

The source

The Newsmagazine of the Plasma Protein Therapeutics Industry



IPPC 2007
Global Supply of Immunoglobulins
Role of Nurses in PID Care

2007 PLASMA PROTEIN FORUM



5-6 JUNE 2007
Hyatt Regency Reston
Reston, VA

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Home

You are cordially invited to attend the Plasma Protein Forum (PPF) 2007 at the Hyatt Regency Reston. Millions of people worldwide rely on plasma-derived and recombinant analog therapies to improve and save their lives. Consumers, regulators and policymakers, physicians, caregivers and industry representatives strive to create a market that supports innovation, quality and access. These groups will come together to discuss the key issues facing the industry today and in the future during the 2007 Forum.

The industry continues to be influenced by national and international dynamics. The 2007 meeting will address policies and regulations that impact consumers' access to life-saving therapies in thought-provoking and engaging sessions. Now, more than ever, the plasma protein therapeutics community must work together to assure patient access. This commitment to the community is steadfast and inspires new solutions to the challenges of today and tomorrow.

Highlights of the 2007 PPF include panel discussions on the future of Source plasma collection; Balancing the Perfect with the Practical: IVIG Analytes; fundamentals of access; and the return of the popular "Q and A" session with regulators. Additional sessions will highlight challenges faced by consumers and hopefully offer some solutions to assuring patient access to lifesaving plasma protein therapies. There is no better opportunity to meet, network, and conduct business related to the plasma protein therapeutics industry. [Register now](#) and together let's continue to demonstrate our collective "Commitment to the Community!"

Best regards,

Julie A. Birkofer
Vice President
PPTA North America

Please visit: www.plasmaproteinforum.com

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

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The QSEAL Standard

These companies have been awarded the QSEAL status. The PPTA QSEAL program is a promise to stakeholders worldwide, that the highest levels of care were invested into the production of these unique lifesaving therapies.

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In my View

Most plasma protein therapies are manufactured from human plasma, a precious source material collected from committed donors. Some therapies are manufactured with alternative (complex) technologies e.g. the recombinant clotting factors. Plasma protein therapies are very important for many people who depend on them. Not only is the quality of life enhanced; in many cases the therapies save lives.

The manufacturing process is very complex and lengthy. The time between collection and distribution of the final therapy to the user can extend to approximately 200 days. The manufacturing process is well controlled and performed according to current Good Manufacturing Practices (GMP). There are many checks in place by the manufacturers and strict Standard Operating Procedures (SOP) are followed.

The plasma protein therapies industry is pervasively regulated. Many regulatory agencies in the world, like the US Food and Drug Administration (FDA), European Medicines Agency (EMA), Ministry of Health Labor and Welfare (MHLW) in Japan, the Therapeutics Goods Administration (TGA) in Australia and the Paul Ehrlich Institute regulate this industry and use their experts to evaluate the information provided by the manufacturers in their dossiers. If all the information is checked and meets the criteria set by the regulators, a marketing authorization is

granted and the therapies can be offered to the medical community.

The above list of regulatory agencies is not complete. There are many more. One can only imagine how wonderful it would be if all the regulatory requirements were the same, and therapies could be shipped from one country to another without additional regulatory requirements. Harmonization is the goal but not today's reality.

Sometimes it is very hard for a manufacturer to predict what the requirements are. It is not uncommon for a regulatory agency to increase the regulatory burden during the process of a marketing authorization application. Specific technical requirements are changed with the justification that change reflects science and the agency's "current thinking". Sometimes companies give in to that regulatory pressure because they want to market their therapy and want to start recovering their very expensive development costs. It is almost impossible for an individual company to say no to such a "new" requirement. That is when the Association can help if such situations of regulatory "encroachment" are communicated.

PPTA will act for and on behalf of their members in cases like this.



Jan M. Bult / President, PPTA



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we are
reinventing
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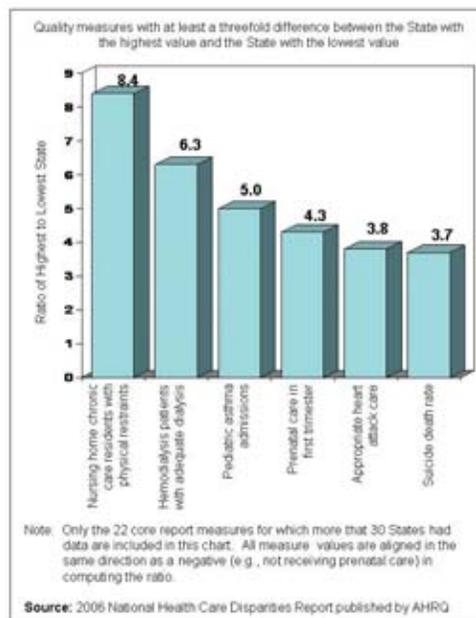
PPTA Committed to Quality and Saving Lives

