THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SPRING 2012

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Donor Hits Milestone

PID Management in Germany

European Regulatory Affairs

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In the interest of encouraging broad and open discussion of issues relating to plasma protein therapies, collection and fractionation, **THE SOURCE** *magazine may contain statements of opinion on such issues. These statements are those of the author and do not necessarily reflect the opinion of PPTA or its members.*

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INSIDE

SPRING 2012

THE MAGAZINE FOR THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

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In My View

Jan M. Bult, President and CEO

2012 IS A SPECIAL YEAR FOR US. We celebrate 20 years of PPTA and 40 years of ABRA. This is a good time to reflect on some developments that occurred.

My first personal experience with plasma protein therapies was in the late 80's when I was working for Rhone-Poulenc in The Netherlands. The therapies that they supplied were manufactured by Merieux in Lyon (France) and derived out of placental plasma. As we all know that is history.

In 1992, three distinguished gentlemen, Ralph Galustian, Guelfo Marcucci and Otto Schwarz, decided that it was time to set up a specific organization dealing with the complex issues around plasma protein therapies. There was no experienced association and the number of people that were able to speak for and about our industry was limited. I personally remember the enormous efforts made by Dr. Juergen Fischer (then Behringwerke) to represent the industry in many difficult situations. I remember that in the early 90's, during an ISBT meeting in Amsterdam, he was the only one and I thought how can it be that this industry has not more individuals like him. Little did I know at that moment that I would play a role in organizing and mobilizing the many experts that we have today!

In the early 90's I worked for Biotest in the Benelux. The industry representation in Europe was done by EFPIA (European Federation of Pharmaceutical Industry Associations) through two half-day meetings per year. It was well understood that this was not optimal. Thanks to the initiative of the three gentlemen mentioned, a decision was made to ask the Executive Director of ABRA, Mr. Robert W. Reilly to head up activities in Europe and set up the association activities. His son Jim P. Reilly successfully continued with ABRA. In Europe we tried at first to continue with the structure that was in place but rename the EFPIA Working Group to European Plasma Product Manufacturers (EPPM). That was the time Biotest asked me to represent the company for Association work. The EPPM construction did not work. On December 7, 1993 it was decided to leave EFPIA and to form our own independent association. It was then called the European Association of Plasma Products Industry. I became a Board member (representing Biotest) and Treasurer of the first European Board. As I write this we have two individuals left from that time: Dr. Giovanni Rinaldi and Charles Waller. In February 1995 I was called by Knut Hansen, the first Chairman of the European Board to consider working full time for the Association. I joined the EAPPI in September 1995 as Executive Director.

In 1996, the first association activities started in North America, followed by the formation of IPPIA North America in 1998. Activities also started in Japan. I moved to the United States to head up the Association as successor of Robert Reilly in January 1998.

In 2000 it was decided to rename the Association to PPTA (Plasma Protein Therapeutics Association) because the many abbreviations that were floating around caused confusion. That was a time where the individual companies had started to have their own collection centers, precipitating a discussion to merge ABRA into PPTA. That merger happened in 2002 with the creation of a PPTA Source Division.

I can only come to the conclusion that PPTA in 20 years has developed to a respected organization with worldwide recognition by patients, regulators and other stakeholders thanks to two important factors:

- The enormous contributions of the many company volunteers who have brought their expertise to further this industry.
- The very competent Association staff who relentlessly represent the industry on many levels.

I am very proud of having played a role in the establishment of this Association and hope to continue this for many years to come.

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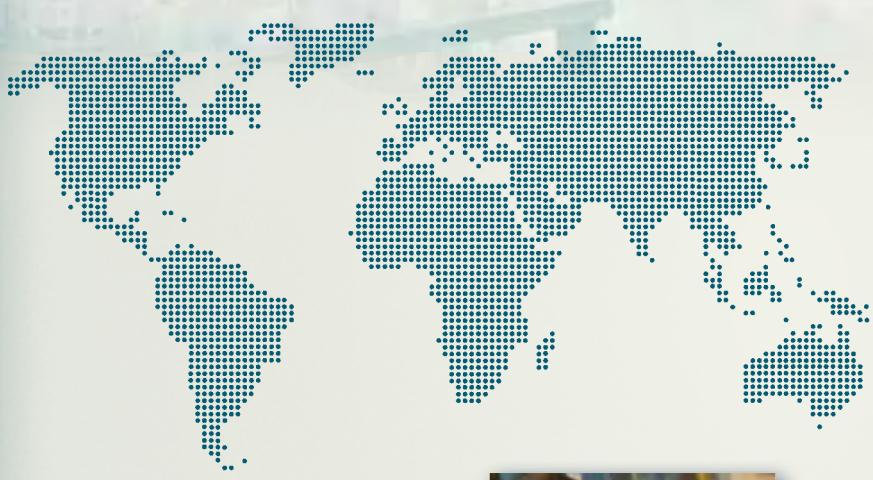

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Source Division Tackles Global Issues

SOURCE PLASMA COLLECTION IS GLOBALLY IMPORTANT. For the most part, private sector collections are primarily in the U.S. and in three European countries: Austria, the Czech Republic, and Germany. Navigating the complexities of this environment, which includes multiple regulatory schemes, requirements and specifications, and differing environments for operations, creates a variegated landscape for activities of PPTA Source. Some of the efforts undertaken may be isolated to one region or country, while others address broader areas of concern.

With the advent of global communications networks, increasing exchanges among regulatory authorities, policymakers, and patient groups, and a 24/7 news cycle, all actions take on global importance.

The Source Division made progress on a number of fronts in 2011 that benefited the industry. The creation and seamless implementation of a new National Donor Deferral Registry (NDDR) in conjunction with our contract partner, Haemonetics, features the latest database infrastructure and complete data encryption. Social media campaigns and German-language websites were launched which feature educational content relating to plasma and plasma donation. New International Quality Plasma Program (IQPP) Standards brochures and Fact Sheets were developed and Plasma Protein Therapies Month was successfully executed in California and Florida. These presented opportunities for legislative education regarding the importance of plasma collection and its role in health care and the economy.

