerstone of the IQPP program. This standard ensures that no plasma is used from one-time only donors. The qualified donor is a committed and regular donor, allowing collected plasma only to be used after the donor has completed two separate screenings and tests.



IQPP CERTIFICATION AND AUDITING

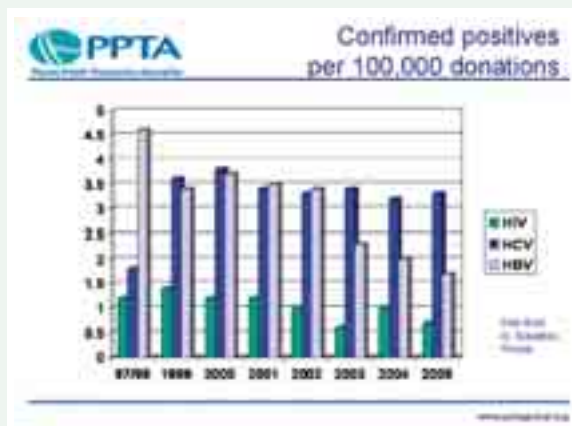
IQPP certification is possible for all Plasma collection centers that have met the FDA requirements and passed an FDA inspection. The IQPP standards are voluntary and focus on areas beyond the regulatory requirements. This minimizes duplication between auditing organizations.

The IQPP program is dynamic and undergoes an ongoing thorough review. The audits are performed by independent certified auditors (five in the United States). Audits can be done on a corporate level, a center level or (with small organizations) as a combined audit.

For larger organizations who own multiple centers, one audit is done on a corporate level to basically review all about “Say what you do”. The purpose is to review Procedures. This focuses on all Standard Operating Procedures (SOP), company policies and on record checking for all the performed functions (customer complaints, viral marker reporting etc). The audits on a center level then focus on: “Do what you say,”, in other words, the implementation. The auditor goes into greater detail about the implementation of the SOP’s, policies and programs and includes training, shipping and unit control, use of the NDDR and so on.

VIRAL MARKER STANDARD

About ten years ago, the Viral Marker Standard was introduced. This standard ensures the collection of plasma from low risk populations. Every center is obliged to report the sero-conversion rates in repeat donors on a monthly basis. **IT IS IMPORTANT TO KNOW THAT NOT A SINGLE POSITIVE DONATION WILL BE ACCEPTED.** These are compiled into an industry average resulting in an alert limit. This limit is corrected for volume, which means that there is a fair approach among the entire range of plasma center sizes. If there would be no correction, then a small center would be seriously disadvantaged. If a center is on the alert list, then corrective actions need to be taken to ensure that the center is no longer on that list after 12 months. If the corrective actions do not show sufficient progress toward remedying the problem, then the certification will be revoked. Following this standard helps ensure that the donors are from a low risk population. The next slide shows the viral marker rates in the last ten years for qualified donations.



CENTER LOCATION

Plasma collection centers are located throughout the country. They can be found in cities, rural areas and in some cases, along the borders. Recently the PPTA Board of Directors discussed this issue and agreed to the following statement:

“PPTA supports the collection of high quality and safe plasma from committed donors. Its IQPP standard program demonstrates that. PPTA does not provide guidance on the location of centers and accepts this as a company responsibility.”

This statement was necessary after some discussion about centers located along the United States border with Mexico. Currently there are ten centers along the border of the state of Texas and Mexico. This represents 3 percent of the centers in the United States. These centers collected in 2006 a total of 560,000 liters of source plasma, which represents 4 percent of the total volume collected in the USA. This volume results in:

- Approximately 10 million units Factor VIII
- Approximately 1,500 kg IVIG
- Approximately 75 kg A1P1
- Approximately 10,000 kg Albumin

These quantities are important to supply various patients in critical need of plasma protein therapies. There seems to be many misperceptions stemming from the fact that not many people have personal experiences with the collection in these (excellent) centers. It is important to know that along the U.S.-Mexico border there are many cities, in some cases divided by the Rio Grande River. One example is the city of Eagle Pass, Texas with 20,000 U.S. citizens and 300,000 Mexican citizens in Piedras Negras, the adjacent city. People cross the bridge every day and visit family or go out for shopping. One of the concerns is that “poor Mexican nationals in a poor health condition” cross the border and become plasma donors. This is so different from the reality that this needs to be corrected. During a recent visit to these centers, I was able to learn first hand what the facts are.

The centers in question have a mixed donor population. There are donors living in the United States and there are donors that cross the border. Every person from Mexico

crossing the border into the United States needs to have a valid Visa. In most cases it is a B1/B2 Visa that allows entry into the United States for one day and within 14 miles limit of the border. This Visa is used for visiting and shopping. The U.S. requirements to obtain a Visa are very strict. An applicant needs to have a job and a permanent address. In addition a fee is required to be paid to the U.S. immigration authorities.

These requirements are crystal clear and immediately remove any concern about the social status of a Mexican national donating plasma in a U.S. center under the strict requirements set forth by the FDA. Having a Visa is an enormous incentive and there is no desire to do anything that would jeopardize the ability to obtain or maintain a Visa!

I have visited five centers along the border in Del Rio, Laredo and Eagle Pass. And what I have seen is very impressive. When PPTA started with its IQPP standard program, one of the goals was to have committed donors from a low risk population donating high quality and safe plasma. Well, I have seen it in these centers. The donor population consists of many committed donors, with most of them (over 90 percent) return donors. Each donor is checked at every donation for: pulse, blood pressure, weight, hematocrit and total protein. Only when all criteria are met, can the donation occur. When speaking with these donors, you hear the same message over and over again: "I am pleased to know that my donation helps other people."

The staff at the centers is very good in explaining what the plasma is used for and many donors are proud that "part of them" is traveling to help others. The donations are done in a very professional, clean, well organized center in a medical setting. These are centers that the industry can be proud of! The staff is also proud of its donors, as shown in the next picture.



Profile of a Hero

By Rose Noyes

Irma Martinez answers her phone. She is a diminutive, pretty woman with soft brown hair and a face that can express intense concentration, deep concern or delighted amusement. Behind her organized desk topped with photographs of her children and a computer keyboard, her office wall is decorated with framed poems and an iridescent Philippine capiz mother of pearl cross. Her face lights up with a smile - the caller is her youngest son asking if she will be home in the daytime or nighttime. He is seven years old and an A+ spelling scholar. He calls everyday with this same question and she looks forward to hearing his voice and taking a few moments to clear her thoughts before resuming her demanding responsibilities. Her phone rings again and now she responds with calm authority to the questions she is asked. Her staff poke their heads in to ask more questions.

She handles all with a dexterity that only experienced managers possess. In the next 30 minutes she will complete several reports, answer correspondence and leave her desk to journey downstairs for a closeup assessment of operations. Irma takes her job as Center Manager for Talecris' Plasma Resources in Eagle Pass, Texas very seriously. But this commitment to donors, her staff and the operations of her center mean working long hours. It means that she will sometimes miss helping with homework. And so her three children, especially the spelling champion and the junior. high school honor roll student, understand their mother will be at work long after other mothers and fathers are home with their families. They are proud of her status as the center manager and joke that they "have a mom who can fire their mom". Her rise to the top position was quick - after two months as a Physician Substitute at Seracare in October 2000 she took over as Center Manager under Biomat. She then relocated to her present position in Eagle's Pass to work for Talecris. The companies she has served under recognized her talent, drive, and professionalism.

Irma has a simple philosophy - customer service and customer respect make or break a business. After all the operating procedures, technical guidelines and good

manufacturing practices are met she knows that each donor who returns and continues donating feels safe with the pheresis process and feels welcome at her center. Her 95 percent donor return rate shows this commitment. Irma treats her donors like an extended family. As she makes her daily rounds to monitor operations, she is greeted by smiles and inquiries about her health, or the status of her soon to be born grandchild. She counters with her own questions about donor's children (many call her Tia or Aunt) and sometimes extends incentives for a good report card. When she needs a midday break, she takes a short walk in the fresh air to see the sun dappled palmettos and banana trees that accent the neighborhood. She knows by name the



Irma Martinez

passersby on the sidewalk who call out hellos. This family atmosphere brings in the donors.

