A Vision for the Future
Plasma Protein Therapies Industry Leading the Way

College Student Karissa Ybarra Uses Experience With PIDD To Help Others

President Obama Signs Seminal Health Care Reform Legislation

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IN THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

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These companies have earned QSEAL certification. The PPTA QSEAL program is a promise to stakeholders worldwide, that the highest levels of care were invested into the production of these unique life-saving therapies. BAXTER BIOSCIENCE Biotest AG Grifols, S.A. Talecris Biotherapeutics CSL Behring Kedrion
IN MY VIEW

PPTA STAFF ENDURES COMPLICATIONS DURING RENOVATIONS,
But continues Excellent Service to Members

FOR THIS EDITION OF THE SOURCE, I want to focus on the good work that the PPTA employees are doing for all members, donors, patients and other stakeholders. I am very privileged to work with such a competent team of colleagues. Our team that works for you has various skill sets that we need. These skills are technical and also include language skills. We have people that speak English, French, German, Dutch, Flemish, Italian, Spanish, Japanese and Maltese. Those language skills enable us to participate in many meetings in the native language.

Our two offices are in Annapolis, Maryland in the U.S. and Brussels, Belgium in Europe. We also have a small satellite office in Washington D.C. All offices went through renovations last year and have resulted in professional and visually appealing working places, however...

Our landlord in Brussels notified our staff that the building was going to be renovated, that air conditioning was going to be installed and the inconvenience would be minimal. Nothing could be further from the truth!

An enormous scaffold was built that made it difficult to enter the narrow garage entrance. Workers took the parking places and driving in the garage was a challenge. One day an extra wall was built, without notice to employees, that made it even more complicated. Several car damages followed. Not to mention the many water leakages that made your car look like it was never cleaned.

In the office it was unbelievable. Because the front of the building was renewed, all windows needed to be taken out. Now a new wall was constructed that reduced the offices at that side by at least four feet, took away our meeting room and all the heating was disconnected. This all started in the fall and all winter staff needed to use electric heaters. The wall took away the light and some staff had to use extra floor lamps from home.

For many days the electricity went out, servers did not work, emails could not go in and out, and phone service wasn’t available. Only thanks to handheld devices were staff able to continue to work.

The drilling was phenomenal. One day, one person was trying to have a call when suddenly the outside wall was pierced by a drill that ended up in her office. Needless to say, having conference calls and regular calls were a challenge.

Many meetings needed to be held off site and the only quiet moments were at lunch at a local restaurant. There were many surprises. One time staff realized that the toilets were removed without notice, and the water supply was disrupted several times. On another day the ceiling in the bathrooms was removed giving open view to the workers on the floor above. Needless to say, the staff was not thrilled with this privacy intrusion.

The end is in sight. The landlord realized the impossible situation and offered to build a new office on another floor at their expense and according to the wishes of PPTA Europe. That office was ready in May.

I want to express my gratitude to the Brussels staff who worked under primitive circumstances for eight months, but never complained or reduced the service to our members. PPTA members are fortunate to have a great team working on your behalf in both Brussels and Annapolis.
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DR. ALAIN FISCHER WAS HONORED BY PPTA

with the prestigious Hilfenhaus Award at the 2010 International Plasma Protein Congress (IPPC) in Berlin, Germany, for his outstanding research in the area of pediatric immunology.

Named after Dr. Joachim Hilfenhaus, former head of PPTA’s Viral Safety Working Party who died in 1996, the Hilfenhaus Award recognizes individuals who have made contributions to patient access to safe plasma protein therapies.

The last two award winners were Prof. Jose-Luis Valverde, a well-known politician, professor, and publisher and Prof. Johannes Oldenburg, who was honored for his contribution in the fields of immuno-haematology and transfusion medicine and the treatment of people with hemophilia in Bonn, Germany – the world’s largest hemophilia center.

Dr. Alain Fischer studied biochemistry and pediatric immunology at the Universite Paris Jussieu and has been the director of a unit dedicated to “normal and pathological development of the immune system” at the National Health Institute of Medical Research (INSERM) since 1991.

In addition to his position at INSERM, he also serves as a professor of pediatric immunology at the Universite Rene Descartes and as a professor at the Institut Universitaire de France. Since 1996, Dr. Fischer has served as the director of the pediatric immunology department at Hospital Necker. His main fields of research are gene therapy, genetics of immunological disorders, primary immunodeficiency diseases and development of the lymphoid system.

In November 2002, Dr. Fischer was elected member of the French Academy of Science and he is a member of the National Advisory Committee of Ethics since 2003. In 2008, he received the INSERM “Grand Prix” for his career achievements. He has also received the Halpern Prize in 1984, the Behring-Metchnikoff Prize in 1992, the Prix du Comité du Rayonnement français in 1994, and the Jung Prize for medicine in 1998.

Kara Flynn is PPTA’s Director, Global Communications
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Roche’s cobas s 201 system is a reliable, operator-friendly platform with fast and easy daily start-up and minimal maintenance.

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In February 2010, Karissa Ybarra, had the chance to visit a plasma collection center for the first time and meet the donors who contribute to the immune globulin therapy that makes it possible for her to live the typical life of a college student. It was an eye opening experience for her to share her story with donors, most of whom had never met someone who they have helped through their plasma donations, and to express her thanks.

The 22 year-old student at Houston Baptist University in Texas has been fighting a series of respiratory and other illnesses since being diagnosed with an IgA deficiency at 15 months old and, finally, with common variable immune deficiency disease (CVID) in her early teens.

Today, Ybarra, from Deerpark, Texas, not far outside of Houston, is an active member of the Immune Deficiency Foundation (IDF), helping young people cope with their disease through participation as a Teen Council mentor she travels to Washington, D.C. to meet lawmakers and to advocate on behalf of individuals with immune deficiencies as part of IDF’s annual legislative day.

At 18 months old, Karissa had been diagnosed with an IgA deficiency, which made it difficult for her to fight infections.

Early Diagnosis, Childhood Laced with Hospital Stays

When Ybarra was 15 months old, she was diagnosed with an IgA deficiency, one of the most common primary immune deficiency diseases (PIDD) that makes individuals susceptible to infection. Her mother describes her first year as one spent always sick and always in the hospital. When she was just over a year, Ybarra had become so ill with a respiratory infection that she was admitted to the neonatal intensive care unit (NICU) on a ventilator at Texas Children’s Hospital in Houston. Finally, one of the doctors insisted that they would not leave until they knew what was the cause of the infection. Several different doctors were consulted including allergy and immunology specialists, who finally discovered the deficiency in her immune system.

Ybarra says that there was not much doctors could do for her IgA deficiency and she was constantly on antibiotics and suffering from skin and ear infections and respiratory illnesses. By the time she had reached her early teens, the illnesses became...
more frequent, causing her to miss school. Her illness eventually landed her in the hospital for a month when she was 15. Doctors eventually discovered that her IgA deficiency had progressed and diagnosed her with CVID. At that time, Ybarra began her first round of monthly intravenous immune globulin (IVIG) infusions at the hospital’s allergy and immunology clinic that she continued until she was about 18. Now Ybarra takes weekly Ig injections that she has been taught to administer on her own at home.

After a few weeks on the IVIG, Ybarra began to notice a positive change in her health and after two months, she stopped having recurring infections. A simple cold no longer turned into pneumonia, and her body could fight off illness.