By Ryan Faden

Background

In the U.S. health care system, extensive gaps in health care often exist between the care that is recommended and the care that patients actually receive. These gaps occur across all disease states and vary from state to state. In some instances, the care that is delivered to patients does not meet the accepted standards of quality for a given condition. As a result, people suffer from medical complications that could be prevented, hospitalizations that could be avoided, decreased quality of life, disability, and in some cases, shortened life expectancy. These complications cost the U.S. health care system billions of dollars per year in additional expenses. These variations in care delivery are of particular importance with respect to the provision of plasma protein therapeutics.

The Agency for Healthcare Research and Quality (AHRQ) is the primary U.S. Federal agency supporting research into the quality, cost effectiveness, and safety of health care. (See www.ahrq.gov). In 2003, AHRQ released the first ever *National Healthcare Quality Report* (NHQR) and *National Healthcare Disparities Report* (NHDR). Since 2003, reports have been issued each year. These reports, mandated by Congress, collect and analyze national and, where available, state-level data to measure health care quality and health disparities in the U.S. The data in the NHQR and NHDR indicate that the gap between health care research and practice is not just an occasional occurrence, but is pervasive throughout the various facets of the health care system. This disparity impacts all patient groups, from those with common medical conditions to those with more complex conditions. A central theme of the reports is to encourage health policy leaders and health care professionals to consider ways to improve the quality of care in the United States. For illustrative purposes, please see the following chart which shows disparities in health care by state across some common health care conditions. This chart depicts the substantial variation between states in key quality measures. The takeaway message is that quality of care is much better in some states than others. This data suggests, by analogy, that there exists an opportunity for action by the plasma protein therapeutics community to work with state officials to address deficiencies in quality of care for this vulnerable population.



Quality of Care for Plasma Protein Therapies

Therapies manufactured from human plasma, and their recombinant analogs, (collectively, “plasma protein therapies”) are used to treat a variety of life threatening diseases and serious medical conditions. Intravenously infused plasma protein therapies include blood clotting factors for the treatment of people with hemophilia and other bleeding disorders, immune globulin intravenous (IGIV) used to prevent infections in people with immune deficiencies and other serious conditions, and alpha-1 proteinase inhibitor used to treat people with alpha-1 antitrypsin deficiency, also known as genetic emphysema. It is estimated that over 1,000,000 people worldwide receive plasma protein therapeutics each year.

In the U.S., state government officials must recognize the unique nature of plasma protein therapies and focus on creating an environment that preserves patient access to the appropriate therapy in the setting most appropriate to each beneficiary’s unique health care needs. Accordingly, PPTA recommends avoiding state health care utilization controls like prior authorization programs, clustered pricing, single source provider contracts, and step therapy protocols which are barriers to access for individuals who utilize plasma protein therapies. For consumers of plasma protein therapies, access to the appropriate therapies from the appropriate providers constitutes a vital first step in

ensuring quality of care. This access question carries much more weight for consumers of plasma protein therapies than for other traditional pharmaceuticals and biologics.

Specifically, plasma protein therapies are not interchangeable, and there are no generic substitutes. Individual therapies are approved by the FDA for specific clinical indications. The needs of each patient are unique, and patients respond to the same treatment differently. The ability to tolerate a specific treatment over time may also change. Accordingly, patients, with the support of their physician, must have access to the full range of plasma protein therapies to assure proper patient care and treatment. Furthermore, these are highly fragile populations that require specialized care. For example, even though hematologists typically treat persons with hemophilia, many hematologists have limited experience with treating patients with the condition.

Ultimately, quality improvement occurs at the nexus of health care between professionals supplying care and consumers who actually need care to treat their conditions. However, state leaders, including elected officials, health care agency officials and the patient community can be accelerants for changes in health care by supporting and encouraging quality improvement to improve health outcomes, reduce the burden of disease, and increase the efficiency of the health care system.

In developing quality of care improvement approaches, the AHRQ has developed five key considerations:

- Understanding any established outcomes measures for tracking the quality of care for a particular disease state
- Comparing state data with national benchmarks and identifying any gaps in state data
- Develop an inventory of data systems available at state and local levels
- Using any published studies to arrive at state or local estimates, and;
- Calculating the direct and indirect costs for State Medicaid programs and other providers if available.