There is a circle of life and hope that surrounds the day-to-day activities at Irma's center. The donors all know that donations of plasma are the basis for multiple therapies that save lives and improve quality of life. Irma describes with a smile how every donor reacts with wonderment when they watch a video that details the journey of their plasma to a fractionation facility on the east coast. In their belief, this is a piece of themselves that is making the long journey to be transformed into a substance that can do so much good. Many of these donors have never been more than a few miles from their homes, but in a way their plasma can put on seven league boots and travel the world. But many of the donors and Irma's staff know that she herself relies on their donations. Irma was diagnosed with cancer and wears a chemo pump three days a week on

alternating weeks. She has had infusions of plasma therapy. They have saved her life. Many days when she feels too sick and weak to move, the encouragement and love of her donors and staff give her the will to keep going. She wants to show them that they help her overcome her illness, they make her stronger. On her birthday the whole center gave so many gifts and flowers that Irma had to place some out in the hallway. What goes around comes around - her donors donate their plasma and their good wishes, but they and the communities they live in are grateful for the helping hands of Irma and her staff. On Tuesday evening, April 24, 2007 a severe thunderstorm with baseball-sized hail pummeled the Eagle Pass area and Piedras Negras in Mexico. It spawned an F-3 category tornado that left a quarter mile wide swath of destruction for one mile. Seven people were killed and 80 injured. Residences, businesses and an elementary school were destroyed. Many were left homeless and without food and water. Those affected included many donors and their families. Using her month's pay, Irma and her staff organized a caravan of three vehicles with groceries and building materials and distributed the supplies to as many of the most needy families as she could. One small boy brought tears to her eyes when he thanked her repeatedly for a tube of toothpaste, even though he had nothing to eat.

The measure of a successful businessman is the profitability of their enterprise, the measure of a person's humanity is the goodwill of those around them. On both of these scales, Irma Martinez is at the top. She is an inspiration to all who know her, a savvy businesswoman, and a loving mother. Irma Martinez, Center Manager, Hero.

Impact of Medicaid Reimbursement on Access to Therapies

By Bill Speir

Medicaid is the fastest growing segment of many state budgets.¹ Medicaid funds provide enrollees with access to vital health care services. Basic economic theory shows that access to this care depends upon adequate reimbursement. The federal Medicaid statute codifies this principle by requiring states to have reimbursement mechanisms, "sufficient to enlist enough providers so that care and services are available."² This creates a difficult balancing act for decision-makers who must control costs while ensuring enrollees access to health care.

A common cost control strategy employed by most states in recent years is to reduce reimbursement levels for pharmacy services. This creates access problems for Medicaid enrollees when the reimbursement becomes so low that providers are unable to provide the therapies. This situation often arises when acquisition costs are greater than reimbursement, commonly referred to as being "under water."

The basic formula for reimbursement is ingredient cost of the drug plus a dispensing fee, which is designed to approximate the costs associated with preparing the drug and other miscellaneous costs associated with the practice of pharmacy. Some states include a copayment in their reimbursement structures. States currently set ingredient cost reimbursement based on the four pricing concepts below:

- **Average Wholesale Price (AWP)** is the average price that wholesalers charge entities in the retail class of trade.
- **Wholesale Acquisition Cost (WAC)** is the average net cost the wholesaler pays the manufacturer.
- **Federal Upper Limit (FUL)** is the maximum amount to be paid for a drug as established by the federal government. State Maximum Allowable Charge (SMAC) is the maximum amount a state will pay for selected multi-source and generic drugs; these can be lower than FUL prices.

To reduce pharmacy reimbursement costs states may: 1) reduce ingredient cost reimbursement; 2) reduce the dispensing fee; and/or 3) increase the enrollee copayments.

The enactment of the Deficit Reduction Act of 2005 (DRA) provides options for states in reducing pharmacy reimbursement by providing more information about the costs of prescription drugs and biologicals, and allowing states to charge higher copayments. The DRA requires the federal government to provide states with Average Manufacturer Prices (AMP) on a monthly basis. AMP prices are believed to be lower than the methods described above. This may result in states changing their ingredient cost reimbursement to AMP-based reimbursement to contain pharmaceutical costs in their Medicaid programs.

Prior to the passage of the DRA, an enrollee's copayment could not be more than a "nominal" fee and a pharmacist could not deny the recipient the product if they did not pay the copayment, these amounts typically ranged from \$1 to \$3. Under the DRA, states may require aggregate copayments up to 5 percent of an enrollee's income and allow pharmacists the right to deny prescriptions when the enrollee does not pay the copayment. It should be noted that many observers have speculated that the increased administrative burdens associated with copayment enforcement and tracking would exceed any additional savings resulting from the copayments.

Reduced reimbursement levels may result in providers deciding to no longer serve patients within the Medicaid program. A parallel can be drawn to the impact lower reimbursements for IVIG have had on the Medicare program.³ The federal government reduced the reimbursement for IVIG to Medicare physicians beginning in January, 2005. The reimbursement did not equal the physician's cost for providing the IVIG to patients. As a result, many physicians stopped providing IVIG and their former patients received their IVIG treatment in hospital outpatient facilities. Physicians referring Medicare patients to hospitals have found that hospitals are sometimes unable to procure the same products, thus in some instances, requiring more time for clinical monitoring. Reductions in



a state's Medicaid prescription drug budget could have the same potentially negative impact on patient access if reimbursement for plasma protein therapies is reduced to save costs.

PPTA is concerned that reductions in state Medicaid pharmacy budgets could ultimately lead to unintended increases in the state's total Medicaid costs and poor health outcomes for its Medicaid enrollees that need plasma protein therapies. Reimbursement should be sufficient to ensure access to care for Medicaid enrollees who require life-saving plasma protein therapies to lead healthy productive lives, because access to care equals quality care.⁴

For further information, please contact Bill Speir, Manager of PPTA State Affairs, at bspeir@pptaglobal.org.

¹ THE FISCAL SURVEY OF STATES: JUNE 2007, the National Governors Association and the National Association of State Budget Officers.

² 42 U.S.C.A. §1396a(a)(30)(A)

³ Assessing the Cost of IVIG Infusion Services in Physician Offices & Hospital Pharmacy Departments, The Lewin Group (March 23, 2006).

⁴ See Aiming Higher: Results from a State Scorecard on Health System Performance, The Commonwealth Fund (June 13, 2006).

A National Framework Agreement

By Alexandra de Mello-Gouveia

The United Kingdom's (UK) National Health System (NHS) recognizes the importance of adequate and secure stocks of human intravenous immunoglobulin (IVIG) as critical to patient care. In a recent publication by the NHS Purchasing and Supply Agency (NHS PASA), the issue of supply shortage and the steps the UK authorities and the Agency have consequently taken were outlined.

In the last year, the UK Department of Health (DH) observed reduction in the available supply of IVIG and in turn an increase on prices. A number of factors have triggered this situation: the increased cost of plasma, increased manufacturing costs as a result of rigorous safety controls, reduced demand for other plasma protein therapies. In addition, increased demand for plasma by overseas governments and the decision taken by the Scottish National Blood Transfusion Service to halt manufacturing has led Scotland to source its production on the open market and overall there has been a substantial rise in demand.

The UK DH carried out an exhaustive assessment of the IVIG market in 2006, which revealed a shortage of supply globally and a lack of commitment by the NHS and suppliers. The assessment essentially recommended a resolution which depended on the commitment from both the NHS and suppliers to ensure adequate supply for critical patients. Additionally, a set of guidelines on the use of IVIG and a demand management plan for the use in periods of shortage is being drafted by the UK DH, modeled on proposals from their advisors, Deloittes.

In response, the NHS PASA has worked in collaboration with the National Pharmaceutical Supply Group and the Pharmaceutical Market Support Group to develop a national framework to ensure the availability of the therapy. As of June 2007, the framework has been supported by commitment from the NHS at either local Primary Care Trusts (PCT) or regional purchasing group level. NHS PASA solicitors have drafted commitment contracts, which once signed by the NHS and suppliers, legally bind both parties to deliver, receive and pay for the agreed volumes under the terms and

conditions within the national framework agreement. The NHS PASA believes that under this new framework agreement suppliers will have to commit to minimum guaranteed volumes to the NHS PCTs will have contractual commitments for crucial volumes which will ensure that critical patients receive care, suppliers will be able to plan better and assure a supply. NHS PASA will collect data monitoring supplier stock availability thus avoiding future shortages becoming critical, and the NHS will have a strengthened position in the global market.

In order for the framework to be successful, the NHS PASA requires that trusts deliver solid commitments to suppliers to purchase therapies, and in turn, suppliers will have to make commitments, to make minimum volumes available. Expert clinicians within trusts have been consulted by purchasing pharmacists to accurately determine future volume requirements. DH believes sufficient volumes to meet the entire demand are unavailable for the coming two years, so trusts have had to identify which usage is vital and where other treatments or alternatives can be used. Trusts are not obliged to sign the commitment contracts and will still benefit from the framework agreement; however, those who do sign the contracts will be given priority. Trusts will collaborate as regional groups respecting commitment duties and making sure stock excesses or shortages are controlled. The NHS PASA will play a managerial role to ensure commitments are upheld by all parties and that potential severe shortages are detected in advance.