In 2012, there are several things on the horizon. The U.S. Food and Drug Administration (FDA) is gathering information relating to acceptable hemoglobin levels and iron capacities in plasma and blood donors and recently held a workshop on this topic; and, it is likely to be discussed in future advisory committee settings. Additional workshops on other subjects have been or are planned to be held, such as the FDA's data needs, potential hemovigilance topics, and areas of importance to our industry. In addition, the FDA maintains interest in the actions and current thinking of the collection industry, and it is in the best interest of the Association and its member companies to participate and be prepared to address topics as needed.

DREAMSTIME, THINKSTOCK, ISTOCK, SHUTTERSTOCK

In Canada, the regulatory agency for blood and plasma collection, Health Canada held a workshop in late January discussing acceptable volume limits for plasmapheresis. PPTA presented information on the experiences of the industry in the U.S. and will continue to monitor outcomes.

In 2012, we anticipate ongoing interest in relation to European regulatory issues, in addition to action in North America, particularly in the context of epidemiology. Other issues of social concern in certain areas of Europe will also continue to create opportunities for industry dialogue, such as behavioral deferrals.

Austria

For example, Austrian plasma collectors currently face a complicated policy situation that makes it virtually impossible to screen for risk behavior as stipulated in Directive 2004/33/EC, and to consequently defer such donors. In particular, the inability to screen for males who have sex with males (MSM) poses an issue for blood and plasma collection establishments. The Austrian issue is not the common questions of whether to defer and duration of deferral. The problem is that the collection center staff cannot use "discriminatory language" to screen for high-risk behaviors. Therefore, regardless of the outcome on a policy for deferral on MSM, the issue will remain unresolved due to likely difficulties in discerning reasonable screening under one policy and perceived discrimination under another policy. The industry is awaiting the results of a study to be conducted by EDQM that will likely provide some guidance.

Czech Republic

Another recent challenge occurred in the Czech Republic. The government enacted provisions which introduced new language pertaining to the remuneration of donors, that aimed to align with European regulation by referencing Council of Europe Recommendation 95(14) regarding definitions of non-remunerated donation. In effect, however, this wording left it up to legislator's discretion as how to interpret and determine what exactly is in line with 95(14) with respect to plasma donation. To overcome the ensuing legal uncertainty, independent Czech collectors sought clarification and were given a written confirmation from the Ministry of Health (MoH) that current practices are in accordance with the MoH's understanding of 95(14). A short time later, though, the Czech collectors and PPTA became aware of a new proposed amendment to reduce the current remuneration level for plasma donations. The proposal had already reached the parliamentary level of debate; however, after significant advocacy and discussions, the final amendment ultimately sustained the previous conditions for remuneration in the country. In 2012, PPTA and its member companies will carefully watch events in the Czech Republic relating to these issues, and advocate as necessary.

These current topics are complex. Many issues the industry faces can be controversial, or even bewildering to someone unacquainted with plasma collection.

Outreach

These current topics are complex. Many issues the industry faces can be controversial, or even bewildering to someone unacquainted with plasma collection. This creates a compelling mandate for continued education of our various constituencies and the general public. We will continue to build on the recent success, including a revitalized web presence and will seek new approaches to expand our reach. These efforts must include an awareness of the regulatory policies and the importance of the industry's standards program.

IQPP

We are studying the global applicability of standards, particularly IQPP. The IQPP program was originally designed to apply to collection centers in the U.S. As private sector plasma collection expanded to other regions, the program was amended, in part, to apply to centers with business models that encompassed the core values of the IQPP but which were differed North American models. Over time collection practices have evolved and the industry has become increasingly more global. To address these changes, the Association has established the Global IQPP Task Force. This new group is comprised of members with diverse regional experiences and expertise, who seek to identify ways in which the standards may become a truly global program.

This overview demonstrates the global nature of our work. Local, concerns, challenges, and opportunities for plasma collectors create a vibrant tapestry of possibilities. Ranging from technical discussions involving standards and regulation to programs educating stakeholders and the public about our industry, and including everything in between, PPTA is active in representing the interests of all members regardless of geography.

JOSHUA PENROD, *Vice President, Source,* SONIA BALBONI, *Manager, Source and Standards,* SYBILLE BECK, *Assistant Director, Source Europe and Germany*

EUROPEAN REGULATORY

BY ILKA VON HOEGEN

REGULATORY AFFAIRS AND THE SCIENTIFIC INTEGRITY of the industry provide the foundation and form the backbone of the plasma manufacturing industry. A constant and ongoing dialogue with the world's leading regulators has helped build a relationship based on mutual respect and appreciation. As in life, the best, most constructive and productive relationships are based on truth and respect.

PPTA and its member company experts focused on a better balance between regulatory costs and workload in 2011:

The European Medicines Agency

- In response to the appearance of **thromboembolic** complication with immunoglobulin preparations, the European Pharmacopoeia (Eur. Ph.) has amended the monograph for normal human immunoglobulin to request that the method of preparation also includes a step or steps that have been shown to remove thrombosis-generating agents.

  PPTA has succeeded in removing the requirement to demonstrate absence of thromboembolic activity from the already endorsed monograph. PPTA has also objected to the procedural announcement to require a Type II variation when complying

DEM10 / ISTOCK

AFFAIRS A YEAR OF SUCCESS

with the changed monograph. The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) as well as the Co-ordination Group for Mutual Recognition and Decentralised Procedures – Human (CMDh) are in the process of discussing PPTA's proposal for a more risk based approach.

- PPTA successfully convinced the EMA to review the newly introduced fee structure for variations to the Plasma Master File (PMF). In the future PMF holders can group variations together and only pay one fee of 57,200€ (US $73,700) when one of the variations is a Type II. In the initial fee structure each variation was payable in full whether submitted alone or in a grouping. Some companies were charged more than 250,000€ (+ US $ 322,000).

The European Directorate for the Quality of Medicines

- Upon intervention by PPTA, the European Directorate for the Quality of Medicines (EDQM) has refined the requirement for notification of all variations to a Marketing Authorization to only variations that are relevant to section 3 of the Official Control Authority Batch Release (OCABR) provisions. A change, mainly appreciated in the regulatory department work load, has improved quality and helped reduce waste.