These considerations could form the basis for developing data to support quality of care improvement mechanisms in individual states.

Specific Recent PPTA Activities

PPTA has been working in conjunction with its member companies and partners in the consumer communities to raise awareness of the importance of quality of care for several years now. A key focal point in PPTA's perspective on quality is the linkage between access and quality. Taking this one step further, there is also an important linkage between access and safety. PPTA remains committed to working with consumer organizations to ensure that

both public and private payors are aware of the adverse impacts of economics driven health care decisions on quality of care. In fact, the fragile nature of this population, as well as the documented differences in patient tolerance of plasma protein therapies exacerbates the potential impact of cost-based decisions on overall quality of care and the overall health and safety of the patient. PPTA views quality of care legislation as an approach that counters the effects of broader cost-containment mechanisms at the state level. Such laws would help ensure that consumers continue to have access to the full range of therapies from the appropriate providers as determined by patients and their physicians, and not merely based upon the least costly alternatives.

Advancing quality of care legislation requires that PPTA and its member companies work closely with representatives of the consumer community at both the national and state levels. Because of the variations that have been discussed above in state care systems, any approach would have to be tailored to the specific needs of a given state population. The only way that those needs can be addressed is through a grassroots approach involving consumer group representatives.

To this end, PPTA has held two meetings with member companies and key consumer group allies in 2007 focused on quality of care. Through these meetings and an associated comment process, PPTA has built significant consensus around draft model legislative language and identified strategies moving forward. PPTA has also led efforts aimed at raising awareness as to the importance of quality of care legislation by working to enact proclamations and resolutions in Minnesota, Florida and Maine calling for increased awareness of the importance of access to quality care for patients who utilize plasma protein therapies. PPTA views the proclamations and resolutions as an important incremental step in laying the groundwork for legislation addressing quality of care for the plasma protein therapies community.

Conclusions

Research conducted by the U.S. government and others has repeatedly shown wide variation in the quality of care received by patients in the health care system. In the context of plasma protein therapies, PPTA believes that access to the full range of life-saving plasma protein therapies from the appropriate providers is a fundamental aspect of ensuring high quality care. PPTA plans to continue its efforts in working with the consumer community in the states to educate state officials about the importance of quality of care. The ultimate goal of this approach is to impact health care policies in both the public and private sector to ensure that quality is an important priority in the treatment of consumers who utilize plasma protein therapies.

By Johan Prévot

This year's International Plasma Protein Congress (IPPC) saw yet another record breaking number of attendees. Over 320 participants from 32 different countries descended on Vienna, Austria for an event summarised by one delegate as having offered "good and valuable presentations, excellent attendance and networking, wonderful location".

The Congress took place over two days and was opened by Mr. Charles Waller, Vice President Europe, PPTA. The opening session started with a presentation from Mr. Clemens Auer representing the Austrian Ministry of Health, which provided participants with a detailed overview of Austria's leading role in the field of plasma protein therapies and plasma collection. Mr. Mark Skinner, from the World Federation of Hemophilia (WFH), was next to take the stage updating participants on the WFH's "Treatment for all" campaign. Participants were reminded that unfortunately even today 70% of people with hemophilia are not diagnosed and 75% do not get any treatment, which sadly means that many of them will die young or grow up with severe disabilities. Mr. Skinner explained that the main objectives of the campaign were to ensure safe and effective treatment products are available for all people with inherited bleeding disorders; to ensure that proper diagnosis, management and care by multidisciplinary teams of trained specialists are available and to expand services beyond hemophilia to those with von Willebrand disease, rare factor deficiencies and inherited platelet disorders. Participants were informed that the next WFH World Congress will be held in Istanbul, Turkey on 1-5 June 2008 whilst the 5th WFH Global Forum will take place in Montreal, Canada on 24-25 September 2007. In his conclusion, Mr. Skinner stressed the importance of finding a cure for hemophilia.