### Time as a Patient May Transform into Work as a Care Giver

Ybarra is now in her fourth year at Houston Baptist University, living off campus with the ability to administer her therapy at home. Luckily her roommates, whom she has known for years, take everything in stride—spending time with her during her infusions, which can take several hours. She is majoring in psychology and child development and hopes to become a child life specialist at Texas Children’s Hospital where she continues to be treated and where the staff knows her well. Ybarra describes the specialist she had as someone who, after her diagnosis, talked to her on her level and explained what was happening and how she would be treated. Child life specialists also work with the family, particularly other siblings, to answer questions and find out how the family and young patient are coping, especially if the patient will need to undergo surgery and a sustained recovery. “Texas Children’s saved my life a billion times and it is a special place for me,” Ybarra says. “I’ve gone through a lot there and I would love to be able to work there and give back.”

### Mentoring Teens with PIDD

About two years ago, Ybarra got a call from Kathy Antilla with the IDF who had contacted Texas Children’s Hospital to learn if it was treating any PIDD patients. Her doctor put the two in contact with one another, and Ybarra has been involved with the patient organization ever since, starting out as a member of the Teen Council and eventually becoming a mentor to the group, helping them plan events for young people with an immune deficiency and participating in a leadership conference earlier this year.

Ybarra says that her therapy has changed her life and the way she views things. "I’m able to do everything every other 22 year-old is doing and without it, I’d probably be stuck in a hospital. Infusions have given me the opportunity to excel in all of the things I want to do and because of that I can live a very normal life,” she says.

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

Last year, Karissa traveled to Washington, D.C. as part of IDF’s legislative day on Capitol Hill. She is pictured here with U.S. Rep. Kevin Brady (R-TX).
AT THE END OF MARCH, just shy of the statutory deadline, the U.S. Food and Drug Administration (FDA) issued final guidance, “Standards for Securing the Drug Supply Chain – Standardized Numerical Identification for Prescription Drug Packages” (Final Guidance). The Final Guidance identifies the standardized numerical identifier (SNI) that should be used for package level identification in the U.S. The Final Guidance defines package level as the smallest unit of sale that is placed in interstate commerce by the manufacturer or repacker that will be dispensed by the pharmacy. The Final Guidance states that the SNI for most prescription drug packages should be a serialized National Drug Code (sNDC). At this time, the sNDC is not a federal requirement. The Final Guidance only delineates the standard that FDA has developed which may be part of future legislation and subsequent FDA regulations.

What is the sNDC?
The sNDC is a combination of the National Drug Code (NDC) and a unique serial number. The sNDC is generated by the manufacturer or repacker, for each individual package and should have no more than 20 characters (letters and/or numbers). The Final Guidance emphasized that the scope of the document is limited—it does not cover implementation nor does it address other standards for validation, authentication, and tracking and tracing of prescription drugs. The FDA stated this is an initial step and will be the first of a series of guidance documents related to Section 505D of the Federal Food and Drug and Cosmetic Act (the Act).

Why is Section 505D important for the FDA?
Section 505D was created through the enactment of the Food and Drug Administration Amendments Act of 2007 (FDAAA). It requires the FDA to “develop standards and identify and validate effective technologies for the purpose of securing the drug supply chain against counterfeit, diverted, subpotent, substandard, adulterated, misbranded, or expired drugs.” Specifically, Section 505D created a hard deadline for the FDA to meet regarding development of a SNI. FDA was required within 30 months of enactment of FDAAA to develop a SNI that would be applied to a prescription drug at the point of manufacturing and repackaging, at the package or pallet level, and be
sufficient enough to facilitate the identification, validation, authentication, and tracking and tracing of the prescription drug. Section 505D also required FDA to consult with a wide range of groups during the development process and harmonize with international standards.

FDA began the public consultation process for the development of the SNI in 2008. FDA issued a Federal Register (FR) Notice seeking information from drug manufacturers, distributors, pharmacies, and other interested parties. The FR Notice asked a series of questions soliciting specifics like: whether the SNI should contain recognizable characteristics or be random codes; should the SNI include the lot number; or should the SNI be machine readable or human readable. The FR Notice also delineated a series of questions on standards for validation, track and trace, and authentication and asked for input on how the FDA should prioritize the development and identification of these standards. According to the FDA, the input received from this FR Notice was used to create the “Draft Guidance for Industry on Standards for Securing the Drug Supply Chain – Standardized Numerical Identification for Prescription Drug Packages” (Draft Guidance) that was issued in January of 2009. In general, there were no unforeseen changes made to the Final Guidance.

The Final Guidance is compatible with an internationally recognized standard – GTIN, which was established by GS1. The GTIN is a global standard for item and object identification. The GTIN can be serialized using an Application Identifier (A1) to create a serialized GTIN (sGTIN). As other countries consider legislation or regulatory requirements related to serialization, continued support and use of this global standard will be imperative for the global pharmaceutical supply chain.

**PPTA was active throughout this guidance development process and was pleased to see that comments made to the Draft Guidance by the association and others from industry materialized to changes published in the Final Guidance.**

PPTA commends the FDA on the process for developing this guidance, in particular the ability of the Agency to issue final guidance within a reasonable amount of time. It is safe to say that a looming statutory deadline always helps and goes to show everyone needs a deadline.

**Bridget Elis is PPTA’s Assistant Director, Regulatory Policy**

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2. 21 CFR Part 207 defines the NDC, which corresponds to the specific drug product
3. FDA ACT § 505(D)(a)
4. FDA ACT § 505(D)(b)
PPTA’s International Plasma Protein Congress (IPPC) 2010, held on March 16-17 in Berlin, attracted a record number of attendees with over 300 people participating in the discussions and listening to presentations on a wide range of topics.

Highlights of the event on Tuesday, March 16 featured an opening session headlined by Reinhard Burger, of the Robert Koch Institut who spoke about pandemic preparedness and recent experiences with the H1N1 virus. A panel on developments in health care reform in the U.S. and long term care for aged populations featured perspectives from Bartosz Przywara, European Commission, Peter Turner, CSL Behring and Jay Greissing, PPTA, and offered a patient perspective from Mark Skinner of the World Federation of Hemophilia. A second panel focused on rare disease policies from the European Union member states and included perspectives from Rainer Seitz of the European Medicines Agency (EMA), Nisha Jain of the U.S. Food and Drug Administration (FDA), David Watters of the Platform of Plasma Users (PLUS), Rita Kessler of the Association Internationale de Mutualite (AIM) and Mirjam Mann of the Alliance of Rare Chronic Diseases (ACHSE). Wednesday’s first session featured Patrick Robert of the Marketing Research Bureau, Ileana Carlisle of Biotest Pharma-
ceuticals, Bill Bees of Cangene, Knud-Peter Krause of Haema AG and Michaela Rethwilm, a consultant. The panel discussed the latest developments in demand and supply, ethical and regulatory aspects of collecting hyperimmunes plasma and possible barriers to self sufficiency in the regulatory arena. A final session featured a discussion on therapy developments and included insight from John Winer of Queen Elizabeth Hospital in Birmingham, United Kingdom; Thomas Kuhne of University Children's Hospital in Basel, Switzerland; and Pietro Caironi of the Instituto di Anestesio-logia e Rianimazione, Milan, Italy.

The full conference program featured several noteworthy sessions including topics addressing clinical developments, patient access, self-sufficiency, regulatory considerations, plasma collection, health policy, among others. Delegate feedback of IPPC 2010 was very positive and a solid program, high-level speakers, a location in one of Europe’s most exciting capital cities and an impressive number of relevant attendees combined for a very successful event.

The IPPC 2010 presentations are available for download from the website: www.ippc2010.com.