European Commission

- Since 2009, PPTA has lobbied to retain a flexible approach towards contract manufacture in the EU GMP **Annex 14**. PPTA challenged the EU authorities' proposal not to address contract manufacture in GMP Annex 14. The Association's proposal to refer to the World Health Organization's (WHO) recommendations on contract manufacture was accepted. Furthermore, the revised GMP Annex 14 no longer requires mandatory inspections by National Control Authorities (NCAs). PPTA has prepared a position statement outlining industry's interpretation of Annex 14 to be used in negotiations with NCAs.

- At the request of PPTA, the European Commission (EC) consulted the National Competent Authorities (NCAs) as to whether **HTLV I/II** positive donors should also be excluded for donations used exclusively for plasma for fractionation. It was concluded that the request letter sent by PPTA should be circulated to all NCAs for analysis and that feedback should be provided in writing to the Commission.

Answers will be compiled and presented by DG SANCO for discussion during the next in NCAs meeting.

- The economic crisis in Greece has surfaced a major flaw of the EU MRP/DCP procedure because the recent strike of the Greek NCA has put all MRP/DCP procedures with Greek involvement on hold. The Greek authorities have immediately responded to a letter from PPTA to demonstrate their commitment to resolve the situation as soon as possible. PPTA wrote to the EC requesting that emergency measures to avoid that the whole system is halted in case one NCA is not able to perform their duties.

Pathogen safety

- **Hepatitis E Virus (HEV) infection**, the major cause of acute hepatitis in developing countries, is being increasingly reported in industrialized countries and it appears to be more prevalent than originally believed. Infections with HEV may be particularly severe for pregnant women, for immune compromised individuals and for those with existing liver diseases. There have been reports of transmission of HEV by transfusion of blood components. Regulatory authorities have started discussions on possible test requirements for blood establishments. PPTA's Pathogen Safety Steering Committee (PSSC) has discussed the HEV situation with experts from European Regulatory Authorities and it was agreed to continue the dialogue on the establishment of tools for HEV NAT and serology assay development.

The Greek authorities have immediately responded to a letter from PPTA to demonstrate their commitment to resolve the situation as soon as possible.

Encouraged by the accomplishments, PPTA and the regulatory experts can look forward to another challenging year with positive outcomes and further progress in the optimization of regulations to benefit patients.

Ilka von Hoegen, *Senior Director of Quality and Safety, Europe*

Celebrate World PI Week

The 2nd Annual World PI Week will be celebrated April 22 29. The mission is to raise awareness of the importance of primary immunodeficiency (PI) diseases and stimulate efforts to improve the recognition, diagnosis, treatment and the quality of life for people with PI world wide. The focus this year is on access to appropriate treatment. World PI Week provides an opportunity to inform and educate health policy makers, schools, families, and the general public about primary immunodeficiencies (PI) and to drive early diagnosis and optimal treatment. Through events and activities promoting the warning signs of PI, seminars, public lectures, video diaries, and press conferences, the global PI community hopes to bring about positive changes in healthcare systems and practices around the world in support of people living with PI.

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German Donor Makes Remarkable Contribution

BY SYBILLE BECK

THE GERMAN REGULATORY SYSTEM only recently increased the annual number of permitted donations from 37 to 45. Given that, the high level of engagement and commitment demonstrated by Ralf Riedel is more than impressive. Mr. Riedel has donated plasma 430 times.

Riedel was born in 1964 and raised in Dresden. He completed an apprenticeship as a big engine mechanic. But Riedel, was willing to risk his future and even prison to leave the German Democratic Republic (GDR). In August 1989, there was much turmoil in the GDR, and the borders were still closed. Riedel says he "will never forget this day [August 23, 1989]. My girlfriend at the time, and I visited a friend in Budapest. We were watching a TV report on Eastern Germans leaving the country via Hungary. We then confessed to our friend that this was also our intention and they advised us to get the train to Sopron and to try crossing the border from there. We did as told and when we arrived went at first to the local camping site that was filled with other Germans. A lot of them approached us and asked if we were also there to go to Austria, but we were cautious as we assumed they could as well be from the STASI [the secret police of the GDR]. But, one guy told us that in ten minutes there would be about five Austrian cars to arrive to take us to the border region from where we would be on our own. We just took our chance and went there. With handmade maps we walked through the woods in the frontier area and finally made it safely to Austria."

From there, Riedel went to Freiburg and Ingolstadt where he lived for the past 20 years, before moving to Regensburg last year. He still works in Ingolstadt as a programmer for a 3-D-laser cutting line, producing prototypes for the automotive industry. He started donating plasma when the center in Ingolstadt was still owned by the Bavarian Red Cross in the 1990s before it was taken over by KEDPlasma.

When asked why he decided to become a donor, Riedel replied, "I always had a social attitude. While I would not describe myself as somebody with a helper's syndrome, I am somebody who usually gives more than he asks back and when I discovered plasma donation I thought that was a great way to help other people without real discomfort." Two other factors influenced his willingness to donate. As a climber and a former biker, he lost several good friends

ENJOYNZ / ISTOCK

Celebrating the 400th donation. From left: Alexander Giss MD, KEDPlasma Center Manager Ingolstadt, Ralf Riedel, Stephan Walsemann MD, Managing Director KEDPlasma GmbH

in accidents. Although, he was unable to help them, the idea that in his donation could contribute saving live was very encouraging. In addition, the birth of his daughter Romana in 1997 inspired him to contribute to something larger, so that in case his daughter might need a transfusion or a replacement therapy she might get it.

With the investment of a few hours per donation for more than 20 years, Ralf Riedel has contributed to improving and to saving a multitude of lives. In his modest way, he has assumed responsibility for a larger cause and to help other people without getting anything in return. Riedel intends to donate for the next 20 years or another 400+ donations.

Sybille Beck, *Assistant Director, Source Europe and Germany*

KEDPlasma in Germany

KEDPlasma GmbH was founded in 2008 in Graefelfing close to Munich, Germany. KEDPlasma Deutschland procures European plasma for its parent company, Kedrion, and owns three plasma centers located in Bayreuth, Fürth and Ingolstadt. The plasma centers in Bayreuth and Ingolstadt were previously owned by the Bavarian Red Cross Blood Service, whereas the Fürth Center was established in 2008 by KEDPlasma. All three centers are IQPP certified. Being a PPTA Source member since 2009, KEDPlasma is represented at the European Plasma Collectors' Committee (EPCC) by its Managing Director Dr. Stephan Walsemann.For more information, please visit **www.kedplasma.com**.