The second session of congress day one, which was chaired by Dr. Eva Bastida, Grifols, looked at 'Global Developments'. Prof. Dr. Vladimir M. Gorodetsky gave the first presentation of the session focusing on 'Manufacturing and transfusion of plasma and its derivatives in Russia'. Dr. Gorodetsky provided participants with an overview of key developments in

Russia such as plasma donation patterns, manufacturing capacities, viral safety and import of coagulation factors. Dr. Surjit Singh from Chandigarh Hospital, India, spoke about "Healthcare developments in India". In his presentation, Dr. Singh went through the main healthcare and social issues confronting people in India such as child malnutrition. Importantly, he stressed that whilst tertiary care in India was very well developed with state of the art facilities and knowledge for molecular diagnosis, organ transplantation and micro-surgery, primary and secondary care were unfortunately lagging behind. Hygiene and public health, immunization coverage, infant mortality, malnutrition, diarrhoea and pneumonia are still significant issues confronting the Indian population, he pointed out. In concluding, Dr. Singh highlighted the wide disparities in availability of healthcare resources, the poor health insurance coverage and the need to strengthen primary healthcare. (see interview with Dr Singh on page 16) Mr. Jan Bult, President of PPTA, closed session 2 with a presentation on "Global Developments". Mr. Bult talked about global developments from four different perspectives: patient, clinical, regulatory and industry. Mr. Bult emphasised the important differences between the traditional pharmaceutical industry and the plasma protein therapeutics industry linked to the biological origin of the raw material (plasma) and the unique production cost structure.



Prof. Dr. Vladimir M. Gorodetsky

The third session on "Accessing care for small population" was chaired by Ms. Julie Birkofer, Vice-President North America, PPTA. The session started with a presentation by Mr. Larry Warren, President of Alpha-One Europe. Mr. Warren pointed out that Alpha-One Europe strives to increase awareness of alpha-one antitrypsin deficiencies and lobbies for improvement of diagnosis and assistance to patients. Mr. Warren mentioned the increasing importance given to 'rare diseases' by the EU Institutions and the need to actively represent the Alpha-One community at EU level. Ms. Christina Mayer, Austrian Foundation of Alpha One Deficiency Syndrome, was next to speak on behalf of the Alpha one community providing del-

egates with a moving personal testimony of living with alpha-one deficiency. Ms. Mayer stressed the importance of early diagnosis for alpha-1 patients and the adequate choice of treatment to improve quality of life. The main activities of the Austrian Foundation were also outlined. The third World Congress of Alpha-One Antitrypsin Deficiency Patients will be held in Rome, Italy on 28-30 September 2007.



Mr. Larry Warren

Dr. Bodo Grimbacher, European Society for Immunodeficiencies (ESID) gave an update on the progress of the ESID online patient and research database which was launched in August 2004. Participants were provided with data on quality of life and days missed at school, clearly indicating the significant improvements in patients treated with immunoglobulin replacement therapy. "Demonstrating efficacy for small patient populations" was the topic explored by Dr. Manfred Haase, Paul-Ehrlich-Institut. Dr. Haase reviewed the mandate and objectives of the EMEA's Blood Products Working Party (BPWP) and the key components of the EMEA's Guideline on Clinical Trials in Small Population. Dr. Haase concluded with some examples of products with limited clinical data for which marketing authorisations were granted under exceptional circumstances.

Day one of the IPPC closed with a session focusing on regulatory developments, chaired by Dr. Mirella Calcinai, Kedrion. Dr. Jean-Marc Spieser, European Department for the Quality of Medicines (EDQM), discussed the role of the EDQM in global harmonization. The new extended activities in healthcare pertaining to blood transfusion and organ transplantation recently transferred to the EDQM were outlined in detail. Dr. Spieser highlighted the importance of EDQM's collaboration with key stakeholders such as the European Commission, the European Medicines Agency, EU Member States, the World Health Organisation, the USA, Canada and Australia. Dr. Albert Farrugia, Therapeutic Goods Administration, Australia, presented on the topic "Is Regulation Hindering Global Availability of Plasma?". Dr. Farrugia provided an overview of the challenges facing regulatory agencies. He stressed that whilst some of these challenges influence their decision making so as to impede access, regulations do confer benefits. He concluded that the signs for increasing harmonization were hopeful if tampered by reality. In his presentation on "Alternatives to Randomized Clinical Trials", Dr. Hartwig Gajek, Baxter BioScience, pointed out that whilst randomized clinical

trials (RCTs) were considered as the gold standard for clinical drug development, they had a number of severe limitations in small and heterogenous populations. Well-designed observational studies are able to produce evidence at a level comparable to RCTs and single-subject design is particularly useful in identifying individually optimised treatment regimens, he explained. Mrs. Glenda Silvester, European Medicines Agency (EMA) gave the last presentation of congress day one on the EMA approach to International harmonization. She stressed the benefits of the EU Enlargement and of the EMA's international collaboration with other regulatory authorities.