For more information on this issue, please visit the NHS PASA website at:
www.pasa.nhs.uk/PASAWeb/Productsandservices/Pharmaceuticals/Bloodproducts/Bloodproducts/Immunoglobulins/LandingPage.htm

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A Mother Fights for her Family and Sheds Light on the Realities of **Living with Hemophilia**

By Kara Flynn

The Western Pennsylvania Chapter of the National Hemophilia Foundation works to help improve the lives of members of the bleeding disorders community through educational programs, guidance, and support.

Kerry Fatula serves as the vocal and passionate executive director of the organization. She brings a great deal of enthusiasm and a wide range of knowledge to her work, stemming primarily from her own personal experiences that have been shaped by a lifetime of living with and caring for family members who have been affected by hemophilia.

even before she got married and started a family, that being the daughter of someone with hemophilia meant you were almost inevitably a carrier of the rare disease, however, it didn't stop her from wanting a family of her own.

Ms. Fatula went on to have four boys – three of whom have hemophilia. The Fatula brothers — Paul, 18, Nathan, 16 and Ethan, 5 — have regular intravenous infusions of clotting factors to prevent them from bleeding uncontrollably.

At the beginning, Ms. Fatula said she lacked confidence and doubted her ability to take on the considerable responsibilities of caring for children with hemophilia. She

Caring for a child with hemophilia can be quite difficult not to mention the extraordinary financial considerations for a family...

Ms. Fatula formed her understanding of hemophilia at a very young age, as her father suffered from the rare disease. "Often, if my dad was in pain, we were asked to go to our rooms, while he stayed on the sofa," she said. "He didn't want us to see him that way, and it was kind of scary."

She says her father's sister had two boys with hemophilia, and often shared her experiences with her when she was growing up. "My educational background largely came from my aunt," Ms. Fatula said. "She explained a lot of things to me and provided me with some sense of what to expect. But even after all of her stories, I still really wasn't prepared for the reality of living with hemophilia. Even if someone thinks they know what they are doing, there are always surprises."

During a stint in the U.S. Air Force, Ms. Fatula met her husband Chuck, who was an aircraft mechanic. She knew

says the learning curve is long at least two to three years and every life transition from daycare to pre-school is magnified a hundred times over given that certain consequences need to be made part of the considerations.

For example, when her son Paul was a toddler, she said a closet door accidentally fell in his bedroom on the little boy as he was playing, unbeknownst to her, and landed on his stomach, where he started to bleed internally. She said if it wasn't for her intuition, her son would have bled to death, and that's just one example of the many times there have been life-threatening episodes in her home. A random fall at the playground could have had dire consequences for her children beyond just the normal bruises and scrapes that a typical child might endure.

Caring for a child with hemophilia can be quite difficult not to mention the extraordinary financial considerations for a family, and it is for this reason that Ms. Fatula has devoted



Kerry Fatula works to bring a face to hemophilia in her Western Pennsylvania Community.

her time to educating people about the disease. “I didn’t start out being proactive about becoming involved in these issues, but it became necessary over time, given that it’s critical to bring attention to these issues – particularly with policymakers at the state level,” she said.

Her eldest son, Paul, is no longer covered under her husband’s insurance, having maxed out his benefits taking the therapies that he depends on to live his life since birth. Ms. Fatula said Paul now needs to either apply for disability insurance or obtain a job with excellent medical coverage. Either way, the choices can be difficult. Again, it is for this reason, and the numerous access to care issues facing individuals in their community with bleeding disorders, that she says she works overtime on a multitude of projects at

the Western Pennsylvania Chapter of the National Hemophilia Foundation. The chapter provides advocacy programs and outreach to others who might be experiencing the same difficulties that she is going through.

“We are grateful for the plasma-derived and recombinant therapies produced by the members of PPTA, because they really do save lives,” Ms. Fatula said. In fact, she is excited to tell her stories to an international audience when she speaks at the next International Plasma Protein Congress (IPPC) hosted by PPTA and to be held in Warsaw, Poland on March 3-4, 2008. In her remarks, Ms. Fatula says she plans to discuss access to therapies and provide anecdotal stories about people who have experienced a need for one product over another. “Educating people about hemophilia isn’t so difficult, however, it’s hard to make people understand why it’s worth the expense,” she says. “A small portion of the population needs these critical therapies, and without them, my kids wouldn’t be alive. This is the message I want to bring to people who might not think insurance coverage for these therapies is of the utmost importance.”

For more information on IPPC, please contact Sophie van Puyvelde via email at sophie@pptaglobal.eu.

RN Puts Young, Vital Face on Hemophilia

By Kym Kilbourne

At a very young age, two things were quite clear to Matt Stinger; he had hemophilia and it wasn't going to hold him back.

The 24-year-old Hatboro, Pennsylvania native is now a registered nurse, working in the emergency room of one of the same hospitals—Children's Hospital of Philadelphia—that treated him as a child. Matt even has seen some of his former doctors while walking the halls, a sight he believes his physician never would have predicted.

Matt's earliest memories of his health extend back to elementary school where he simply remembers being different. His parents, Susan, formerly with the Delaware Valley Chapter of the National Hemophilia Foundation, and Arthur, who worked for Philadelphia Electric for 29 years, passing away in June from pancreatic cancer, took some of the standard precautions for a child with hemophilia to help keep their son safe. From kindergarten to third grade, Matt wore a helmet and knee pads, almost all the time when at school or playing, though he characterizes it as no big deal. "I had a lot of friends from an early age that accepted it," he says. Even when Matt was forced to use a wheelchair at school due to his ankles—his joint most prone to bleeds—his classmates clamored to volunteer to push him around for the day.

His folks helped make it easy for the students as well, "My parents used to come to class and demonstrate what hemophilia was," Matt says. "My parents broke it down into kid terms, explaining why I wore a helmet and what infusions were." Matt's parents created a presentation that turned each factor into a construction worker, each with his or her own, unique job to complete. However, construction worker number eight was always missing, Matt has Hemophilia A, so, they explained, he needed to add Factor VIII by infusing. Susan and Arthur also involved their friends and community in Matthew's illness. Susan recalls the time when Matthew was little and using cryoprecipitate, in which many of Arthur's coworkers donated plasma at one of three centers within an hour of their home.

While many cases of hemophilia are hereditary, Matt's diagnosis came out of the blue for his parents, who have no family history of the disease and whose older son does not have the disease. Deemed a random mutation, his parents came home from the hospital and looked hemophilia up in a dictionary, but were still unsure about what it actually was. To see what they thought was their healthy baby boy end up in the Neonatal Intensive Care Unit (NICU) right after he has born was frightening, but, Matt says they took it on as a challenge and vowed to make their life as normal as possible.

Matt has needed to replace his Factor VIII his whole life and today usually infuses three times a week. Recently, he's had problems with his left elbow, so for several months now he has infused every day. The medication Matt uses allows him to complete the five-minute infusion at home, but he could do it remotely given that the medication does not need to be refrigerated.



Matt's late father, Arthur, made up stories about hemophilia to educate his classmates and teachers about his condition.

While Matt does need to be careful, having hemophilia is not a huge interruption in his life, especially with the therapy he is taking. "When I was younger, it was completely different," he adds, explaining how he needed to push 60CCs of factor, which would take upwards of 15 minutes. Now, the constitution is just six (mls), and Matt credits advances in manufacturing and technology for bringing about this change.

As a child, he was limited not being able to play football or hockey, but Matt maintains that his parents were good at figuring out what worked for him. Matt played soccer and t-ball when younger and then starting swimming when he was eight, continuing throughout high school, where he also picked up golf.



As a child, Matt wore protective head gear so that he could safely engage in normal activities.

Matt admits that his family did have to adjust, “but, overall we tried to have as few interruptions as possible.” When the family visited Disney World, they needed to make arrangements with the hotel to store his medication on dry ice, but Matt credits his parents with doing a great job of not letting his hemophilia hold them back from anything and they’ve learned to adapt. He adds that they taught him to manage his disease at a very young age, enabling him to learn the importance of taking care of his health and allowing him attend college and live on his own in a dorm. Susan, calling Matthew a “character” recalls that he was able to infuse himself by the time he was six or seven, and demonstrated a bit of a capitalist spirit by charging neighborhood kids \$.25 to watch until his brother, Patrick, informed his parents of his sibling’s entrepreneurship.