Kara Flynn is PPTA’s Director, Global Communications

IPPC Workshop Focuses on Epidemiological Data Reporting

In conjunction with IPPC 2010, PPTA hosted a panel discussion on the value of epidemiological data reporting. Representatives from industry, including Dr. Ruth Offergeld, Robert Koch Institut, Dr. Micha Nübling, Paul-Ehrlich-Institut, Dr. Glenda Silvester, European Medicines Agency (EMA) and Dr. George Schreiber, WESTAT participated on the panel which was moderated by Prof. Albert Farrugia, PPTA.

IPPC 2011 will be in Lisbon, Portugal • March 15-16
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IN LATE MARCH, President Barack Obama signed into law the Senate health care reform bill, H.R. 3590, the Patient Protection and Affordable Care Act (PPACA), and the bicameral reform budget reconciliation package H.R. 4872. This action is, without question, an historical achievement because of the sweeping changes to America’s health care system amongst the back drop of deep partisanship and the economic recession.

Specifically, within six months of enactment, group health plans and health insurers offering group or individual coverage may not establish lifetime limits on the dollar value of benefits for any participant or beneficiary. Beginning January 1, 2014, such plans and insurers may not impose any preexisting condition exclusion with respect to such plan or coverage.

In order to provide immediate health insurance coverage to those uninsured individuals with pre-existing conditions, the new law requires the Secretary to establish, within 90 days of enactment, temporary national high risk health insurance pools. These risk pools will exist until 2014, when the insurance exchanges will be operational.

Annual limits on coverage are also banned, beginning January 1, 2014. Prior to that date, group health plans and health insurers offering group or individual coverage may impose annual limits on coverage, but only to the

Highlights of the New Health Reform Law

Insurance Reform

Many stakeholders within the plasma protein therapies user community worked hard to secure the PPACA’s elimination of lifetime limits on insurance benefits and of the practice of insurers denying coverage based on preexisting conditions. These insurance reforms will help all Americans access and maintain the medical care they need.
extent necessary to preserve participant or beneficiary access to needed services with a minimal impact on premiums.

Other insurance reform provisions of note include: (1) an extension of health insurance coverage for adult child dependents that are not married, allowing such individuals to remain on a parent or guardian’s insurance plan until age 26; (2) a prohibition against coverage rescissions by group health plans and health insurers offering group or individual coverage; (3) fair premiums; (4) guaranteed coverage and renewal; and (5) a prohibition against discrimination based on health status of covered individual.

**Follow-on Biologics (Biosimilars)**
The follow-on biologics provisions signed into law provide innovators with four years of data exclusivity and 12 years of market exclusivity. These exclusivity provisions were favored not only by the entire biologicals industry, including PPTA, but also by the majority of lawmakers. Despite speculation in recent months that a more generic friendly exclusivity provisions would be in the final bill at the President’s desk, a scaled back biosimilar bill never came to fruition.

**Comparative Effectiveness Research (CER)**
As part of its section establishing an independent patient-centered CER body, the PPACA includes a rare disease advisory panel, which PPTA championed. This provision requires the appointment of an expert advisory panel during each instance a rare disease is being considered for a CER study for the purpose of assisting in the design of the research study and determining the relative value and feasibility of conducting the research study. Because patients and physicians with relevant experience in the rare disease must serve on such a panel, PPTA believes this provision successfully empowers patients and physicians to contribute to the CER process. PPTA looks forward to working with consumer organizations as the new CER institute is implemented and engaging appropriately as CER studies are proposed.

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**A History of Overhauling Health Care in the United States**

- **1934**
  President Franklin D. Roosevelt’s New Deal omits health insurance plan

- **1945**
  President Harry S. Truman calls on Congress for health care overhaul

- **1988**
  Medicare Catastrophic Coverage Act overwhelming approved by Congress and signed into law by President Ronald Reagan

- **1989**
  Catastrophic Coverage Act repealed

- **1993**
  President Bill Clinton starts a reform effort which would provide universal coverage based on the idea of “managed competition”

- **1994**
  President Bill Clinton’s Health Security Act fails to pass in Congress
Independent Medicare Advisory Board (IMAB)
While the CER provisions have strong safeguards for patient access, the IMAB provisions fail to protect patients in a similarly adequate manner. The purpose of the IMAB that will be created as a result of the passage of health care reform is to reduce the per capita rate of growth in Medicare spending. Beginning in 2014, the IMAB would submit to Congress and the President recommendations to this effect if the Chief Actuary of the Centers for Medicare and Medicaid Services (CMS) determines in the previous year that such growth rate will increase faster than an established inflation rate. These recommendations would automatically go into effect the following year unless subsequent legislative action is taken by a certain date.

Some of the structure of the IMAB is controversial; such as, its ability to require the Secretary of the U.S. Department of Health and Human Services to unilaterally promulgate new Medicare policies with limited Congressional oversight. One hundred Members of the House of Representatives—both Democrats and Republicans—have expressed opposition to the IMAB. In a January 15, 2010 letter to Speaker of the House Rep. Nancy Pelosi (D-Calif.), the concerned lawmakers contend that the “unelected, unaccountable Medicare commission as envisioned in the Senate’s IMAB proposal... would end Congress’s ability to shape Medicare to provide the best policies for beneficiaries in [their] communities around the country.” Because of the opposition to it, one could certainly see this provision modified before it goes into effect in 2014. While this board will have a consumer advisory panel, the IMAB is generally bad policy that has the potential to not only adversely affect patient access to the complete range of plasma protein therapies in each therapeutic class, but also hinder the ability of patients to follow the most appropriate treatment plan developed with their physician.

Julie Birkofer is PPTA’s Senior Vice President, North America and Jay Greissing is PPTA’s Director, Federal Affairs

1962
President John F. Kennedy takes up the issue of health benefits for Social Security recipients

1965
Deput of Medicare and Medicaid

1971
President Richard M. Nixon backs plan requiring employers to provide a minimum level of insurance to employees

1973
Health Maintenance Organization (HMO) Act is signed into law

1976
President Jimmy Carter calls for a comprehensive national health insurance system with universal and mandatory coverage

1976
Congress signs the Consolidated Omnibus Budget Reconciliation Act or COBRA into law

1996
The Health Insurance Portability and Accountability Act (HIPAA) establishing medical record privacy and protecting people from being barred for preexisting conditions is signed into law

1997
The State Children’s Health Insurance Program (S-CHIP) is enacted

2003
Medicare is expanded to cover prescription drugs when President George W. Bush signs the Medicare Modernization Act into law

March 23, 2010
President Barack Obama signs the Affordable Health Care for America Act into law
A considerably smaller number of people know that Memphis and PPTA are even more intimately linked than Memphis and Elvis. Elvis was born in Tupelo, Mississippi. PPTA was born in Memphis—almost 40 years ago.

Morris “Buddy” Moss was president and co-owner of what was then Interstate Blood Bank Inc. Its first U.S. Food and Drug Administration (FDA) license, which is still active, is #173. It is one of the oldest licenses in the industry. A family owned and run business from the start, Buddy teamed with partners Joe Rubert and Jack Gary to grow Interstate into a network of blood banks that supplied small bed hospitals throughout the eastern half of the U.S. that were not being effectively served by the Red Cross. In Memphis alone, for more than 15 years, Interstate drew whole blood on weekends, from servicemen stationed at the military base outside Memphis for St. Jude Children’s Research Hospital. The donors received their donor fee and got a duty free weekend, the Interstate lab processed, tested and labeled the units, Interstate gave St. Jude Hospital credit in their account for 50 percent of units collected, and Interstate performed the cross matching of units for their leukemia patients before surgeries—all at no charge.