After an invigorating first day, IPPC attendees were then invited to attend the Hilfenhaus Award cocktail reception at Vienna's Palais Ferstel which was followed by a gala dinner. (For details on the Hilfenhaus award ceremony please see page 10)

Congress day two opened with session 5 on "Collecting Plasma for Fractionation", chaired by Dr. Michaela Rethwilm, Haema AG. Mr. Patrick Robert was first to start with a presentation providing a 5-year outlook on plasma volume demand. In order to meet plasma volume demand which is driven by IVIG by 2012, it will be necessary to either further improve production yields or collect an additional 3.6 million liters of plasma increasing the current global volume from 21.6 million liters to 25.2 million liters, Mr. Robert concluded. Mr. Shinji Wada, CEO of Biomat USA, concluded that from proven experience the plasma industry will be capable in collecting sufficient source plasma to meet near term global demand and that an increase in fractionation capacity will be required to support the next phase of growth of global demand. Ms. Ileana Cramer, Nabi Biopharmaceuticals, provided a presentation on "Mastering Donor Recruitment", exploring various topics such as the reasons behind donating to recruitment and retention of donors, incentive programs and customer service in plasma collection centers. Dr. Jürgen Wallner, from the University of Vienna followed with a presentation on the ethics of donating plasma. Dr. Wallner touched upon issues such as the symbolic power of blood, empirical results concerning motivation and ethical models of donating.

Ms. Betty Van Zant, Talecris Biotherapeutics chaired session 6 which looked at the "Challenges of Contract Fractionation". Mr. Ian Mumford, Chief Operating Officer at Canadian Blood Services, started off with a presentation on "Challenges of contract fractionation: the Canadian Experience". Contract fractionation of Canadian plasma is a key part of the strategic approach to security of supply for plasma protein products, he highlighted. In his conclusions Mr. Mumford stressed that collecting enough plasma to attain the desired level of plasma product sufficiency requires organizational creativity and focus and that diversifying the supplier base where possible ensures access to both commercial and contract-fractionated products. Mr. Uwe E. Jocham, CSL Behring, took participants through the technical challenges of contract fractionation, amongst which he reviewed customer specific requirements and their implementation and the requirements of contract fractionators. The World Health Organisation (WHO)'s perspective on contract fractionation was outlined by Dr. Ana Padilla, WHO. Dr. Padilla explained that the use of local plasma for contract fractionation to improve supply of plasma products in developing countries is a legitimate option as well as an opportunity to have access to plasma derivatives and will have a high positive impact on public health. However, compliance with GMP and building up appropriate technical expertise at local level as well as the formation of regional regulatory networks are highly important aspects that need to be implemented, she stressed. Dr. Johannes Kurz, Austrian Ministry of Health was the last speaker of the session. In his presentation Dr. Kurz reviewed the regulatory aspects of contract fractionation.

The Congress closed with a session on "Patient Access to Immunoglobulins" chaired by Dr. Helen Chapel from Oxford Radcliffe Hospital, United Kingdom. Prof. Janne Björkander, Ryhov County Hospital, Sweden talked about Primary Immunodeficiencies (PIDs) in Sweden. Delegates were provided with an informative presentation on prevalence rates, diagnostic procedures, and data showing the reduced need for hospital visits and better quality of life when PID patients are treated with immunoglobulin replacement therapy. Prof. Thomas Szucs, University of Zurich, Switzerland, provided a presentation on the pharmacoeconomic perspective. Prof. Szucs pointed out there was until now only limited empirical data available on the pharmacoeconomic evaluation of immunoglobulin treatment, but stressed the benefit of learning from analogies such as pharmacoeconomic studies on hemophilia replacement therapy. Dr. Surjit Singh gave his second presentation of the congress on "Intravenous immunoglobulins: 16 Years of Experience from a Developing Country". Dr. Singh compared the clinical uses for

immunoglobulins in developed countries to those in developing countries. PID patients are regularly diagnosed in India and immunoglobulin treatment is available, but reimbursement coverage remains the main issue for patients in India, he emphasized. In his presentation patient access to care worldwide, Mr. David Watters, International Patient Organisation for Primary Immunodeficiencies (IPOPI), stressed the importance of working together with all stakeholders of the plasma protein therapy community. Mr. Watters updated participants on the outcomes of IPOPI's recent EU PID Consensus Conference in Germany which was co-sponsored by the European Commission and on IPOPI's joint campaign with concerned stakeholders to reinstate immunoglobulins on the World Health Organisation's List of Essential Medicines. The last presentation of the Congress was given by Dr. Jacqueline Kerr from the Paul-Ehrlich-Institut who provided the European perspectives on IVIG usage. Amongst other topics Dr. Kerr updated participants on the currently ongoing revision of the IVIG guideline and core SPC and reviewed potential new indications. The congress was formally closed by Mr. Charles Waller, PPTA.