In high school, Matt volunteered with the ambulance squad, going on calls, learning first aide and becoming CPR certified, which is where he caught the medical bug. After earning a degree in psychology, Matt went on to become a registered nurse after completing a 14 month course for his Bachelor’s degree in nursing. Having a self-proclaimed love of the medical field, Matt now works several 12 hour night shifts a week in the hospital’s Level I trauma center.

Matt says he’s never had to leave a shift because of bleeding. Far from holding him back, he says he’s had

opportunities that others don’t. Frequently, he’ll work with children with Hemophilia or other chronic medical conditions that come into the emergency room. “To connect on some level with those families is pretty neat.”, he says. Matt adds that some parents never think their child will be able to do anything, so in that sense he is a role model. He’s also able to empathize with what the family and patient are feeling when they are faced with a serious medical illness.

Matt’s also been able to give back to the hemophilia community through volunteer work with the Delaware Valley Chapter of the National Hemophilia Foundation, giving the occasional speech and stuffing envelopes. For five summers during college, Matt also worked as a counselor at the Double H Ranch, a Hole in the Wall Camp in upstate New York funded by actor Paul Newman for children with chronic illnesses such as HIV, hemophilia, sickle cell anemia and cancer. As a child, Matt attended the camp, which he describes as a, “really cool experience,” and exploration was encouraged (and supervised under the watchful eye of doctors and nurses). For starters, children at the camp can participate in a ropes course and use a zip line, two activities he was not allowed to do as a kid. The kids also visit an amusement park, work with arts and crafts and, of course, roast marshmallows around the campfire.



Today, with the benefit of his plasma protein therapy, Matt is able to play some sports and take part in athletic activities like horseback riding.

Next spring, Matt will be one of the keynote speakers at the Plasma Protein Forum, June 17-18 in Washington, D.C. One topic Matt is poised to discuss is just exactly how his life works, along with the daily challenges of growing up, versus where he is today with the advances in therapy. He says the medication has just gotten better and better and that he can’t imagine what new advances will come in the next 10 years. “Really, nothing holds me back now.”

For more information on the Plasma Protein Forum, please go to PPTA’s website at: www.pptaglogal.org

Donor Stories: Hans Jörg Bacher and Karin Schneeberger



My name is Hans Jörg Bacher. I am married and a father of two children.

I have been working as a public prosecutor for 10 years, three years in youth criminal cases, five years in drug and addiction cases. Since 2006, I have been dealing with general criminal cases and child pornography.

I am constantly asked why I still find time to donate plasma despite my stressful job. To explain, I'd like to share with you my personal story, a story that left a lasting mark on me.

My sister is a nurse and worked for a while on an immunology ward in a Graz clinic. There she met two young men who suffered from hemophilia. During a blood transfusion, they were infected with the HIV virus. A deep friendship grew between my sister, me and the two men. And through this friendship I started to give serious thought to this disease myself.

When I learned, for example, that life-saving therapies are made from plasma, I started to look into the possibility of donating plasma. I also encouraged my sister to do something about it.

That is how I decided to become a plasma donor. For me, giving plasma once a week has become a matter of course. I have been coming to one of the plasma centers in Graz for over four years now and enjoy coming here. The donation process is handled professionally by a highly qualified and very friendly team, as, for me, giving plasma is a matter of trust.

Whether children with genetic diseases, victims of accidents or cancer sufferers, all have a right to the best medical care. If I can do something to help, then I will, and for as long as I can.



My name is Karin Schneeberger. I am married and have one daughter and one son.

I have been a plasma donor for some years after being prompted to do so by a serious illness in my own family.

In 2000, when she was just ten, my daughter Jacqueline fell ill with a brain tumor. She spent two months in the hospital, and underwent seven operations as well as radiotherapy and chemotherapy. Even after leaving the clinic, she had to continue to visit for a long time as an out-patient and still has to go for regular check-ups.

Today, Jacqueline is a healthy young girl. She is 17 years old and is in the third year of her apprenticeship.

During her treatment, my daughter was prescribed a great many drugs, including some that were made from plasma. However, at the time, I didn't think twice about where the drugs were actually coming from.

In 2002 I read in an informational brochure on the subject of plasma donation that life-saving therapies are made from plasma, including therapies used to treat cancer



Jacqueline 10 years old.



Jacqueline today.



Jacqueline after her surgeries.

sufferers, that cannot be produced artificially. I decided to start giving plasma myself because of my own personal experience but also after meeting other ill children during Jacqueline's time in hospital.

I'm happy to say that a friend of mine also decided to donate plasma after I mentioned it, in order to do her bit.

So for the past five years I have been coming almost weekly to one of the plasma centres in Graz in order to help people in life-threatening situations with my donation. I am welcomed each time in a very pleasant atmosphere by qualified and friendly staff and actually enjoy coming along to each donation session as, over the years, you tend to make friends.

I think that it is only natural that I should donate plasma regularly, for as long as I am able to, first of all as it is my way of saying thank you and secondly in order to help seriously ill people receive the best possible treatment, just like my own daughter.

The Source wants to thank Maria Fradler for submitting these two stories.

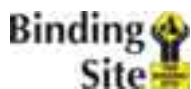
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 Dr. Johannes Blümel, Biotech Working Party, United Kingdom
 Prof. Rainer Seitz, Paul-Ehrlich-Institut, Germany
 Prof. Rudolf Bliem, University of Vienna, Austria
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 Dr. Georg Göstl, Baxter BioScience, Austria
 Dr. Rudolf Meixner, Humanplasma, Austria
 Dr. Martin Bezdekovsky, Fenwal, Austria
 Dr. Peter Pustoslemsek, Biotest AG, Germany
 Mr. Brian O'Mahony, Irish Haemophilia Society, Ireland
 Mr. Karl Freese, European Commission, DG Sanco
 Dr. Hubert Hartl, European Health Consortium, Austria
 Mrs. Kerry Fatula, Western Pennsylvania Chapter of the National Hemophilia Foundation, USA
 Dr. Adam Wanner, Alpha One Foundation, USA
 Dr. Phil Wood, St. James Hospital Leeds, United Kingdom
 Dr. Rajiv Jalan, London Medical School, United Kingdom
 Dr. Mark Weinstein, FDA, USA

Organised by:



Tuesday, March 4, 2008

- 09:00 - 10:15** **SESSION 1: Keynote session**
Keynote speaker
State of the Industry
- 10:15 - 10:45** **BREAK**
- 10:45 - 12:30** **SESSION 2: Self-sufficiency: can we depend on our neighbors?**
To fractionate or not to fractionate: experience from Finland
To fractionate or not to fractionate: experience from Brazil
To fractionate or not to fractionate: experience from Australia
Challenges of contract fractionation
- 12:45 - 14:00** **LUNCH**
- 14:00 - 16:00** **SESSION 3: Regulatory: what's up in Europe?**
Variations - the new European Commission proposal and how it would relate to plasma products
Variations - is there hope for relief?
Existing and emerging pathogens
Clinical evaluation: the Blood Products Working Party
- 16:00 - 16:30** **BREAK**
- 16:30 - 17:45** **SESSION 4: Quality in the 21st century**
Principles of the GMP
Regulator's perspective on GMP regulation
Manufacturer's perspective & experience on GMP regulation
ICH - risk and science based approach
- 17:45** **CLOSING REMARKS**
19:00 **Hilfenhaus Award Cocktail Reception**
20:00 **Gala Diner**

Wednesday, March 5, 2008

- 08:30 - 10:15** **SESSION 5: Availability of plasma for fractionation in the future**
Impact of the new blood transfusion developments and availability of recovered plasma
How to improve source plasma availability - barriers and removing them
Opening a new plasma centers - a global perspective
European approach to plasma for fractionation
- 10:15 - 10:45** **BREAK**
- 10:45 - 12:00** **SESSION 6: Health Policy: how can the EU make a difference**
Current European patient access issues
Rare diseases
Report on communicable diseases in Europe
European Commission view
- 12:40 - 14:00** **LUNCH**
- 14:00 - 16:00** **SESSION 7: Established products, new indications, great future**
Living with hemophilia: the need for access to a choice of factor concentrates
COPD
Optimal therapy and early diagnosis including cost benefit
New developments in the use of albumin
Variations in international clinical practice
Immunoglobulin, treatment of Alzheimers?
- 16:00** **Closure of IPPC 2008**

To Build Or Not To Build?