KEDRION
BIOPHARMACEUTICALS

Teen with PID Finds Voice to Mentor Youth

By Kym H. Kilbourne

TIFFANI PEKKALA WAS SICK FROM THE MINUTE SHE WAS BORN. She suffered the familiar chain of illnesses that many people with primary immune deficiency face prior to diagnosis, including multiple pneumonias and frequent ear infections. Tiffani went to live with her grandmother Bette-Jo Poser in 2001, when she was five, but constantly fought illness until they found a new doctor in 2004. Bette-Jo says it is the best thing that happened to her. She knew something was wrong, but had never heard of an immune deficiency.

Bette-Jo took Tiffani to an allergist who ran a panel of tests that all came back positive; a second set of the same tests proved negative results. A blood test finally confirmed that Tiffani has common variable immune deficiency (CVID).

Tiffani was referred to an immunologist who was treating other PID patients and was familiar with the diseases. Living near Camas, Washington, Tiffani and Bette-Jo had access to the Portland, Oregon medical community. Bette-Jo went online to learn more and found the Immune Deficiency Foundation (IDF) and Jeffrey Modell Foundation (JMF). Fortunately, when Tiffani received immune globulin treatment, her health improved dramatically.

Despite the specialized care, Tiffani needed to try several immune globulin products before finding the one that she and her physician believed was the best course of treatment and alleviated side effects ranging from headaches to nausea to incapacitating exhaustion.

Tiffani, who is now 17, was eight when she was diagnosed. Bette-Jo found information to help guide her through the series of questions that made those early days a real struggle. "You feel so alone," Bette-Jo said. "IDF was a real lifesaver."

Bette-Jo described how she valued the opportunity to talk with other parents who were coping with the same challenges, and credits her rapid education with PID to learning from other parents and exchanging information. Today, Bette-Jo is a peer volunteer with IDF. When someone is diagnosed with a PID, she reaches out to provide support for the patient and family.

Tiffani is also an advocate for PID patients, participating on the IDF Teen Council for the last two years. Her experience with dealing with a rare, chronic illness that her school peers didn't understand is helpful to other young people with PID. At first, Tiffani didn't want her classmates and friends to know about her disease. She had an especially troubling experience in elementary school, when a school employee essentially announced that she had an immune deficiency. It was misunderstood to be AIDS and Tiffani was forced to change schools.

"I've had to face a lot of things that most kids my age haven't," Tiffani said. "But, I wouldn't have done it any other way. I've been able to help people through outreach and by organizing blood drives. Helping people is so rewarding, and I hope that people will see what I do and my story, and be inspired to help people in their own lives."

By the time Tiffani entered seventh grade, things began to change for her. She spoke to her homeroom class about PID and why she missed school periodically. She now speaks with many groups including nurses' associations and high school students about PID and is very forthcoming about her illness. Tiffani has organized blood drives and even has physicians sharing her contact information with newly diagnosed patients. She was recognized with a Blood Hero Award, by her local Red Cross last year. Today, Tiffani is student body president and aspires to be a graphic designer. Bette-Jo says, "She is an amazing kid."

Tiffani has blossomed into a volunteer advocate. In addition, to her efforts with PID patients, she has helped organize fundraising events, including a benefit concert for the *Make a Wish Foundation*. "She's a brave kid, Bette-Jo said.

Kym H. Kilbourne, *Director of Federal Affairs, North America.*

"I wouldn't have done it any other way."

"I've had to face a lot of things that most kids my age haven't."

"I've been able to help people through outreach and by organizing blood drives."

"Helping people is so rewarding, and I hope that people will see what I do and my story, and be inspired to help people in their own lives."

Tiffani Pekkala (left) with friend Danielle Kay

BETTER MANAGEMENT OF PRIMARY IMMUNODEFICIENCY IN GERMANY

BY LAURA SAVINI

ON NOVEMBER 23, Mrs. Bracht-Bendt, Chair of the German Parliamentary Working Group for Children and Mrs. Aschenberg-Dugnus, Spokesperson for the German FDP Parliamentary Group on Care organized a launch event at the German Parliament for the German Expert Recommendations for the Better Management of Primary Immunodeficiency (PID).*

From left: Steffen Ball, Deputy Chairman Deutsche Selbsthilfe Angeborene Immundefekte (dsai), Nicole Bracht-Bendt, Chair of the German Parliamentary working group for family affairs, Christine Aschenberg-Dugnus, Spokesperson on Care for the Liberal Party, Prof. Volker Wahn, Head of the Immundeficiency Center Charité Hospital Berlin.

This event proved to be a great success for German patients and physicians, whose concerns regarding access to diagnosis and treatment met with an audience that was receptive to their unique needs, and with the potential to improve their quality of life.

The document was presented by Professor, Dr. of Medicine, Volker Wahn, Head of the Department of Immunology at the Charité Campus Virchow-Klinikum, Berlin, Germany and Mr. Steffen Ball from the DSAI (the German Support Group for Primary Immunodeficiency), two of the experts and authors of the German recommendations.

This document aims to support physician and patient advocacy with policy-makers with regards to the implementation of programs for a better diagnosis and treatment of PID. This initiative originated from Jorgo Chatzimarkakis, (ALDE), Member of the European Parliament). In 2010, Chatzimarkakis presented to the European Parliament a working document drafted by a PID Expert Group of physicians, researchers and patients, which emphasized the need to take action at both the EU and Member State level to raise awareness and increase the diagnosis rate of PID and improve access to treatment (*see The Source, Fall 2010*).