PPTA would like to extend their gratitude to the leading experts, authorities, patients and industry representatives who presented and provided such an exciting forum for information exchange and discussion, and of course to all the IPPC 2007 delegates.

Information on IPPC 2008 will be available in the coming months at www.ippc2008.com



Ms. Christina Mayer

Ms. Christina Mayer: Update

Christina Mayer who gave a powerful presentation at IPPC 2007 on her personal experience of living with Alpha-1 anti-trypsin deficiency underwent a lung transplant just one week after the congress. The operation was successful and Christina is currently undergoing post-operative physical therapy. Thanks to the transplant, Christina no longer needs to carry an oxygen bottle when she walks and cannot wait to go home and start her new life. Christina is 36 years old and lives with her 11 year old son in a small village near the wine capitol "Wachau", 100km from Vienna. She is the second Chairperson of the Austrian Foundation of Alpha One Deficiency Syndrome and an active member of Alpha-1 Europe.

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Hilfenhaus Award 2007

Every year at the International Plasma Protein Congress (IPPC), PPTA awards the Hilfenhaus award to an individual who the PPTA Europe Board recognizes as having made an outstanding contribution in some way to the provision and access to safe plasma protein therapies.

The Hilfenhaus award is named after Dr. Joachim Hilfenhaus who died prematurely in 1996. Dr. Hilfenhaus was amongst other things Chairman of PPTA's Viral Safety Working Party and he dedicated his efforts to the provision and access to safe plasma protein therapies.

Previous Hilfenhaus award winners include: Prof. Dr. Inger Nielsson from Stockholm, Dr. Florian Horraud from the Pateur Institute in Paris, Prof. Wolfgang Schramm from Munich, Prof. Brackman from the Bonn Haemophilia centre, Dr. Helen Chapel Head of Clinical Immunology in the Nuffield Dept of Medicine at the University of Oxford, Dr. Bruce Evatt from the Centres for Disease Control in Atlanta, USA. Dr. med. Wolfhart Kreuz from the Johann Wolfgang Goethe University in Frankfurt am Main and Prof. Vicente Arroyo Professor of Medicine, Director of the Institute of Digestive and Metabolic Diseases and Chief of the Liver Unit, Hospital Clinic, University of Barcelona.

This year in Vienna, it was an honour to recognise Prof. Reinhold Schmidt for his outstanding contribution to the field of immunology.

Prof. Schmidt is Professor of Medicine & Clinical Immunology and Head & Director of the Department of Clinical Immunology, Hannover Medical School, Germany. His current research focus is on the role of innate immune response in immunodeficiency and the regulatory role of anaphylactic proteins such as C5a on the Fc receptor regulation.

He is a member of the steering committee of the HIV/AIDS Competence Network in Germany. In addition, Prof. Schmidt is President of the German Society for Immunology (DGfI), and a board member of the European Federation of Immunological Societies (EFIS). He also serves as Chairman of the Advisory Board of the Paul Ehrlich Institute and