In the early 1960’s, seeing the need to reconstruct its business model, Interstate began developing the plasmapheresis side of the company. Drawing donors via the “double-stick” method into glass collection bottles, Interstate was one of the earliest companies to produce what we now know as source plasma in considerable volumes. As the public forum in the U.S. began its move away from compensated donors as a source of blood for direct patient transfusions, there began to be concerns about pressure from local, state and even federal agencies that were against compensating donors in any form.

MOST PEOPLE KNOW MEMPHIS, Tennessee as the “Home of Elvis Presley and Good Barbeque.” Elvis’ home, Graceland, is in Memphis and, as a residence, it is second only to The White House in the number of tourists that visit annually. Memphis also hosts the world’s largest barbeque cooking contest every year during its annual “Memphis in May” Festival.
In the early 1970’s, these pressures were growing and Buddy Moss saw the importance of unifying the efforts of the dozens of independent blood component producers as well as the fractionators and diagnostic manufacturers to preserve our industry. He managed to get what was, at that time, a sizable group of competitors with a common interest, to agree to meet in Memphis to see if there was a possible unified way to fight for the way we do business. At a hotel which is now owned by Crowne Plaza, that first meeting was held. The participants could hardly have been expected to know then, what the significance of their discussions would ultimately mean.

Larry Moss has worked at Interstate since he was 16 years old. Although only in his early 20’s at that time, he was told by his father, Buddy, that he would be going to this meeting too. When he questioned why, Buddy responded, “You’ll get to meet some people that you’ll hopefully be doing business with in the future, you might learn something, and since everyone in the room are true competitors, I may need a bodyguard!”

That meeting was the beginning of The American Blood Resources Association (ABRA), the organization that evolved into what became PPTA in 2000 and the division now known as PPTA Source. Apparently Larry Moss got a lot out of that meeting. ABRA’s first secretary, Judy, ultimately became Larry’s wife.

In 1981, when Buddy Moss retired, Larry became President of the group of businesses that now make up The Interstate Companies. Larry and Judy live in the house that Buddy built in 1966. When asked why Buddy retired at the young age of 51, Larry responded, “I took his secretary, I took his job, and I took his house—why not?”

Judy and Larry, the proud parents of three kids, have been married 37 years—a marriage that has survived even though Larry fired Judy only six months into their marriage. Their son,
The three mosses work side-by-side every day supplying plasma and blood components to a broad customer base that includes multiple fractionators and a large group of diagnostic and reagent manufacturers.

Matt, works for Interstate in Operations and the Whole Blood division. Judy and Larry bought a candy company that has operated in Memphis since 1902. Judy works there with their youngest daughter, but they insist that Larry stay away from the customers. They are of the firm opinion that Larry’s brutally honest approach to things is not always a very good way to handle the retail public. It sounds like Larry has been put in his place.

Larry owns Interstate Blood Bank, Inc, the parent corporation of The Interstate Companies. He shares ownership with his brother, Stephen, and his son, Matt, in the other divisions of the group, which collectively owns 23 plasma collection centers, eight diagnostic whole blood collection centers, a testing lab and its own supply and distribution warehouse. The three Mosses work side-by-side every day supplying plasma and blood components to a broad customer base that includes multiple fractionators and a large group of diagnostic and reagent manufacturers.

Larry served as a Board member of ABRA for over 20 years and has served on the PPTA Source Board since its formation. If you ask Judy or their kids, what is Larry’s goal in life—what drives him? They will all answer the same way—TO WIN!

When asked what PPTA could do better for him, his response is quick—reduce the number of audits and inspections we endure. “With every customer auditing, each U.S. federal agency auditing, each international regulatory group auditing, each state auditing, and even the need to conduct our own corporate audits, the resources committed to dealing with them is outrageous, he says. He adds “This is even made worse by the fact that most of the particulars of each inspection are replicated over and over and over in each audit. There has to be some way to consolidate and share the information garnered in order to reduce the number of audits.”

Jan M. Bult is PPTA’s President
How Manufacturers of Plasma Protein Therapies Ensure the Safety of the Products

The examples for emerging viruses given above have to be considered on a case by case basis. In the case of WNV and B19V, there was an abundance of experience with the viruses themselves or relevant model viruses belonging to the same family. The available data provided a well-founded level of assurance pertaining to the efficiency of virus reduction/inactivation procedures in the manufacturing process of plasma protein therapies. PPTA member companies performed extensive studies on the inactivation of WNV demonstrating that WNV behaves exactly as expected for a flavivirus. Based on these compiled data on WNV and other flaviviruses, it could be demonstrated that WNV does not impair the safety of plasma protein therapies. Regulators came to the same conclusion.

SARS initially caused world-wide concern because of the unknown nature of the pathogen. Shortly after the outbreak of SARS the causative agent was identified as a coronavirus. Coronaviruses are enveloped viruses which are believed to cause a significant percentage of all respiratory infections, e.g. common colds in human adults. Available data demonstrate that coronaviruses are efficiently inactivated through established
manufacturing processes for plasma-derived proteins, reassuring patients, physicians and regulatory authorities on the safety of plasma protein therapies. In the case of SARS, PPTA member companies performed individual studies to demonstrate the efficiency of their virus inactivation/removal steps.

The examples described provide reassurance that model viruses allow a fairly accurate prediction how pathogenic viruses of the same family behave with regard to virus inactivation through established inactivation methods.

Enveloped viruses are more susceptible to inactivation processes than non-enveloped viruses. There are a number of established and efficient methods to remove or inactivate non-enveloped viruses, such as nanofiltration, chromatographic methods, the cold ethanol fractionation process or pasteurization and dry heat.

In 2000, PPTA introduced a voluntary standard for Parvovirus B19 as an additional safeguard. The standard can be found on PPTA’s website www.pptaglobal.org.

Spongiform encephalopathies, such as vCJD, are caused by abnormal prion proteins and follow a completely different paradigm than diseases caused by viruses or bacteria which contain genetic material.

Parvoviruses are non-enveloped viruses; human parvovirus B19 (B19V) was first isolated in 1975. B19V is normally spread via the respiratory route and is considered a childhood disease (“fifth disease”) but can under certain circumstances cause clinically significant diseases (Erythema infectiosum, hepatitis, myocarditis, arthritis). In humans, a number of new parvoviruses besides B19V have recently been identified, e.g. PARV4, which has been isolated from patients with Acute Viral Infection Syndrome, or Bocavirus, which has been isolated by screening of respiratory tract samples. The pathogenicity and clinical relevance of these two parvoviruses is at the moment unclear. Therefore, PPTA member companies and regulators remain vigilant.

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PPTA Member Companies and Regulators Remain Vigilant Against Known and Unknown Pathogens

A Known Pathogen
Specific climate conditions can facilitate the spread of a virus, for example in the case of West Nile Virus (WNV) that caused an unprecedented epidemic in the U.S., starting in 1999 in the New York City area. WNV was known in Africa, the Middle East and Southern Europe. WNV mainly infects birds, but is known to infect humans in certain circumstances, and other species. The main route of human infection is through the bite of an infected mosquito.

Increase of understanding of a known pathogen
Parvoviruses are non-enveloped viruses; human parvovirus B19 (B19V) was first isolated in 1975. B19V is normally spread via the respiratory route and is considered a childhood disease (“fifth disease”) but can under certain circumstances cause clinically significant diseases (Erythema infectiosum, hepatitis, myocarditis, arthritis). In humans, a number of new parvoviruses besides B19V have recently been identified, e.g. PARV4, which has been isolated from patients with Acute Viral Infection Syndrome, or Bocavirus, which has been isolated by screening of respiratory tract samples. The pathogenicity and clinical relevance of these two parvoviruses is at the moment unclear. Therefore, PPTA member companies and regulators remain vigilant.