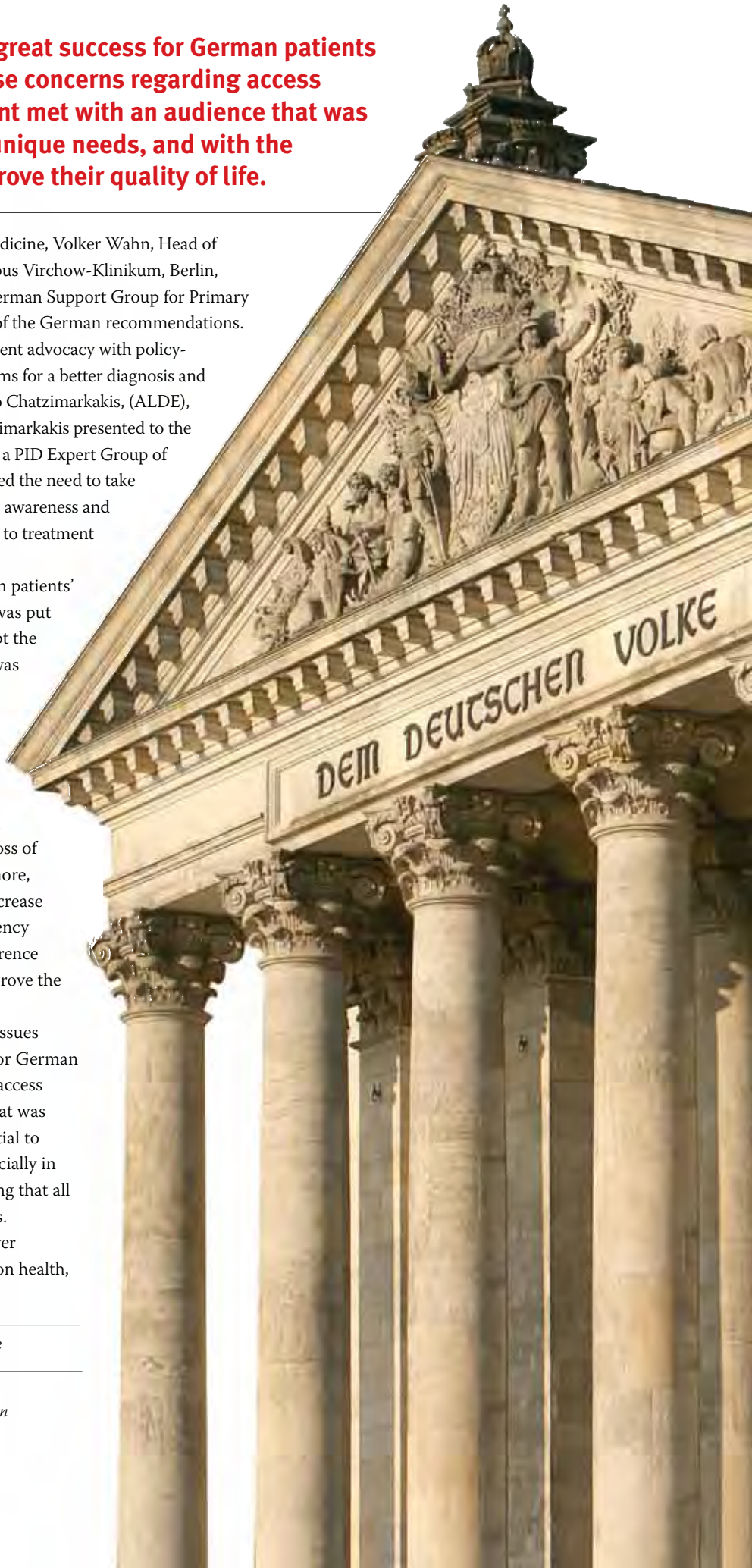
Since then, an expert group composed of German patients' representatives, researchers and leading physicians was put together with the help of the original experts to adapt the European recommendations in Germany. The goal was to maintain the core messages from the European document and to add adapted recommendations to suit the needs of German patients and physicians. The result was a document that addressed issues such as the low diagnosis rate of PID patients in Germany, the lack of proper immunological training at the university level for medical students and the loss of patients from pediatric to adult treatment. Furthermore, the document suggested that German authorities increase support for the European Society for Immunodeficiency (ESID) registry and the creation of a network of reference centers. Finally, the document provided ideas to improve the financing of PID treatment in Germany.

Politicians demonstrated intense interest in the issues presented. This event proved to be a great success for German patients and physicians, whose concerns regarding access to diagnosis and treatment met with an audience that was receptive to their unique needs, and with the potential to improve their quality of life. This is significant, especially in light of the current difficult economy and considering that all European Member States are reducing expenditures.

To date, the document has been distributed to over 400 German regional and state authorities working on health, family and social issues.

Laura Savini, *Manager of National Affairs, Europe*

** The German title of the report is: Empfehlungen für einen besseren Umgang mit angeboren Immundefekten, den Primären Immundefizienzen (PID).*



RJMIGUEL. BAURKA / DREAMSTIME

The Plasma Protein Therapeutics Association Turns Twenty

1973

- **American Blood Resources Association** (ABRA) formed.

1991

- **Quality Plasma Program (QPP)**. Ensured quality is measurable and verifiable.

1992

- **International Plasma Products Industry Association (IPPIA)** formed to represent plasma protein therapeutics industry.

1993

- **National Donor Deferral Registry (NDDR)** established and used by all certified facilities. All donors to who test "reactive" to defined pathogens are permanently prohibited from donating source plasma at licensed and certified centers the U.S. and Canada and added to database. Administered by, Haemonetics.
- Short lived **European Plasma Product Manufacturers (EPPM)** formed with goal of serving as industry voice.

1994

- **European Association of Plasma Products Industry (EAPPI)**

Our industry has made remarkable progress in the past two decades. Much of that success can be attributed to the Plasma Protein Therapeutics Association (PPTA), a dynamic industry trade association. PPTA staff and its member companies have worked tirelessly to:

- Develop and implement voluntary industry standards to ensure donor safety and the quality of plasma protein therapies.
- Establishing ongoing dialog and a relationship of mutual trust and respect with regulators.
- Advocate on behalf of patients who depend on access to lifesaving, plasma protein therapies.

Today, PPTA is respected worldwide. Our dedication to saving and improving lives never wavers. It is in that spirit, that we continue to work on advances that benefit our members and the patients who depend on our therapies.

1998

- **International Plasma Products Industry Association (IPPIA) North America** formed
- Start of activity in Japan

- **Launched** Patient Notification System (PNS) in U.S.

2000

- **EAPP**I and **IPPIA** merged to form **PPTA**

PPTA Plasma Protein Therapeutics Association

- **Launched** French version of PNS in Canada

2001

- **Quality Standards of Excellence,** Assurance and Leadership (QSEAL) established. Voluntary Standards that go beyond regulatory requirements and help define the regulations as they apply to fractionation of plasma for plasma therapeutics. These Voluntary Standards relate to collecting, processing and testing of Source Plasma by member fractionators. In 2000, PPTA established the QSEAL certification program to provide independent certification of adherence by fractionators to the Voluntary Standards.