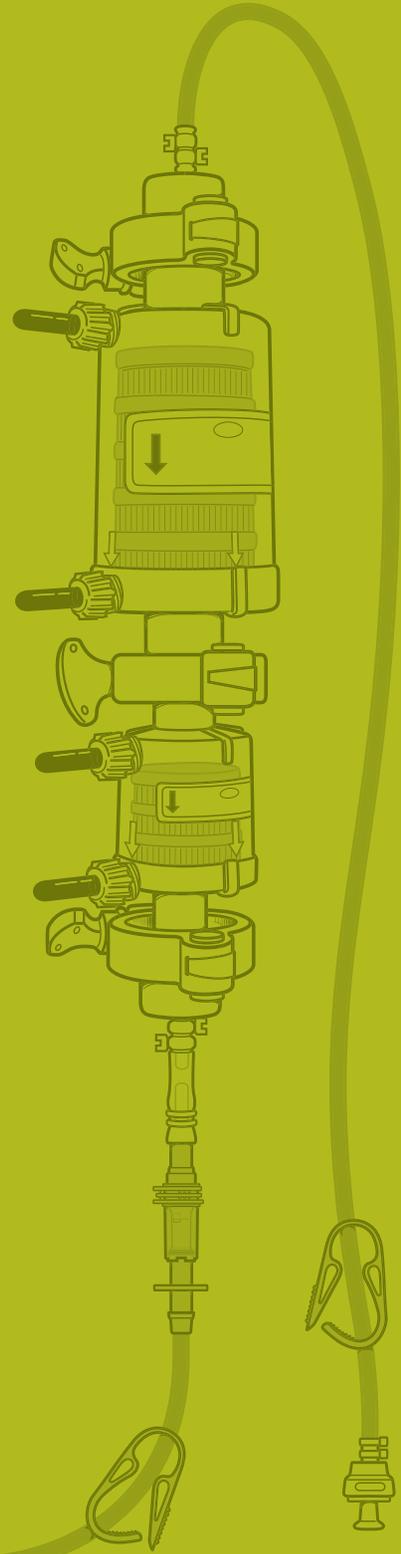
co-Chairman of the Advisory Board of the German Ministry for Social Affairs and Health. He is scientific advisor of the Deutsche Selbsthilfe für angeborene Immundefekte (DSAI). In addition he serves as Dean of the Hannover Biomedical Research School (HBRS).

The award was presented to Prof. Schmidt at a cocktail reception attended by IPPC delegates which was held in the magnificent Palais Ferstel located in one of the oldest parts of Vienna. A gala dinner followed the cocktail reception, in the main hall of the Palais Ferstel with 130 attendees.



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IPPC 2007 Charity Events

This year, for the first time, PPTA organized two charity fundraising events during the International Plasma Protein Congress (IPPC) in Vienna, Austria.

Over 40 delegates braved an early rise to participate in a 1k run/walk on the morning of the second day of the congress. PPTA donated €20 for each participant to SOS Children's Villages and circulated a donation bucket during the congress for those who did not run. A total of €2,200 was raised for this worthy cause.

SOS Children's Villages is the largest private, non-governmental social development organization for children. The first SOS Children's Village was founded in Austria by Hermann Gmeiner in 1949. World War II left a bitter legacy: thousands of orphaned, homeless and traumatized children. Hermann Gmeiner was convinced that, as opposed to traditional institutionalized care, a family-based care environment would ensure a better future for the children. He questioned traditional methods of caring for orphans and for the first time ever established a family-centered approach.

SOS Children's Villages is currently active in 132 countries and territories. 438 SOS Children's Villages and 346 SOS Youth Facilities provide more than 59,000 children and

youths in need with a new home. More than 131,000 children/youths attend SOS Kindergartens, SOS Hermann Gmeiner Schools and SOS Vocational Training Centres. Around 397,000 people benefit from the services provided by SOS Medical Centres, 115,000 people from services provided by SOS Social Centres. SOS Children's Villages also helps in situations of crisis and disaster through emergency relief programmes.

Additionally, evaluation forms were distributed during the course of the congress as PPTA highly values feedback from IPPC delegates on the contents and logistics of the congress as well as suggestions for improvement for upcoming events. PPTA donated €20 for each completed evaluation form to Dr. Surjit Singh (IPPC 2007 presenter) from Chandigarh Hospital in India. A total of €2,300 was raised. Dr Singh who is the additional Professor of Pediatric Allergy and Immunology, Department of Pediatrics, Advanced Pediatric Centre at the Chandigarh Hospital's Post Graduate Institute of Medical Education and Research (PGIMER) requested that the money be donated to the Indian Patients Society for Primary Immunodeficiency (IPSPI).

IPSPI was set up by Mr. and Mrs. Chawla who suffered the tragic loss of two of their sons to Primary Immunodeficiency.



Participants in the Charity Run



The Chawla family

The Chawla's first son endured periodic infections from childhood (ear discharge, skin infections, cold & cough, diarrhea) he was hospitalized on several occasions and misdiagnosed, his condition deteriorated so severely and tragically he succumbed to severe pneumonia at the age of 9. Their second son suffered from recurring skin infections, which at times took the form of eczema. This was treated on and off with a long course of antibiotics. The Chawlas were never advised to test their son's immunoglobulin levels. Sadly, at the age of 4 they lost their second son to a drug allergy. Their remaining son has been correctly diagnosed and is receiving regular IVIG treatment on a monthly basis. He is now 13 years old and is leading a nearly normal life.