An Unknown Pathogen
Unknown viruses are (previously undiscovered) zoonotic viruses, or any infectious disease that can be transmitted from non-human animals to humans or from humans to non-human animals, entering the human population. For example when the first cases of Severe Acute Respiratory Syndrome (SARS) were recognized the cause of the disease was unknown. Soon the causative agent was identified as SARS coronavirus (SARS-CoV) This virus caused nearly a pandemic between the months of November 2002 and July 2003, with a case-fatality rate of 9.6% worldwide (World Health Organization Reference). SARS represents a true emerging infection. Other members of the family of Coronaviruses have been known for a long time and are easily inactivated by established methods.

An Unknown Form of a Known Pathogen
Creutzfeldt Jakob Disease (CJD) was first described in the early 20th century and occurs roughly in one per million humans per year. Variant Creutzfeldt Jakob Disease (vCJD) is caused by abnormally folded prion proteins. Infection occurred initially by consumption of bovine spongiform encephalopathy (BSE) infected beef. Today, there are four known transmissions through blood transfusions. In 2009, a patient was described who died of causes unrelated to vCJD. A post-mortem biopsy found abnormal prion protein in his spleen. As of today, no conclusion has been established about the route of transmission in this case. Transmission of abnormal prion proteins from human-to-human has been described for related forms of the disease, but vCJD represents an example of an interspecies transmission of abnormal prion proteins through oral intake of contaminated beef.
information. In this case the disease is caused by the abnormal prion protein, which is able to replicate but contains no genetic information. PPTA members conducted many investigations including a collaborative study to demonstrate the prion inactivation by sodium hydroxide (NaOH) for sanitization of production equipment (Bauman et al., 2006). The aim of this study was to ensure patients and regulators on the efficacy of common cleaning and sanitization procedures. Other studies demonstrated that the manufacturing process has the capacity to remove prions (if they would be present) from the plasma. An overview of publications is listed in the Committee for Proprietary Medicinal Products (CPMP) Guideline on the investigation of manufacturing processes for plasma-derived medicinal products with regard to vCJD risk (CPMP/BWP/CPMP/5136/03). Other PPTA initiatives to reassure patients and other stakeholders were workshops on specific pathogen related topics. For example, PPTA held specific events on selected topics in 2002, 2005, 2008, and 2009 in conjunction with the annual International Plasma Protein Congress. The aim of these events was to ensure a continuous and transparent communication and information exchange among all interested parties.

How Can the Industry be Best Positioned to Respond to Emerging Pathogens?

The infrastructure for dealing efficiently with a spreading epidemic ideally needs to be in place before the arrival of the virus. The U.S. experience has shown that it is difficult enough for one country under the same regulatory jurisdiction to implement the different measures necessary to deal with the epidemiology. Just imagine what it needs to achieve this on a global basis. Different countries have different regulatory frameworks, different communication practices and a different perception of risk. PPTA member companies must comply with these different requirements worldwide. It is generally accepted that plasma is global and plasma protein therapies are unique medicinal products provided to patients worldwide. Consequently, a harmonized global approach would be needed to react timely and efficiently to new emerging pathogens.

In 2004, PPTA launched the Emerging Infectious Diseases (EID) Roundtable. The EID Roundtable included policymakers from regulatory and standard setting organizations, representatives of patient groups, international experts in print media, bioethics and risk management, and scientists, with the goal to begin a dialogue to improve global communications related to decision making in the face of EID threats. In the second Roundtable in 2005 it was reaffirmed that only a harmonized approach can avoid negative impact on availability of plasma protein therapies. Education of patients and physicians about the difference in risk between components for transfusion and fractionated, virus inactivated plasma protein therapies is key to sensible decision making and risk mitigation.

PPTA’s dedicated group of scientists from all member companies, the Pathogen Safety Steering Committee (PSSC) diligently survey the literature and other publicly available data in order to post safeguards against the potential risk of transmitting emerging pathogens. In general, bacteria, fungi, and parasites transmissible by blood are not considered relevant for plasma-derived products as these pathogens can be removed by sterile filtration which is a prerequisite for all plasma protein therapies. Other pathogens as viruses and prions are evaluated constantly and thoroughly for potential epidemiology in the donor population (including donor deferral measures), for a potential virus load in the plasma of an apparently healthy donor and for virus reduction capacity of the manufacturing processes, employing relevant viruses and relevant model viruses in order to perform a risk assessment regarding the safety of plasma protein therapies.

REFERENCES


Albrecht Gröner and Herbert Dichtelmüller are members of PPTA’s PSSC. Ilka von Hoegen is PPTA’s Senior Director, Quality and Safety
HUMAN ALBUMIN IS A MULTIFUNCTIONAL PLASMA PROTEIN, with a molecular weight of 69000 Daltons. At physiological concentrations of 40 to 50 g/L, albumin is the most abundant protein in human plasma, constituting 50% of the total plasma protein content and accounting for 70 percent of plasma’s capacity to retain water in the circulation, known as colloidal oncotic pressure (COP). In addition to maintaining COP, albumin plays other roles related to its capacity to bind other substances. These include, transporting hormones and neutralizing toxins such as bilirubin, and binding and transporting drugs.

Development of Albumin Solution
Edwin Cohn’s development of a stable albumin solution during World War II was based on a fractionation scheme which was rapidly adopted—and adapted—by a number of pharmaceutical companies. Cohn never took a patent on his work. His technology quickly yielded other therapeutics including immunoglobulin and pro-coagulants like fibrinogen. Albumin, however, remained the mainstay product of the plasma protein industry for decades. Despite the development of synthetic plasma expanders from the 1950s onwards—dextran, gelatin and starches—albumin retained its status as the plasma expander of choice well into the nineteen nineties. As alternative plasma expanders continued to show adverse events not shared by albumin, albumin’s status appeared unquestioned.

This situation changed with the publication of a metaanalysis (MA) claiming an increase in mortality when using albumin. Subsequent MAs of trials using other albumin preparations, as well as data of adverse events associated with albumin did not confirm the MA’s expectations. In 2005, a large clinical study in Australia and nearby countries compared albumin and normal saline in intensive care patients. The results show no difference in mortality between albumin and saline.

Reflections on the Manufacture of Albumin
Albumin is a biological therapeutic, manufactured from a variable source material using a variety of techniques. Manufacturers have evolved variations to it in order to optimize protein yields, stability and access to more proteins. Different protein composition profiles ensue as a result of these different methods, and are associated with different adverse event patterns. Because of their inherent variability ensuing from the source material and the manufacturing process, biological therapeutics including albumin cannot be considered as generic drugs. The development of highly standardized and well-characterized cell lines for the production of biotechnology products such as monoclonal antibodies and some small recombinant molecules allows some of them to be considered as “follow-on” biologics. This term describes protein products where the level of manufacturing and product consistency, together with highly sophisticated characterization techniques, can demonstrate comparability between products from different manufacturers. Irrespective of the extent to which small protein molecules derived from well-characterized sources can be comparable, this concept is difficult to apply for plasma protein derivatives. Recent legislative moves to establish a universal pathway for “follow-on” biologics should be viewed with concern. Currently, the U.S. Food and Drug Administration (FDA) already considers that all the albumin preparations used and described are pharmaceutically, pharmacologically and therapeutically equivalent.
The question of whether the different manufacturing methods can affect albumin’s function merits consideration. The capacity of albumin solutions to bind drugs and fatty acids varies significantly between preparations.