Quality Standards of Excellence, Assurance and Leadership QSEAL

- First issue of **The Source** published, an international magazine of the plasma protein therapeutics industry.

2002

- **PPTA** merged with **ABRA** and formed **Source Division**
- **ABRA** and **PPTA** merged to form the collection division, **PPTA Source** and create an industry trade association with one voice.

2008

- Web site **Donatingplasma.org** launches. **PPTA** maintains a hostof websites including:

PPTA Global
www.pptaglobal.org

PPTA Deutschland
http://agp-plasmaproteine.de/

PPTA France
http://www.pptafrance.fr/

PPTA Netherlands
http://www.pptanederland.nl/

Donating Plasma
www.donatingplasma.org

Patient Notification System
www.patientnotification systems.org

DREAMSTIME

2012

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Plasma Protein Therapeutics Association

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Invitation only event

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Inside PPTA

NEWS FROM AROUND THE GLOBE

IPOPI HOLDS SECOND PID FORUM AT EU PARLIAMENT

On December 6, 2011, IPOPI held its second PID Forum at the European Parliament focused on Health Technology Assessments (HTAs) and Primary Immunodeficiencies.

The event, hosted by Member of the European Parliament (MEP) Glenis Willmott (UK, PES), was attended by numerous stakeholders including patients, physicians academic representatives, industry experts, and nine MEPs from different political parties. Professor Albert Farrugia, V.P. Global Access and Charles Waller, V.P. Europe represented PPTA. There were three presentations at the meeting, including:

- The Physician Viewpoint on HTAs and PID, Dr. Teresa Espanol
- Patient Involvement in HTA Processes, Brian O'Mahony
- PID Patient's Viewpoint on HTAs, Johan Prevot

These presentations were followed by a productive discussions which led to agreement on several action steps including the adoption of a set of EU recommendations highlighting the need to take patients' and physician's viewpoints into consideration when performing assessments on life saving therapies or diagnostic tools.

Professor Farrugia outlined the issues around HTAs which are of particular concern for patients with rare disorders. In particular, he emphasized the aspects of pharmaco-economic analysis which are detrimental to access. These include arbitrary willingness to pay thresholds, the discounting of benefits to levels which nullify their effects and the lack of suitable utility instruments to elicit accurate Quality Adjusted Life Year (QALY) estimates. (see: *The Global Financial Crisis, The Source Winter 2011*).

continued on page 20

BORISOW / SHUTTERSTOCK, THINKSTOCK

continued from page 19

The set of recommendations will be finalized and circulated.

These will include:

- Member States and the European Commission should make sure that the spirit of the Cross-border Healthcare Directive is respected by ensuring appropriate consultation of stakeholders and therefore patients in HTA processes
- In addition to its policy goals, HTA techniques must have a macro-economic approach and provide clear information on the impact of decisions on patients' quality of life.
- Economic arguments should not be used to limit access to well-established life-saving medicinal products that ultimately will prevent unnecessary expenses such as hospitalization or days-off work/school due to the disease.
- Physicians should be protected from any sort of pressure, including economic considerations aiming at limiting access to life-saving treatments.

Reprinted with permission, International Patient Organisation for Primary Immunodeficiencies (IPOPI).