To build or not to build? . . . a fractionation facility – that is the question (Apologies to the Bard)

By Charles Waller

This article briefly considers the questions that governments, and potential international funding agencies that could finance the building of the manufacturing plant, should ask themselves when considering whether to build or to provide the funding for a plasma fractionation facility. In this article we will look at a number of aspects of this issue, including: cost effectiveness, ethics, patients' interest, self-sufficiency and global experiences that provide helpful guidance.

To paraphrase the Spanish philosopher George Santayana, those who do not, or who refuse, to learn from history are doomed to repeat it. Although initially coined in terms of governments' and generals' approach to warfare there is a lot of relevance to the debate over building a fractionation facility.

Trends and experiences this millennia provide a good basis for better decision making. It is known that several countries are on the cusp of a decision: should we go on depending on imported plasma proteins or instead should we become self-sufficient, rely on our own resources and build our own manufacturing facility?

To a politician, the answer to this question seems like "a no brainer" – it's obvious. Even if it costs a lot, even if our country will have to go on financially supporting the facility for a long time into the future we just can't trust foreigners to help us meet our fellow citizens' needs. The enormous costs can be defended on the basis of old fashioned nationalism – always a popular cause with politicians. It is curious then that plasma proteins are the only pharmaceuticals where politicians and ministries of health have this debate. There is very little debate regarding the dependence on "foreigners" to meet the demand for vaccines or other medicines in the contemporary doctor's "tool kit". Why is this?

Big factors are reality and money. The scale is completely different. Vaccines typically have a direct relevance to the whole or at least a substantial percentage of the population – as part of the public health agenda. As such, the associated costs may not be in the range of millions, but in tens or even hundreds of millions of Euros.

Plasma proteins on the other hand, are used to treat relatively rare conditions – the scale of production is, as a result, relatively smaller and the sums of money needed are significantly lower. Simplistically, a fractionation facility seems to be "affordable" as a one off investment. It is here that the problems tend to start.

In the vast majority of countries there never was a national vaccine manufacturer lobbying for financial and political support and new investment in the latest technology. The production of plasma proteins, on the other hand, has evolved from the need for blood and blood components for transfusion. In Europe in the early years of producing labile blood components, national "serum" institutes developed a capability to produce plasma proteins. There was a national stakeholder advocating for more resources. Over time, the larger ones, those that were more successful at advocacy, thrived; the smaller ones fell by the way side and were replaced in different ways.

Box one summarizes how this process has developed in Europe since 2000. National manufacturing facilities in Denmark, Finland and Scotland have stopped producing their own plasma proteins and this capacity has shifted to different producers. Two other countries, who shall remain nameless here, have accepted tens of millions of Euros to build fractionation facilities. Despite the fact that the money was all spent, neither facility will ever produce a gram or an international unit of anything. The details are irrelevant here, but these real cases demonstrate the need for tight controls and the importance of getting sound advice from skilled, competent professionals and not just by those who stand to gain from the project going ahead.

Box 1

European Union Member States / Status of Fractionation arrangements	
<p align="center">Government/not for profit fractionator</p> <p>In each of these countries the market is open to all organizations whose medicinal products are approved for use in the country and therefore that fulfil the national quality, safety and efficacy requirements.</p>	<p align="center">No national state owned fractionator</p>
<p>France* Luxembourg, jointly with Belgium</p>	<p>Austria, Cyprus, Czech Republic, Estonia, Germany, Greece, Hungary**, Ireland, Italy**, Latvia, Lithuania, Malta, Poland**, Portugal**, Slovakia, Slovenia, Spain**, Sweden</p>
<p>The Netherlands - Jointly with Belgium</p>	<p>Denmark - formerly national fractionator</p>
<p>UK: Eng - Using US, PPTA member plasma</p>	<p>Finland - Collaboration with the Netherlands / Belgium</p>
<p>* Limited access</p>	<p>UK: Scot - Using imported plasma, fractionation plant closing</p>
	<p>** Restrictions apply to national plasma</p>

YIELD CONSIDERATIONS

Making full use of the donated plasma is part of a commitment to donors not to waste or under use what they have donated. Therefore, the demise of these smaller producers has been an appropriate evolution. Those older, small facilities required significant investment of public funds to keep track with the developing state-of-the-art. They also lacked the capacity to establish the critical mass needed to ensure contemporary safety and high quality production in a sustainable way.

More importantly, these older facilities simply were not making optimal use of the plasma available for fractionation. For example, recovering less than two grams of immunoglobulin (IG) from each liter of plasma simply resulted in a loss of more than 50 percent in the quantity of IG available for patients than if the plasma was fractionated in a state-of-the-art facility where a yield of four or more grams per liter is a realistic and achievable goal. In conclusion, it is not only financially unsustainable, it is irresponsible.

The greater efficiency, extracting the maximum possible yields, is the result of substantial experience, sustained investments over years and the research and development that comes from years of commitment.

History shows that governments that take a hard critical review of fractionation have come to a similar conclusion. By 2007, the number of countries with their own production capability has declined to only four in Europe, more than halving the number of government owned facilities of ten years ago.

We can learn a lot from this experience which is also confirmed by trends in the private sector.

IT'S A MATTER OF SCALE

If governments choose the route of subsidization or in some other way reducing the manufacturers' true costs, these domestically subsidized or protected organizations should not be allowed to compete in international markets as this presents a breach of international trading rules that exist to protect the consumer.



PATIENTS' INTERESTS

Curiously, the potential users of the fractionated plasma proteins – patients and doctors - are usually excluded from the decision making and are only rarely consulted on questions of whether to fractionate domestically or not. Typically, nationally fractionated products become the dominant products available in the country. On the one hand, this seems good news as these self-sourced products provide a secure percentage of the national need. On further investigation, however, there are good patient and physician-oriented reasons why most countries have chosen not to have their own national fractionation facility. Patients and physicians tend to prefer a diversity of products. From experience, they know that the production of biological medicinal products is a sensitive manufacturing process and disrupted supply must be expected for any production facility. With only one producer, a dominant market supplier or only two or three producers exposes patients and physicians to unnecessary risk.

COMMERCIAL CONTRACTING

The best route of ensuring a sustainable supply of plasma derivatives at the best prices is to engage in normal contracting that is as close to the end user – the patients and physicians - as possible. Health authorities that contract in this way can be confident that what they have ordered will be delivered. If the inbuilt challenges of producing biological medicines cause a disruption in supply then it is the responsibility of the manufacturer to find an acceptable alternative and to bear the cost of any difference in price.

CONTRACT FRACTIONATION

If governments or health services, like the majority, choose not to build a fractionation plant but nonetheless want to meet the clinical needs of their fellow citizens from nationally collected blood or plasma then what are the options. The first question relates to the quality of the plasma to be fractionated. If it meets the high standards required by regulators in most developed countries then they can consider “contract fractionation” and come to an agreement with a competent manufacturer.

Most PPTA members have extensive experience in contracting successfully with governments, or health services and providing state-of-the-art safety and product yields. In fact, it is these two aspects that challenge national fractionators that lack the size and political support to finance the necessary developments in research and development. Reliable and sustained production will help ensure that patients and physicians can have confidence in the manufacturer and the sustainable access to the therapies they need.



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Third World Congress of Alpha-1 Antitrypsin Deficiency Patients

By Johan Prévot

PPTA staff attended the Third World Congress of alpha-1 antitrypsin deficiency (AATD) patients which was held on September 28-30, 2007 in Rome, Italy. The Congress was co-organized by the Alpha-1 Foundation Europe and the Italian Alpha-1 Association and was attended by approximately 150 participants. The chairpersons of both organizations, Larry Warren and Nuccia Gatta, formally opened the Congress. (Alpha-1 Foundation Europe also announced that they will soon launch their new website.)

The welcome address was followed by presentations from national alpha-1 associations, that provided delegates with an update on the objectives and challenges facing each association. Interestingly, the Dutch Alpha-1 Association represented by Benjamin Wesseling encouraged other national associations to seek funding from larger lung diseases associations. He advised that the Dutch Alpha-1 Association is primarily funded through the national lung disease association. At the end of the session Ms. Gatta and Mr. Warren invited John Walsh, President of the U.S. Alpha-1 Foundation, to join them on the stand. Mr. Walsh was presented with an award for his long-lasting commitment and leadership for the Alpha-1 Community worldwide.