Clinical Studies
One of the problems in treating biologics such as albumin like mainstream drugs is that the patient populations needed to show efficacy are often too small to conduct conventional clinical trials. A number of studies compare albumin to other fluids in sick infants. More low birth weight infants at risk of respiratory distress survive with albumin compared to dextrose, and also show enhanced weight gain and relief of hypotension. Lower rates of septicemia and pneumonia occur in hypoalbuminemic infants on total parenteral nutrition given albumin. Albumin is safe, effective and associated with lower morbidity and neurological sequelae in children with malaria. In all these studies the numbers of patients was too small to get regulatory approval for these beneficial indications. Clearly, new approaches are needed to show benefits in small patient populations.

Albumin's physiological role in binding toxins has been the basis of the concept of albumin-assisted haemodialysis in patients with liver dysfunction. The molecular adsorbent recirculating system (MARS) operates by passing blood over a non-albumin permeable dialysis membrane with an albumin-containing dialysate on the other side. Albumin-bound toxins are absorbed on charcoal and an anion exchanger and the regenerated albumin solutions may be recirculated for further passages. In acute liver failure the treatment leads to an improvement in clinical, hemodynamic and neurological conditions, while reducing blood levels of bilirubin and ammonia. Comparison to a non-albumin detoxification device showed significantly superior outcomes with MARS, indicating that the binding of toxins with albumin is important. In a recently reported trial treatment with MARS in patients with acute liver failure showed improved renal and hepatic function and enhanced transplant-free survival with three or more MARS treatments.

Albumin's Future
Controversy regarding the role of albumin solutions in clinical therapeutics continues to reign. This is the result of an evidence base for the therapies which is sparse and conflicting. Albumin solutions differ in their manufacture, composition and clinical properties, and this may affect the results of studies. Albumin has had a history in which complacency has been succeeded by controversy, to be succeeded by the current era of active investigation. Rather than a passive expander of plasma volume, albumin should be viewed as a complex, multi-faceted biologic, with the potential to contribute to a number of niche indications. The transition from tradition to therapeutic may be long and painful, but may cement albumin’s position as a vital therapy.

Albert Farrugia is PPTA’s Senior Director, Global Access
PLUS is a coalition of patient organizations representing a number of disparate, inherited or acquired, serious or life threatening conditions which are routinely treated with plasma protein products. These are hemophilia, primary immune deficiencies, alpha-1 antitrypsin deficiency, Guillain Barré syndrome, hereditary angiodema, idiopathic thrombocytopenic Purpura and von Willebrand disease. The conditions vary enormously in the level of recognition, diagnosis and treatment. Together, these conditions account for some 90,000 known patients in Europe and a probable prevalence of 380,000.

The conditions are disparate, but the main therapies used in all these conditions are manufactured from human plasma. The organizations that represent the patients with these conditions are acutely aware that diagnosis is insufficient for many of the conditions and access to therapy needs to be increased and optimized. Patients in Europe who rely on plasma-derived therapies deserve the opportunity to have access to treatment and the possibility of freedom from the life-threatening consequences of lack of treatment. Access to safe and effective therapy is affected by government or national healthcare priorities, budgets and availability of specific therapies. However, it is also affected by the views of regulatory authorities, the European Union (EU) Commission, industry, the not-for-profit sector, national blood authorities, donors, the World Health Organization (WHO) and other stakeholders. Patient organizations including the European Haemophilia Consortium (EHC), World Federation of Hemophilia (WFH), and International Patients Organisation for Primary Immunodeficiencies (IPOPI) have made both separate and coordinated representations in the past on issues including the Directives in 1989 and 2002 on blood and plasma and have responded to views expressed by stakeholders and others on issues including proposals on self sufficiency, donor deferral measures, donor remuneration and product importation.

In many cases, these views have been set out as a response to proposals or views which were already well advanced or published. We were concerned that directives, guidelines and recommendations which may have a major impact on access to safe and effective plasma therapies have been promulgated in Europe without proactively seeking the views of the relevant patient organizations. This was unacceptable. Our voices, as the users of these lifesaving and enhancing therapies, must be heard and we must be consulted when measures are being drafted, which will have a major impact on our access to therapy. We further recognized that it was impractical to ask the EU Commission at any level to meet regularly with each of the separate organizations. However, recognizing that we share a common and vital interest in the optimum access to the safest and most efficacious plasma-derived therapies, in 2009 we formed a broad coalition of seven organizations of plasma users—PLUS.

The genesis of PLUS came from the recommendations of a European Platform for Patient Organisations, Science and Industry (EPPOSI) Conference in December 2007 on Best Practice on Communicating Risks and the Value of Safety to Patients with Chronic Diseases. One of the recommendations was that the European Commission should be encouraged to establish condition specific Consultation Groups for chronic diseases. Following this conference we met with the EU Commission officials and with the then EU Health Commissioner and persuaded them to set up such a group with a coalition of organizations representing constant users of plasma products. When they agreed to this proposal, we established PLUS, the Platform of Plasma Protein Users.

The terms of reference for PLUS are as follows:

- To facilitate the exchange of information towards the building of consensus views when possible among the organizations that represent regular users of plasma, plasma proteins or plasma-derived therapies

PLUS+
• To ensure that the consensus views of the organizations are communicated to the EU Commission, Members of Parliament (MEPs), the Council of Europe and other relevant bodies and individuals.
• To ensure that the collective views of the organizations are proactively considered on a timely basis when Directives, Guidelines and recommendations are being framed in relevant areas.
• The collective views expressed on behalf of the organizations in no way detract from each organization’s ability or right to express their own individual view on any particular issue. PLUS will express the collective view on an issue when agreed.

The views expressed by PLUS represent the collective position of the membership. The member organizations are EHC, WFH, IPOPI, Afia Europe, GBS/CIDP Foundation International (GBS/CIDP), Hereditary Angioedema International (HAEI) and the ITP Support Association, which improves the welfare of people with immune thrombocytopenic purpura.

Since our inception we have had a number of meetings with the European Commission officials (DG Sanco and DG Enterprise) and we have ensured that PLUS will be proactively consulted when issues such as the review of the blood directive are being scheduled.

The collective view on these vital issues are not formed in a vacuum. PLUS and the organizations constituting PLUS would have engagement with key stakeholders including industry, the not-for-profit sector, WHO, the International Society Blood Transfusion (ISBT) and national blood authorities. We have been concerned that key stakeholders including those who collect blood or plasma, those who manufacture plasma-derived products and authorities such as WHO will set out positions, which can significantly impact the availability of safe and effective therapies in the required amounts without first eliciting the views of the organizations whose members are the major users of their blood, plasma or manufactured products.

Issues such as donor deferral, donor remuneration and self sufficiency tend to elicit definitive statements from stakeholders, which leave little room for the views of patient organizations. Decisions in these vital areas which can have such a profound impact on the therapy available for our members should not be taken in isolation and should not be made without consulting the representatives of those who use the therapies on a regular basis. We wish to engage with all key stakeholders, to listen, to learn and to proactively contribute the collective views of patient organizations.

Decisions about us should not be made without us. Stakeholders can not know the needs of the patients if they do not engage proactively in dialogue with their representative organizations. It is not a good business model to never elicit the views of your customers. We will be proactive in leading this process. In January of this year, we organized a conference in Dublin, Ireland that brought together many of the key stakeholders. This resulted in two days of focused and positive interaction and discussion. There were clearly different views expressed on many issues yet the process was positive and mutually respectful. The result was a statement of principles, the Dublin Consensus Statement, which was agreed to by 14 of the 15 individual participants (with the exception being the donor organization). This was then sent back to their organizations for their decision on endorsement, non endorsement or agreement in principle with some qualifications. This statement, to paraphrase former British Prime Minister Winston Churchill, was not the end but the end of the beginning. It is clear from the meeting that a dialogue between all key stakeholders can take place in an atmosphere which allows for both debate and mutual respect.