PLASMA USERS GROUP CONVENES

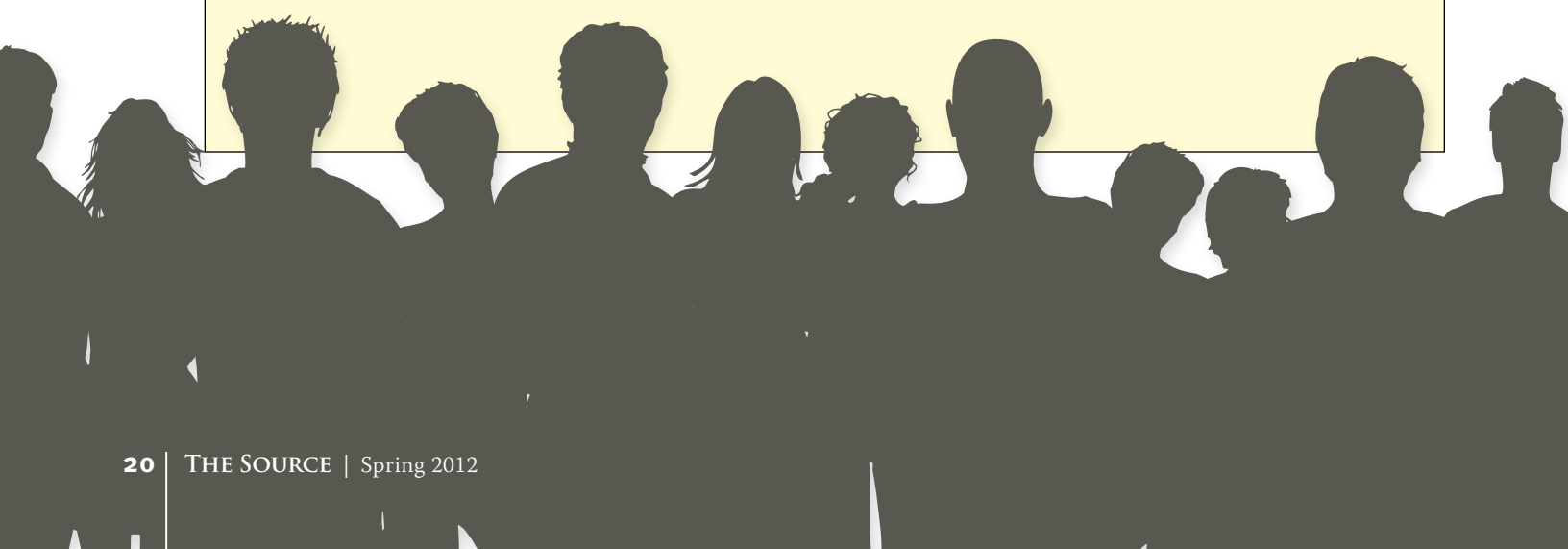
PLUS
Platform of Plasma Protein Users

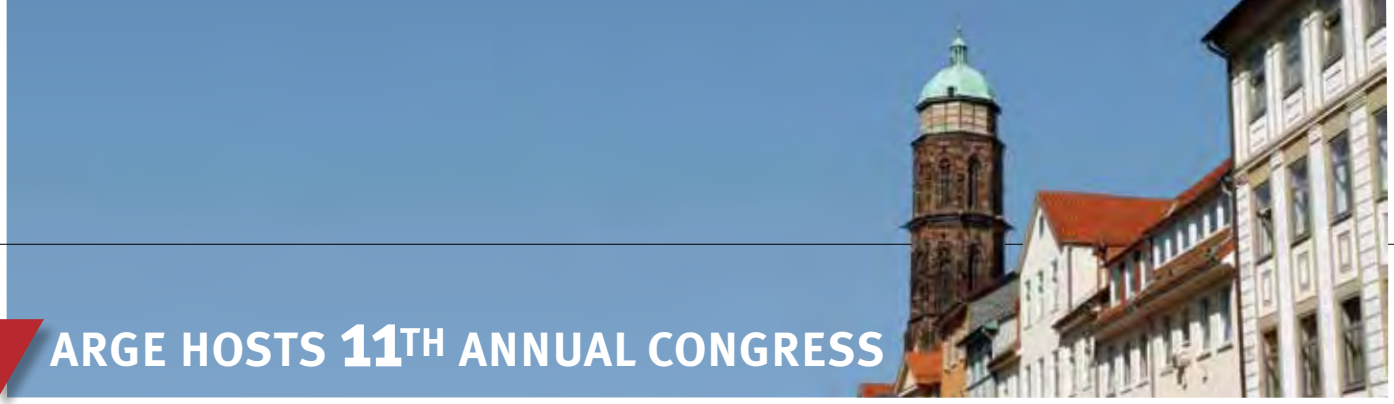
In January, the Plasma Users Group (PLUS) convened their third annual meeting in Ireland. Invited participants included representatives from a variety of patient organizations: hemophilia, immune deficiencies and alpha-1 Anti-Trypsin deficiency and the North American APLUS Group; as well as, also blood and plasma collectors from Europe, the USA and Canada.

Participants were invited to consider *"Perspectives on Sufficient Supply of Plasma Proteins"* to address patient groups' concerns that access to plasma proteins should be taken more seriously as a safety issue. The prevailing opinion of the meeting supported the patient's concerns that steps to increase the availability of plasma proteins should be prioritized.

The meeting concluded with finalizing a draft statement that participants agreed to present to the organizations they represent for their support.

In 2010 and 2011 the statements agreed upon at the PLUS meeting were published in *Vox Sanguinis*.





ARGE HOSTS 11TH ANNUAL CONGRESS

by Alexa Wetzel and Sybille Beck

The Arbeitsgemeinschaft Plasmapherese e.V. (ARGE) hosted its 11th Annual Congress in Göttingen, Germany on November 18 – 19, 2011. The event is geared exclusively to staff from European plasma collection organizations, and the number of attendees increased significantly to more than 220 participants.

On Friday, there were three parallel breakout sessions. With a repeat performance from last year, the two major aphaeresis machine suppliers organized an "olympic contest" on setting up the machines. There was also a workshop on regulatory and quality issues. Two high-caliber speakers from German authorities - Dr. Sabine Wegehaupt from the Paul Ehrlich Institute (PEI) and Mr. Andreas Meißner from the Regulatory Authority Braunschweig gave their perspectives on plasma center inspections and monitoring practices and provoked a lively discussion. A thought-provoking presentation by Mrs. Ursula Pawlowski from the German Red Cross, regarding their experiences with material defects concluded the session. At dinner that evening, Professor Marcell U. Heim, Chairman of the ARGE Board, presented an award to the winner of the set up contest.

The second day featured presentations on various subjects including plasma collection in Europe, hygiene monitoring, plasma for diagnostic purposes, individualized immunotherapy, a patient report about hereditary angioedema, online communication tools for physicians, communication training, and blood groups. The feedback on the program was outstanding, but two presentations deserve special mention: The member of PPTA's Albumin Task Force, Dr. Hartwig Gajek presented an overview on clinical practice in different plasma protein product classes in current clinical trial studies.

Prof. Dr. Marcell U. Heim, Chairman of the Board of the ARGE Plasmapherese, congratulates the winner of the "set-up" contest.

A former drug squad officer and current consultant for the criminal investigation department, Mr. Ingo Seddig, delivered an overview of drugs in society and how to recognize drug users.

Most presentations can be downloaded (in German) at http://www.arge-plasmapherese.de/

For many years PPTA and ARGE have collaborated on plasma related issues in Germany. The ARGE congress offers a unique platform for exchange among the different types of plasma collectors: the Red Cross, industry, independent and community based centers; as well as, regulators, patients, scientists, physicians and many other stakeholders.

The 12th annual training will take place in Munich, Germany on November 23 – 24, 2012.

Alexa Wetzel, *Assistant* and Sybille Beck, *Assistant Director Source Europe and Germany.*

ANDREAS WEBER / SITOCK

The audience at the ARGE congress on Saturday morning.

EUROPEAN HEALTH PROGRAM 2014–2020

The EC adopted the new Health for Growth proposal which aims to support and complement the work of Member States to develop innovative and sustainable health systems, increasing access to better healthcare for citizens and preventing diseases and cross-border health threats. This program will run from 2014 - 2020 with a budget of €446 million. It is launched within the framework of the smart and inclusive growth objective of Europe 2020 Strategy and constitutes an integral part of the EU's financial priorities for 2014 - 2020.

In its program, the Commission stressed that health is not just a value itself but it plays a key role in achieving economic growth. Only a healthy population can achieve its full economic potential. Further, the health sector is driven by innovation and research and is one of the largest economic sectors in the EU. Thus, by helping sustain the health sector, the Health program will enable increased economic growth and generate jobs in the EU area.