Day one of the Congress closed with an excellent overview on AATD entitled "Where We Are, Where Are We Going To" by Prof. Maurizio Luisetti from the University of Pavia in Italy. Prof. Luisetti reviewed the history of the condition from its discovery in Sweden in 1963, by Laurell and Eriksson to the present day. It was pointed out that the highest prevalence of the disorder is amongst people of Northern European origin and that alpha-1 patients have a higher risk of developing Chronic Obstructive Pulmonary Disease (COPD) and liver disease. Awareness of the condition has improved significantly Prof. Luisetti pointed out with the help of national registries and national and international patient organizations. Although the epidemiology of the disorder is now well known, there are still many questions which remain unanswered, such as the definition of the exact ratio between asymptomatic and symptomatic patients affected by AATD.

The second day of the Congress opened with a presentation by John Walsh on patient empowerment. Mr. Walsh emphasized the three main challenges for the alpha-1 community: lack of awareness, lack of resources, and access to therapies. In order to tackle these issues, stakeholders, be they patients, physicians or industry, must work together. Mr. Walsh stressed the importance of 'seizing the moment' and latching on to the current COPD movement for the alpha-1 community. COPD affects 600 million people worldwide, 3-4 percent of which are alpha-1 patients, and is currently being tackled by organizations such the World Health Organization and COPD coalitions. It is therefore important for the alpha-1 community to make their voices heard and emphasize that COPD is not only linked to smoking but also is a genetic disease. Lastly, Mr. Walsh insisted that "the more aware, the better the care."

The remainder of day two boasted excellent presentations by a wide range of renowned specialists, who explored topics such as the pathogenesis of liver injury and lung disease in AATD, the assessment of lung function, the natural history of AATD in childhood and an assessment of the risks to develop lung disease for heterozygous AATD patients. The need for standardization of diagnosis through the systematic testing of all symptomatic adults with





emphysema, COPD, or asthma with incomplete reversible airflow obstruction, as well as individuals with unexplained liver disease and asymptomatic individuals with persistent pulmonary obstruction, was reviewed along with topics such as the importance of screenings, the role of registries and patient involvement. In a presentation on ongoing research and developments by Prof. Robert Stockley (Queen Elizabeth Hospital, Birmingham, UK) and Prof. Mark Brantly (University of Florida, U.S.A.), the importance of smoking cessation and prevention were highlighted. It was also emphasized that research in the field was opening new doors for AATD treatment through the development of AAT augmentation therapy, gene therapy, gene correction, small molecules based treatment approaches and new aerosols. The rest of day two was dedicated to parallel workshop sessions on oxygen therapy, life coping issues and rehabilitation, and nutrition.

The last day of the Congress comprised an early morning session, including presentations on the conventional treatment of AATD, augmentation therapy and its development, novel therapies for AATD and non-pharmacological treatment of lung disease such as pulmonary rehabilitation, lung transplantation and lung volume reduction surgery. The Congress closed with an 'arrivederci' by Mr. Warren and Ms. Gatta. Patients wishing to attend were then transferred to St. Peter Square for an audience with Pope Benedict XVI.

Interview with Professor Rainer Seitz

The views expressed in this article are those of Professor Seitz and do not necessarily reflect the official position of the European Medicines Agency (EMA).

By Ilka von Hoegen

The Chairman of the Blood Product Working Party (BPWP), European Directorate for the Quality of Medicines & HealthCare (EDQM) Expert Group 6B, and Head of Hematology/Transfusion Medicine at the Paul-Ehrlich-Institute sits down for an interview with PPTA

1) Over the years the Blood Product Working Group (BPWG) has established a fairly complete set of guidance documents on plasma proteins. If the major task becomes reviewing and updating the existing guidance documents, would you like to add other responsibilities to the work plan of the BPWG?

First of all, it is important to note that the name of the group has been changed from the Blood Product Working Group (BPWG) to Blood Product Working Party (BPWP), which is a promotion to a more permanent group which shows that the EMA considers the group very valuable and important. It is correct that there is already a set of good guidances and the question is absolutely justified in terms of how to go on.

Increasingly, we have to give advice to the Committee for Medicinal Products for Human Use (CHMP) on blood products and also with regard to routine regulatory procedures like marketing authorization or scientific advice procedures. Secondly, there are overarching topics like neoantigenicity, a topic, which is interesting for all the biologicals and particularly for the recombinant products. Obviously we already have some experience due to the Factor VIII inhibitor issue. Therefore, we can contribute significantly to such overarching topics.

Furthermore we have links to the Pharmacovigilance Working Party, and also the Biological Working Party, so I think we will not get bored.



Professor Rainer Seitz

With respect to new topics, there are a lot of new products around the corner such as recombinant products, transgenic products and even gene therapy in the field of hemophilia. It is not really the expertise of this group to deal with gene therapies but we have to give some input to the clinical assessment of such new therapies because BPWP has the experience with the indications.

2) Where do you see the main focus for the BPWP in the near future?

We have each year a work plan which is published far in advance where you can see what we are going to do and at least in the mid-term we have some challenging topics to address, such as the gene therapy. There are always new developments, and in general the environment for clinical studies is changing, which is still a challenge not only for authorities but also for the industry. There is still a lot to do to establish new standards also for clinical studies. For instance we are just revising the guideline for factor VIII and factor IX, which are, of course, very important

guidelines. I am sure we will have very good discussions in the near future on that.

3) Where do you see the main focus regarding cooperation with industry?

From my point of view, the status quo we currently have is not so bad and actually very satisfying, but of course there is always room for improvement. The main issue is that regulatory procedures contribute to the workload and are a challenge for both the authorities and the industry and we are both interested in smooth and effective procedures. The authorities are not interested in stalling product development; we are interested in having good products on the market, which is really a common interest. We should commonly do our best to make the procedures smooth and effective. Therefore, we are very much interested in good communication. There are certainly things which have to be dealt with bilaterally with single companies, but on the other hand there are a lot of general questions which we can discuss with the industry associations.

We have had meetings with industry and further meetings are envisaged. Next February, we will have a stakeholder meeting on the new Factor VIII/IX guideline and the new intravenous immunoglobulin guideline, which will certainly be very interesting. We will have to gather the patient associations and the industry associations and I am really looking forward to these discussions because they are usually very fruitful and interesting. I would like to add that we are always open for suggestions and ideas from industry. Of course, we cannot accept every idea submitted by the industry, which is only natural for a relationship between industry and authorities, but nonetheless we are always open for discussions and good proposals and the better the data and the arguments the more we will be able to come to consensus.

4) Where do you see potential for improvement?

Maybe I can turn the question around: If you have some points which are really difficult for you to deal with, please let us know. We can always learn and improve and my

message is that we are really committed to smoothing and streamlining the procedures to reduce the workload for all and have the best result.

5) PPTA member companies frequently encounter difficulties because of different interpretation of European legislation and guidance. What is your experience with different Member States in this respect?

The European Community is a very complex and complicated construct and it is still in a dynamic process of integration and development and naturally, this is a difficult process. But also in this case much has to be done to make this possible and feasible. Some years in advance of the enlargement with the Central and Eastern European countries, the candidate countries have been invited to trainings. A lot of things had been done to prepare them for joining the Community and my impression is that they are doing a good job. You have to imagine that they have to build up and establish a lot of capacities. In our BPWP, we have very good and competent colleagues who are really interested in communicating and they are committed. But I think it is in the nature of the European Community that there is a period of transition that may be difficult from case to case and we must not forget that all Member States are sovereign countries with their own plans and attitudes. But in case you encounter difficulties, there are a few mechanisms you can use, for example, raise them with EMEA. The EMEA is, as you know, responsible for the Notes for Guidance and centralized products. There is also a mechanism for the mutual recognition and decentralized procedure, with a link between the EMEA and the Coordination Group for mutual recognition and decentralized procedures (human) (CMD(h)), which is actually meeting at the EMEA premises. Thus, if there are problems with the interpretation, there are mechanisms to address them. It may be a very delicate issue, but once a text of a guideline is adopted it should be the consensus among the Member States. To some extent, these problems are inherent in the nature of the European Community which is a community of sovereign states, where consensus has to be developed. There will always be cases where opinions are different and where compromises have to be found.

6) Is it more difficult to find consensus now with 27 Member States?

It is challenging in the CHMP where there are indeed 27 Member States around the table. In the other working groups, there are not necessarily that many participants. Also in the BPWG we are not really 27 people since not every country is sending an expert. But in the CHMP groups there are several representatives of the new Member States and my impression is that they are very competent, they listen and they are also very constructive. In the BPWG, I have the honor to lead; there has never been a problem to find a consensus.