From the comments received to date, there is clear agreement on many issues. Outstanding issues will be the focus of future meetings because all present recognized the importance of this dialogue and indeed of this process being led by the patient organizations. We pledge to continue this process of inclusive dialogue, to do our utmost to bring stakeholders together and to give them an opportunity to understand the concerns and priorities of the patient organizations represented by PLUS. We further pledge that we will not act in the interests of any sector or stakeholder except our constituent patient organizations. We seek constructive engagement. We welcome dialogue, clarification and information.

We will listen respectfully to all, engage with all and ensure that all are aware of our collective views on these vital issues.

Brian O’Mahony is a representative of the PLUS Steering Group and is Chief Executive of the Irish Haemophilia Society.
One of the most important technological improvements that occurred within the industry is the advent and widespread adoption of the automated plasmapheresis process. This eliminates many safety concerns for donors, as the process is a contained, continuous method for removing blood from the donor, separating and harvesting the plasma, and returning the cells to the donor. This means that donors will never be at risk for unmatched cells upon the return cycles.

The automated collection devices were a significant advance as stand-alone systems, but more recent integration of these devices into the overall computerized information systems of the plasma collection facilities allows electronic capture of information that is important in the operation of the device. The adoption of increased automation helps provide a unified safety net for both the donor and the plasma. The automated collection process is the cornerstone of safety within the collection industry and represents a major contribution to the continued improvement in technology and its usage in plasma collection.

Donor Screening and Center Design: Computers and Information Technology

Evolutionary improvements have occurred in the context of center design and donor screening. These improvements have resulted in better, more efficient donor processing, while also increasing the safety of both the plasma and the donor. The screening process relies on improved technology to ensure the donor’s health and the efficiency in which a donor can move through the process. Computerized donor record files track vital information and allow center staff to track and monitor not only the current donor process, but are able to view the donor’s health and donation history as well.
Improved processes in donor screening have led some companies to automate the health history questionnaires as well. The computer-assisted donor health history questionnaires allow the donor to directly communicate with the computer. This direct, computerized questioning helps ensure the donor’s privacy and enables the donor to proceed at his own speed to better understand the screening process and the purpose behind it.

**Testing Technology**

Testing has changed over the years as well; while the screening process was discussed above, it is also important to mention the testing that is used on plasma donation. As tests to protect the plasma from pathogens were developed over the years, the initial tests were designed to detect antibodies in the plasma. The antibodies are proteins that a person’s body produces in response to a foreign substance, including pathogens. It takes the human body time to recognize the foreign substance and produce antibodies against it. Relying only on antibody tests meant that there could be several weeks from the time of exposure to a pathogen and the test for antibodies to that pathogen to become positive. This time is called the “window period.” During the window period, a potential donor may have been exposed to the pathogen and be infectious but still have a negative test. More recent tests, using Nucleic Acid Amplification Technology (NAT) can detect the pathogen itself. Being able to detect the pathogen directly has reduced the window period to days. With today’s testing methods, there is better assurance that plasma is safe and that any positive result can be communicated more quickly and with greater confidence to the donor.

**Logistics and Shipping**

It is as important as ever to ensure that the plasma which leaves the collection center is transported in a way that continues the quality chain. Specialized procedures for shipping are used to ensure that the donations and test samples are kept stable and tracked throughout the process. This helps ensure the robustness of the testing of the plasma and the integrity of the plasma throughout the extensive manufacturing process. Contract partners specializing in shipping and members of the plasma industry have developed thorough systems to ensure that all of these steps are taken and meet or exceed all regulatory requirements.

**Conclusion**

The advancement of technology and the evolution of plasma collection have resulted in a constantly-increasing level of quality and safety of the process. This improves not only what is produced by a plasma center, but also improves the donor’s safety and the donation experience.
CAROLINE KRUSE FIRST GOT INVOLVED
with the Platelet Disorders Support Organization (PSDA) three years ago, when, after being diagnosed with idiopathic thrombocytopenic purpura (ITP), she started a local support group for patients and their families in her hometown of Cleveland, Ohio. ITP is a bleeding disorder in which the immune system destroys platelets, which are necessary for normal blood clotting. At the time, Kruse didn’t realize the group she began was the first of its kind in the country.

Kruse later went on to meet Joan Young, who founded the PSDA 12 years ago and heard her story about fighting the severe disorder. Kruse is an award-winning journalist with a background as a radio and television reporter and producer and is the creator and co-host of a nationally syndicated talk radio show, Family Matters, that focuses on health and family issues. She invited Young to be a guest speaker and to talk about her experiences with ITP. The two women became friends, and Kruse got more involved in the Association, attending meetings and eventually serving for two years on the Board of Directors. When PSDA developed a need for additional marketing and public relations support, Kruse then joined the Association staff as the director of Public Relations, a position created for her. After founder and mentor Joan Young retired, Kruse was asked to become the executive director, a position she began in January 2009.

PDSA’s Focus on Education
PDSA’s main goal is to provide support to patients and information on diagnosis and treatment. Most people upon receiving a diagnosis of ITP have never heard of it and turn to the Internet to learn more. PDSA provides on its website, a host of booklets to download or to order free and offers the newly diagnosed and their families a chance to talk with someone either one-to-one or as part of a discussion group.

Since Kruse started that first local support group, which she continues to co-facilitate, the number has grown to 25 across the country, and the Association has started hosting a teleconference for parents of children with ITP. Kruse notes that they are hoping to start another group for young adults as well.

Other priorities for the year include the launch of a national walk/run in the fall to raise awareness and funding for ITP research and to promote the first National Platelet Disorder Awareness Month in September.

Kruse mentioned a long-term goal to develop criteria in order to designate Centers of Excellence around the country, to establish a comprehensive care model for people with chronic platelet disorders and to institute a national registry.

Living with ITP
Kruse, who has been in remission from ITP for five years, characterizes it as an emotional disease. Physically, she says, the disease changes week to week and it has a huge impact on a person’s quality of life. Physical activity is very limited. Kruse could not bike ride or ski and was often severely fatigued. She describes the experience as, “living by your platelet count.” Kruse adds that there is a tremendous fear associated with the disease, and that PDSA’s work provides support and hope for other patients and their families.

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

About ITP
Symptoms of idiopathic thrombocytopenic purpura (ITP) can include excessive bruising, tiny red dots on the skin caused by broken blood vessels (petechiae), purple spots on the skin (purpura), menorrhagia, bleeding gums and nose bleeds. Immune globulin (Ig) and anti-D Ig are among the treatments for ITP. Diagnosis is made through a routine blood test to determine the platelet count.
KEDRION AWARDED QSEAL CERTIFICATION
PPTA President and CEO Jan M. Bult awarded Kedrion CEO Paolo Marcucci certification of the international Quality Standards of Excellence, Assurance, and Leadership (QSEAL) Program at a ceremony that took place April 7 at the Lucca, Italy-based headquarters of the company. On hand for the ceremony was a patient representative, Gabriele Calizzani, the president of Federazione delle Associazioni Emofilici, the Italian hemophilia association along with members of the Kedrion staff who made QSEAL certification a reality. "Kedrion has successfully met the criteria of the voluntary program, which goes beyond established government regulatory requirements and is designed to demonstrate the leadership of this industry through the development of initiatives that enhance the continued safety and quality of lifesaving plasma protein therapeutics," Bult said in brief remarks during the ceremony. Kedrion is the sixth company to be QSEAL certified and joins Baxter BioScience, CSL Behring, Biotest, Grifols and Talecris Biotherapeutics.