Among the several actions to attain these objectives, the program intends to:

- Support cooperation on Health Technology Assessment (HTA) with an EU-wide voluntary network of Member States' HTA agencies to share information on the effectiveness of health technologies such as medicines. This will not only reduce duplication and pool expertise, but can also unlock the potential for sustainable innovation in health products and services. Moreover, being conscious that health-related investments under the Structural Funds can be significant in helping Member States reform their health systems, the cooperation and synergies between the "Health for Growth" program and the Structural funds will be reinforced;
- Strengthen cooperation on rare diseases at European level to improve prevention, diagnosis and treatment for patients with rare diseases across the EU. This will include the creation of European Reference Networks, information and registries based on common accreditation criteria. Additionally there will be a focus on chronic diseases and related research and new Europe-wide guidelines.
- Promote among other things measures setting high standards of safety, quality and efficacy of blood, organs, tissues and cells.

This proposal will now be discussed by the European Parliament and Council of Ministers with a view to adoption by the end of 2013, and the start of the new health program in 2014.

GLOSSARY OF TERMS

ABRA	American Blood Resources Association
EAPPI	European Association of the Plasma Products Industry
EDQM	European Directorate for the Quality of Medicines
EMA	European Medicines Agency
ESID	European Society for Immunodeficiency
EC	European Commission
EU	European Union
FDA	U.S. Food and Drug Administration
GDR	German Democratic Republic
HTA	Health Technology Assessment
IDF	Immune Deficiency Foundation
IPOPI	International Patient Organisation for Primary Immunodeficiencies
IPPIA	International Plasma Products Industry Association
IQPP	International Quality Plasma Program
NDDR	National Donor Deferral Registry
PEI	Paul Ehrlich Institute
PID	Primary Immunodeficiency
PLUS	Plasma Users Group
PMF	Plasma Master File
QALY	Quality Adjusted Life Year
QSEAL	Quality Standards of Excellence, Assurance and Leadership

Mike McCormick

Director of Information Systems and Facilities

How long have you served at PPTA?

I was originally hired by Program Management Group (PMG) in October 1997, as a member of the operations team, which served the three former associations of ABRA, IPPIA and EAPPI. PPTA was born out of the merger of those associations and the dissolution of PMG. This is my 15th year working with a very diverse, talented staff at PPTA.

What do you focus on in your role as Director of Information Systems and Facilities?

Like many on the PPTA team, I wear a few different hats. In a nutshell, my main focus is to make sure that their staff members have effective technology tools necessary to get the job done in a safe and comfortable office environment.

I am responsible for the technology in both the US and Europe offices including: phone systems, security systems, wireless smart phones, email server, file/print servers, PC hardware, PC software, routers, secured wireless access points, LCD projectors, copy/print/scan hardware, internet access, spam filters, firewalls and now the next new tool – tablets (eg. iPAD). The biggest challenge with technology is keeping up with the fast pace of that industry, which is now able to produce the next new technological tool in the blink of an eye. There is never a dull moment.

I also oversee the management of office facilities which include office space leases, office space renovations, and in general, dealing with typical property management issues. Occasionally, I have been called upon to catch wild critters that wander into the office and need to be removed (snakes, mice, spiders, lizards and various others). I guess it's the downside to occupying the ground floor of the building in the US office.

Tell us about your background.

I was raised in the Washington, D.C. metro area and I am one of four children. I graduated from Villanova University in 1986, with a BS degree in business administration and a concentration in computer studies. At the time, there were no formal degree programs in computer information systems. In fact, there was no public access to the Internet. I also played collegiate soccer. After graduation, I settled in Annapolis, MD, worked in finance and continued my computer studies at the local community college.

After leaving finance, I worked for a software company in Baltimore, MD to pursue my interest in the computer and technology arena. During that time, I met my wife Julie. We were married in September 1990 and our first son, Andrew was born in 1993.

I then accepted a job as the Assistant Network Administrator for a prominent boarding school in the Washington, DC metro area. The evolution of the Internet caused things to move quickly in the computer technology world. I can remember the first time I connected to Internet using Netscape. It was only a text based medium at that time, no pictures or graphics at all. I also remember when we rolled out Microsoft Windows 95, which was a major leap at that time. I also had teaching responsibilities at the school. I taught 9th grade Computer Studies, Algebra and I was coach of the freshmen basketball team.

A long daily commute and the birth of my second son, James, in 1997 led me to search for work closer to home and I landed the position of Manager of Information Systems and Facilities at PPTA.

What is your proudest professional achievement?

Fifteen years with one organization, and being promoted to Director of Information Systems.

What is most rewarding about working in this industry?

There is no greater feeling than helping someone in their time of need. Knowing that, although indirectly from my position, we (PPTA) are working to make a positive difference in the lives of those less fortunate that require life-saving therapies is in the end what fuels the entire PPTA team.

EVENTS

UPCOMING CONFERENCES & SYMPOSIUMS

2012

March 8 – 11	Second ASID Congress of the African Society for Immunodeficiencies *Hammamet, Tunisia*
March 13 – 14	International Plasma Protein Congress (IPPC) *Madrid, Spain*
March 20 – 23	32nd International Symposium on Intensive Care and Emergency Medicine *Brussels, Belgium*
June 16 – 20	European Academy of Allergy and Clinical Immunology Congress 2012 *Geneva, Switzerland*
June 21 – 22	Plasma Protein Forum *Washington, D.C., United States*
July 7 – 12	XXXII International Congress of the ISBT *Cancun, Mexico*
July 8 – 12	World Federation of Hemophilia, World Congress *Paris, France*
October 3 – 6	15th Biennial Meeting of the European Society for Immunodeficiencies (ESID) Joint meeting with International Patient Organisation of Primary Immunodeficiencies *(IPOPI) and The International Nursing Group for Immunodeficiencies (INGID)* *Florence, Italy*
October 6 – 9	AABB Annual Meeting *Boston, Massachusetts*
October 6 – 9	Source Business Forum *Boston, Massachusetts*
October 13 – 17	The European Society of Intensive Care Medicine Annual Congress *Lisbon, Portugal*
October 26 – 28	European Haemophilia Consortium Conference, 25th Jubilee *Prague, Czech Republic*
November 8 – 10	National Hemophilia Foundation, 64th Annual Meeting *Orlando, Florida, United States*

Plaza Mayor (shown here) was built during the Habsburg period and is a central plaza in the city of Madrid.

KELVINJAY / ISTOCK

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