7) Plasma protein therapies are not part of the International Conference on Harmonization (ICH) work plan, although certain EMEA guidances such as 268/95 are adopted as ICH documents. Would you support that plasma protein therapies are part of the ICH work plan?

To my knowledge the guidance 268/95 is not an official ICH guideline, but nonetheless it is a very influential guideline which is really respected around the world. The ICH process is very demanding and it is even more difficult to find consensus within Europe, because there are really different worlds coming together which necessitates tremendous efforts. In my view, the ICH covers more fundamental and overarching things rather than special product groups. I am not aware of special guidelines for vaccines or other product groups. On the other hand, I really try to promote international harmonization and we have already some mechanisms. I would like to mention e.g. the European Pharmacopoeia (Ph.Eur.) which is not only composed of EU Member States. The Council of Europe goes much beyond EU territory. We also have the group of experts 6B where we have international observers, from Australia, Canada, Israel, from the U.S. Food and Drug Administration (FDA), the World Health Organization (WHO) and the EMEA. This is a very important link and it might be an advantage that the chairman of the group of experts 6B (*editor's note: Expert Group on Human Blood and Blood Products*) and the BPWG is the same person which makes some things easier. I have the impression that at the level of authorities there is also some common interest in harmonization. The difficult part

to achieve a common platform is really on the high political level. On the authority level we really try to harmonize the procedures. If you have special issues where you need harmonized approaches, because otherwise it really causes problems, it is always possible to bring it up. Of course, it is always better to present it with supporting data. One example I would like to mention, is the freezing temperature of plasma for fractionation which was a never ending story for many years. Then industry presented data from their studies and indeed the monograph changed. This is a good example and since we have observers from the FDA and elsewhere in the group 6B this might have some effect really beyond Europe. At least in the area where we have responsibilities we can try to come to progress. However, we are authorities and we depend on the legislation and the political guidance.

8) You are Chairman of the BPWG, Chairman of the Group 6B, Head of Hematology/Transfusion Medicine at the Paul-Ehrlich-Institute. How do you handle all those responsibilities?

It is really a challenge, but the good thing is that I have a very good team here at the Paul-Ehrlich-Institute and also a very good working climate in the EMEA and in the European Pharmacopoeia. So I am happy with that. Still I can cope with all my duties and I try my best.

PPTA PLASMA PROTEIN FORUM 2008



17 - 18 June 2008
AT THE MARRIOTT IN WASHINGTON, DC

Minnesota State Senator Kathy Sheran Weighs in on Quality of Care Legislation



Senator Sheran is the Senate Co-Sponsor of Senate Bill 2290 in Minnesota focusing on quality of care for consumers of plasma protein therapies. Senator Sheran is a Freshman State Senator with a background as a registered nurse. Since taking office, Senator Sheran has distinguished herself in becoming Senate Majority Whip and Vice Chair of the Senate Higher Education Budget and Policy Committee. She also recently received the prestigious award of Freshman Legislator of the year from the group, Politics in Minnesota.

1) Describe your interest in health care legislation.

My life work as a nurse has been in health care. I am committed to public health and the prevention of long-term and chronic illnesses. At the same time I believe those with chronic health problems should be supported in a manner that maintains their highest level of wellness. This means treatments must be science based, timely, and in an environment that reduces further health risks for the consumer.

2) In your mind, why is it important that individuals have access to the full range of plasma protein therapies at the appropriate site of care?

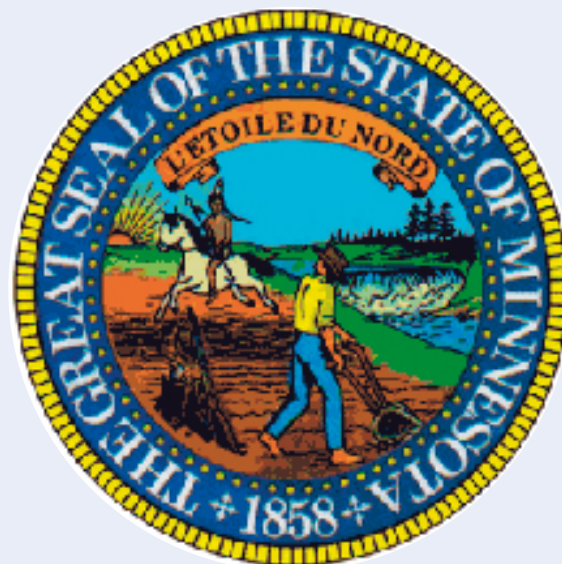
My interest in allowing individuals access to a full range of plasma protein therapies in the safest environment is to reduce the likelihood of adverse reactions which further stress the health status of the individual, and create unnecessary health risks and costs.

3) What are some of the challenges you see in Minnesota?

Challenges in Minnesota are typical. The industry does not like regulation, prefers flexibility, and is unaccustomed to thinking about how patient centered care will reduce costs, and is in the best interest of health care reform as well as the health of the individual. The legislature also has become resistant to regulating the industry due to its concerns about the inflating cost of health care. Getting support for what appears to be more expensive health care at a time of health care cost inflation is a challenge.

4) What suggestions can you offer to the patient community and PPTA to get their message across?

PPTA and the community of patients need to make the case for why the public should take an interest and should support this proposal. Some questions that will need a persuasive and educated response are; why is the current approach unacceptable, why should government intervene, and how can it be justified in an environment in which health care costs are already inflating without additional expectations and requirements.



What Do We Have in Common?



Plasma Protein Therapies Saved Our Lives

The Plasma Protein Therapeutics Association works tirelessly to ensure patient access to critical, life-saving therapies that give patients who lack a specific protein or antibody that should exist in their plasma, the replacement therapies they need to survive. People who benefit from infusions or injections of plasma protein therapies suffer from chronic and acute, often genetic conditions.

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Plasma Protein Therapeutics Association

SAVING AND IMPROVING LIVES

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Meet PPTA Staff: Michelle Mason



Background

My name is Michelle Mason and my title is Associate, Regulatory Policy & Source.

I've worked at PPTA for five years. I grew up in Chestertown, Maryland, not far from PPTA's offices in Annapolis. My husband, Michael, and I have been married for eight years and we have a beautiful daughter, Julia, who is 16 months old. I am an aunt to five nieces and one nephew.

Tell us about your background?

After graduating from high school, I attended a business school and received a small business management certificate in 1997. At my previous job, I organized community outreach activities within the company, such as Habitat for Humanity, Angel Tree, and food drives for local shelters. I also planned and organized company events.

What do you do at PPTA and what do you enjoy most?

My responsibilities have grown since I joined PPTA in 2002. I assist the staff in both the Regulatory and Source divisions of the Association, and service our membership. What I enjoy the most, aside from working for the Source division's Vice President, Joshua Penrod and the Regulatory division's Vice President, Mary Gustafson, is planning various meetings, such as the annual Source division

planning meeting, Source Business Forum and the U.S. Food and Drug Administration (FDA) liaison meeting, in addition to the Global Board/Steering Committee/Task Force meetings that occur throughout the year. This provides me with an opportunity to interact and get to know PPTA's members better. I have also taken on the task of managing the exhibits and sponsorships for the annual Plasma Protein Forum, which will be held in Washington, D.C. on June 17-18, 2008, which has been both challenging and rewarding.

What is most rewarding about working in this industry?

I enjoy working at PPTA, especially because of the family atmosphere that we have. Working at an association that has members that produce life-saving therapies is rewarding, especially when hearing patient stories and how they have been helped.

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News

Leaders in the House of Representatives, Energy and Commerce Health Subcommittee, informally met to discuss follow-on biologics and to work on contentious issues such as the length of time afforded for the exclusivity of brand name biologics, but because of other pressing items on the end-of-the year agenda, the committee members stated that follow-on biologics will have to be addressed in the next session commencing in January 2008. In the meantime, PPTA will continue advocating for a legislative exemption for plasma protein therapies, as recommended by the Food and Drug Administration (FDA). It is noteworthy that during this current congressional session, PPTA successfully lobbied to exempt plasma derived protein therapies from two bills, S. 1505 and H.R. 1956.