ON WEDNESDAY MAY 5, PPTA HELD ITS ANNUAL CONGRESSIONAL FLY-IN
This event provided an opportunity for PPTA staff, member company representatives and patient organization representatives to advocate with concerted effort.

Pictured from left to right: Gabriele Calizzani, Paolo Marcucci and Jan M. Bult
messages on legislative issues regarding access to plasma protein therapies. More than 35 participants met with nearly 60 congressional offices to advocate on important consumer initiatives such as improved home access to immune globulin for Medicare beneficiaries suffering with primary immune deficiencies, bleeding disorders awareness and screening, and improving beneficiary access to respiratory care under Medicare Part B.

**PLASMA COLLECTION INDUSTRY MEETS WITH STATE LEGISLATORS ABOUT THE IMPORTANCE OF LIFESAVING PLASMA DONATION**

Employees from Minnesota’s 12 plasma collection centers met with key state legislators in the Spring to inform them of the importance of lifesaving plasma used to treat rare, chronic diseases and of the thriving business that plasma collection centers contribute to the state. “The importance of plasma donation and the plasma donor cannot be overemphasized,” said Joshua Penrod, PPTA’s vice president, Source. “The plasma collection centers in Minnesota not only contribute to saving lives, they represent a valuable business in the state.” Plasma centers in Minnesota employ hundreds of people with annual payrolls of millions of dollars. In addition, collectively these centers provide an economic benefit to the state through millions of dollars in compensation to plasma donors each year.

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The plasma collection centers in Minnesota not only contribute to saving lives, they represent a valuable business in the state.
MY NAME IS BILL SPEIR. I am PPTA’s Assistant Director, State Affairs. I’ve been at the Association since June of 2007. My role at PPTA requires me to focus on three tasks: educating state officials and staff about the industry; assisting patients in maintaining access to their medically appropriate therapy; and lobbying the various state legislatures and Medicaid departments to seek outcomes that are beneficial to the consumers and producers of plasma protein therapies.

Tell us about your background.
I grew up in Columbia, Maryland and attended Frostburg State University, which is in Western Maryland about 20 minutes west of Cumberland. I ultimately left my home state to complete my education at Widener University School of Law in Harrisburg, Pennsylvania. After law school, I went to work at Blue Cross Blue Shield of the National Capital Area, where I worked in the Legal Compliance Department drafting contracts and negotiating approvals of contracts with regulators in the District of Columbia, Maryland, and Virginia.

After that, I was hired by the Speaker of the Florida House of Representatives to advise the Representatives on health care issues. Most of the work involved advising the Representatives on Medicaid. Among the many issues I worked on in Tallahassee was Governor Jeb Bush’s historic Medicaid Reform program. In fact, the first draft of Medicaid Reform legislation in the House contained my initials in the name of the document.

Blue Cross Blue Shield and the Florida House have proven to be great experience for my current position at PPTA. Many of my efforts on behalf of PPTA require me to use my knowledge of health insurance and Medicaid to craft positions that benefit the industry and the patients we serve.

What is your proudest professional achievement?
I’m proud of the educational program that we’ve developed at PPTA. I believe it is important to reach out to state decision-makers and their staff to educate them on plasma protein therapies so they will have that knowledge to help them make better decisions when considering issues that impact the industry and the patients we serve.

Among my many activities, the one I find most rewarding is helping patients maintain access to their medically appropriate therapies. I spend much of my time educating patients on how to effectively convey to decision-makers the need to maintain open access to medically appropriate therapies. At the end of the day, state decision-makers want to do the right thing for the constituents they serve while being frugal with their health care policy decisions. I hope after I speak with a patient, he or she is able to show the state decision-maker that maintaining open access leads to cost effective health care because it limits hospital visits which—are the big cost-driver in the health care system.
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**EVENTS**

**UPCOMING CONFERENCES & SYMPOSIMS**

### 2010

<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
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<tbody>
<tr>
<td>May 22 – 25</td>
<td>International Society on Thrombosis and Haemostasis SCC Meeting</td>
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<tr>
<td></td>
<td>Cairo, Egypt</td>
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<tr>
<td>May 26 – 27</td>
<td>International Plasma Fractionation Association (IPFA)/PEI 17th Workshop on “Surveillance and Screening of Blood Borne Pathogens”</td>
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<td></td>
<td>Zagreb, Croatia</td>
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<tr>
<td>June 10 – 13</td>
<td>15th Congress of the European Hematology Association</td>
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<td></td>
<td>Barcelona, Spain</td>
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<tr>
<td>June 15– 16</td>
<td>PPTA Plasma Protein Forum</td>
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<td></td>
<td>Reston, Virginia, United States</td>
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<tr>
<td>June 26 – July 1</td>
<td>XXXIst International Congress of the ISBT</td>
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<td></td>
<td>Berlin, Germany</td>
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<tr>
<td>July 10 – 14</td>
<td>Hemophilia 2010 World Congress</td>
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<td></td>
<td>Buenos Aires, Argentina</td>
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<tr>
<td>August 22 – 27</td>
<td>14th International Congress of Immunology</td>
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<td></td>
<td>Kobe, Japan</td>
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<tr>
<td>September 9 – 11</td>
<td>28th Annual Scientific Meeting of the British Blood Transfusion Society</td>
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<td></td>
<td>Bornemouth, United Kingdom</td>
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<tr>
<td>September 22 – 23</td>
<td>VI Baltic Transfusion Practice Conference</td>
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<td></td>
<td>Riga, Latvia</td>
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<td>October 6 – 9</td>
<td>XIVth Meeting of the European Society for Immunodeficiency</td>
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<td>Istanbul, Turkey</td>
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<td>October 9 – 12</td>
<td>AABB Annual Meeting</td>
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<td></td>
<td>Baltimore, Maryland, United States</td>
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<tr>
<td>October 10</td>
<td>PPTA Source Business Forum</td>
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<td>Baltimore, Maryland, United States</td>
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<tr>
<td>October 17 – 20</td>
<td>Annual Scientific Meeting of the Haematology Society of Australia and New Zealand, the Australian and New Zealand Society of Blood Transfusion and the Australasian Society of Thrombosis and Haemostasis</td>
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<td>Auckland, New Zealand</td>
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### 2011

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<tr>
<th>Date</th>
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<tbody>
<tr>
<td>October 21 – 24</td>
<td>XI European Symposium on Platelet and Granulocyte Immunobiology</td>
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<td>Beaune, France</td>
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<tr>
<td>October 22 – 24</td>
<td>European Haemophilia Consortium Annual General Meeting</td>
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<td>Lisbon, Portugal</td>
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<tr>
<td>November 24 – 26</td>
<td>6th Red Cross and Red Crescent Symposium</td>
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<td>Bangkok, Thailand</td>
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<tr>
<td>February 2 – 4</td>
<td>4th Annual Congress of the European Association for Haemophilia and Allied Disorders</td>
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<td>Geneva, Switzerland</td>
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<tr>
<td>March 13 – 17</td>
<td>6th World Congress on Pediatric Critical Care</td>
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<td></td>
<td>Sydney, Australia</td>
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<tr>
<td>March 15 – 16</td>
<td>PPTA International Plasma Protein Congress</td>
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<td>Lisbon, Portugal</td>
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<tr>
<td>March 22 – 25</td>
<td>31th Symposium on Intensive Care and Emergency Medicine</td>
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<td></td>
<td>Brussels, Belgium</td>
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<tr>
<td>July 23 – 28</td>
<td>XXIII Congress, International Society of Thrombosis and Haemostasis</td>
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<td></td>
<td>Kyoto, Japan</td>
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<tr>
<td>October 22 – 25</td>
<td>AABB Annual Meeting</td>
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<td>San Diego, United States</td>
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