PPTA's French Working Group (AMDSA) recently held its second Stakeholders Round Table meeting in Paris, France. The Round Table was attended by industry representatives, patient group representatives, including the newly created French Alpha-1 Antitrypsin Deficiency Patient Organization (ADAAT), physician group representatives and hospital pharmacists. The first Round Table held in September 2006 resulted in several common actions, which led notably to the exemption of coagulation factors and immunoglobulins from the newly introduced national tendering system. This year, Round Table participants agreed a set of common actions amongst which a common position statement highlighting the need for a guideline requesting that all immunoglobulin brands available on the French market be listed in hospital pharmacies along the lines of the guideline (ref. DGS/DSS/DH 97/804) which requested that all available brands of coagulation factors be available in hospital pharmacies, on the basis that you cannot substitute them. The need to optimize the use of immunoglobulin within the hospital distribution network was stressed and a common outreach campaign specifically targeted at the Regional Medicinal Products Monitoring Centers (OMED-ITS) level was agreed upon. A meeting was requested with the French Authorities to discuss the French National Authority for Health's (*Haute Autorité de Santé* - HAS) interest in a post-marketing authorization study on subcutaneous immunoglobulins. It was noted that the parameters of the requested study are in contradiction with EU post-Marketing Authorization study protocol guidelines.

On Thursday, November 8, 2007, the U.S. Food and Drug Administration (FDA) published a proposed rule, "Requirements for Human Blood and Blood Components Intended for Transfusion or for Further Manufacturing Use." Among other proposed changes to the regulations, FDA is proposing to require two of the PPTA voluntary standards. Those are the qualified donor standard and the 60-day inventory hold. Comments on the proposed rule are due February 6, 2008, and comments regarding information collection are due December 10, 2007. PPTA staff will work with the Regulatory Policy and Compliance Steering Committee to prepare comments to the proposed rule. The proposed rule can be found at the following link: <http://www.fda.gov/cber/rules/reqbldtrans.pdf>

PPTA attended the Good Manufacturing and Distribution Practice (GMP/GDP) Inspectors working group meeting with interested parties on September 26, 2007 at the European Agency for the Evaluation of Medicinal Products (EMA) offices in London. The EMA Reflection paper on minor variation was discussed with respect to the level of discretion of the Qualified Person (QP) in decision making processes when minor deviations occur in the manufacturing process. Prior to the meeting, PPTA participated in the preparatory meeting of European Federation of Pharmaceutical Industries and Associations (EFPIA) where a joint position on QP discretion was developed. Industry strongly recommended revising the Good Manufacturing Practices (GMP) regulations to introduce a better definition of the role and responsibility of the QP. The meeting also addressed the introduction of the International Conference on Harmonization (ICH) Q10 pharmaceutical Quality System in conjunction with ICHQ8 Pharmaceutical Development and ICH Q9 Quality Risk Management. The overall aim of this initiative is to maximize benefits from innovation and continual improvements. ICH Q10 compliance and certification will add an additional voluntary layer to the mandatory requirements of GMP. PPTA stressed that before the principles of these ICH documents can be implemented, some provisions of the EU Variations Regulations for biological products have to be revised. The chair of the meeting agreed on this point.

The U.S. Food and Drug Administration (FDA) Amendments Act of 2007 was signed into law by President George W. Bush on Thursday, September 27. This legislation includes the Prescription Drug User Fee Amendments of 2007, the Medical Device User Fee Amendments of 2007, the Pediatric Medical Device Safety and Improvement Act of 2007, the Pediatric Research Equity Act of 2007, and the Best Pharmaceuticals for Children Act of 2007, as well as provisions creating the Reagan-Udall Foundation, addressing conflicts of interest on FDA Advisory Committees, expanding the clinical trial registry data bank, and enhancing post market surveillance of drugs, including post-approval studies, clinical trials and label changes, the implementation of risk evaluation and mitigation strategies, and a provision that calls for FDA to promulgate regulations to prevent counterfeit drugs and establish a national pedigree standard.

PPTA and several stakeholders in the plasma protein therapies community submitted joint comments to the Centers for Medicare & Medicaid Services (CMS) CY 2008 Hospital Outpatient Prospective Payment System (OPPS). PPTA and allied stakeholders made a concerted opposition to the proposed rule's provision that would cut in half the payment to hospitals for the IVIG pre-administration related services code G0332. The joint comments also adamantly oppose the proposed rule's reduction in payment for non-pass through drugs and biologics from Average Sales Price (ASP) plus 6 percent to ASP plus 5 percent.

PPTA submitted a position statement to the European Commission in response to the European Consultation on the Future of Pharmaceuticals for Human Use launched by the Commission in July 2007. The objective of the Commission's Consultation is to improve the regulatory, non-regulatory and Research and Technological Development framework for pharmaceuticals for human use in the EU. Specific initiatives in the field of pharmacovigilance and variations have already been announced. The Commission's broad consultation targeted all stakeholders that are dealing with medicines for human use, with the hope of gathering information and a better understanding of stakeholders' views on the key challenges of the sector, and determining how to tackle them. Key regulatory and patient access actions needed in the European Union for the plasma protein community were highlighted in the PPTA statement.

On Friday, November 2, 2007, the Centers for Medicare and Medicaid Services (CMS) published its CY 2008 Hospital Outpatient Prospective Payment System (HOPPS). As requested by PPTA, CMS will continue to provide reimbursement for IVIG preadministration-related services, however despite PPTA's objections CMS will only pay about half of the CY 2007 levels for the preadministration-related services in CY 2008. In addition, CMS will now pay for drugs and biologics with pass-through status, including plasma protein therapies, at a reduced rate in the hospital setting from CY 2007 levels of average sales price (ASP) plus 6 percent to ASP plus 5 percent.

PPTA staff and members of PPTA's French Group (AMDSA) met with the French Ministry of Health to discuss the report recently submitted by Prof. Rémy Pellet on the review of the French Marketing Authorization (MA) regime for blood products and plasma protein therapies. The report had to be submitted to the Ministry of Health earlier than planned, following a letter from the European Commission to the French authorities indicating that article L5121-11 of the French Public Health Code did not comply with EU legislation and requesting France to take necessary measures to adapt their marketing authorization regime in compliance with EU legislation. The letter from the Commission was triggered by the complaint lodged by PPTA against the discriminatory market access restrictive measures imposed by the French Public Health Code against plasma protein therapies produced from compensated plasma donations. The French response to the Commission is therefore expected to be built mainly around questionable ethical arguments rather than legal ones. In parallel, PPTA's French group has embarked on a lobbying campaign in France to challenge the Ministry of Health's current position.

Calendar of Events

March 4–5

International Plasma Protein Congress
Warsaw, Poland

March 16-19

International Conference on Emerging
Infectious Diseases (ICEID)
Atlanta, Georgia, USA

April 3-4

9th Annual NATA Symposium, Network for
Advancement of Transfusion Alternatives
Lisbon, Portugal

April 7-9

48th Annual Scientific Meeting of the British
Society of Hematology incorporating the 6th
Bi-Annual-BFM Leukaemia Symposium
Glasgow, Scotland

April 9–12

American Society for Apheresis 2008 Annual
Meeting
Galveston, Texas, USA

June 1-5

Hemophilia World Congress
Istanbul, Turkey

June 7-12

XXXst International Congress of the
International Society of Blood Transfusion
(ISBT)
Macao, China

June 12-15

13th Congress of the European Hematology
Association (EHA)
Copenhagen, Denmark

June 17-18

PPTA Plasma Protein Forum 2008
Washington, D.C., USA

June 19–22

13th International Congress on Infectious
Diseases
Kuala Lumpur, Malaysia

September 6–9

32nd Annual Meeting, American Association of
Tissue Banks
Chicago, U.S.A.

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 Immunohaematology
(DGTI)
Düsseldorf, Germany

October 4–7

AABB Annual Meeting and TXPO
Montreal, Canada

October 5

PPTA Source Business Forum
Montreal, Canada
By Invitation Only

October 16-19

XIIIth Meeting of the European Society for
Immunodeficiencies (ESID)
's-Hertogenbosch, The Netherlands

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**AsahiKASEI
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NORTH AMERICA

Asahi Kasei Medical America
Long Island Branch
1600 Stewart Ave., Suite 602
Westbury, NY 11590, USA
Tel: 877-752-6682 (toll free, USA & Canada)
Fax: 516-620-3135
planova_us@om.asahi-kasei.co.jp

EUROPE

Asahi Kasei Planova Europe
Paepsem Business Park
Boulevard Paepsem 22
1070 Brussels, Belgium
Tel: +32-2-526-0501
Fax: +32-2-526-0550
planova_de@om.asahi-kasei.co.jp

ASIA

Asahi Kasei Medical
Planova Division
9-1 Kanda Mitoshirocho
Chiyoda-ku, Tokyo 101-8482, Japan
Tel: +81-3-3259-5723
Fax: +81-3-3259-5725
planova_jp@om.asahi-kasei.co.jp

PPTA wishes you a happy holiday season