The Chawlas are still coming to terms with the nightmare they have lived through. In lieu of the trauma they have gone through they are committed to helping those suffering with Primary Immunodeficiencies (PIDs) and their

families. They do not want others to experience the same tragedy they have. The Chawlas have immense gratitude for Dr. Surjit Singh who helped them through this terrible experience. They are privileged to have him as Honorary Advisor of IPSPI.

IPSPI works to improve the quality of life of people with Primary Immunodeficiency. The main aim and objective of the Society is to secure an early diagnosis and adequate treatment for all those affected with PIDs in current medical standards and to propagate and run programmes and projects for the treatment and education of PIDs. IPSPI is a fully-fledged member of IPOPI (International Patients Organization for Primary Immunodeficiencies). For more information please visit: <http://www.ipspiindia.com/>

PPTA would like to thank all those who contributed to these two fund raising events.

PMF Round Table 2007

By René Büchel

This year, as a curtain raiser to the International Plasma Protein Congress (IPPC), PPTA held a one day Plasma Master File Round Table entitled the "Impact of European Directives on the Plasma Master File".

The aim of the Round Table was to explore the impact of the European Directives on the Plasma Master File (PMF) and to address a range of issues faced by the plasma protein industry in its aim to adhere to the PMF requirements while ensuring safe and effective medicinal products in sufficient quantities to address the needs of those who depend on these life-saving therapies.



Guest speakers at the Round Table

The round table exceeded PPTA's expectations with over 70 participants from regulatory authorities, industry and collection establishments attending. The presentations were followed by a very lively and interactive discussion. The round table provided an ideal setting for regulators, authorities and industry to come together to exchange and discuss the latest topics associated with the PMF and to explore ways of moving forward and achieving an optimal system for all those involved, particularly in order to avoid redundancies and overlap between regulations.

Several leading experts in the field were invited by PPTA to present. Mr. Nicolas Rossignol (DG Enterprise) introduced the audience to European Pharmaceutical legislation and its impact on the PMF: where are we? He summarised the

content and meaning of the Community code and the Pharma legislation which defines the framework of the EMEA's activities. With the publication of Annex 1 the PMF has received a legal basis and the EMEA has been given the task to certify the PMF centrally. This central procedure has the goal to be less redundant and burdensome than having to go through multiple national procedures. The harmonisation of the evaluation can therefore be achieved while still protecting public health. Mr. Rossignol provided a real case example on the efficiency of the PMF system. One PMF has been evaluated once and connected to eleven marketing authorisation holders, representing more than 80 medicinal products in 25 Member States. Currently, 11 PMF holders have received a centralised certification. Some challenges remain: the 2nd Step procedure, the variation system and the yearly changes to Guidelines. Mr. Rossignol concluded that whilst some issues are still problematic particularly with respect to the EU Blood Directive, the outcome of the centralised certification is overall positive.

Mr. Thomas Bregeon (DG Sanco) spoke about the Blood Directives and their interaction with the European Pharmaceutical Law. He made it clear to the audience that the objective of Article 152 on Public Health is health protection and therefore only minimum standards have been established by the Blood Directive 2002/98/EC. On the other hand, Article 95 defines free circulation of goods (internal market) and therefore focus is on harmonisation of the legislation like the Medical Products Directive 2001/83/EC. Mr. Bregeon briefly reviewed the "mother" and "daughter" Directives on traceability, quality systems and



technical requirements. He clearly portrayed the expected interactions between the Blood Directive (affecting the sourcing of plasma) and the pharmaceutical legislation (affecting processing of plasma) and the fact that a grey zone exists creating boundary issues. These issues include inspections, screening, and GMP issues (etc). Some issues remain unresolved but are currently discussed between the different involved organisations – European Commission, EMEA and industry.

Dr. Raffaella Sardelli - Istituto Superiore di Sanità (ISS), provided a case study on Italy and summarized the problems which industry has faced within the last year, due to more stringent implementation of requirements at national level of the EU Blood Directive. Dr. Sardelli provided an insight into how the PMF is managed between the Italian Pharmaceutical Agency (AIFA) and ISS. She detailed the differences between the Blood Directive requirements and the Italian legislation implementing more stringent requirements. Concerning ALT testing, Dr. Sardelli informed the audience that the test will not be carried out on each donation but on each donor with an optimised method thereby allowing industry to suspend ALT testing for plasma for fractionation.

Dr. Gerd Werner (Paul Ehrlich Institut) analysed the different regulations an inspector has to follow and attempted to address the question: If industry can comply with all the rules? He went through the different regulations and Guidelines setting standards for inspections and highlighted the problems faced by plasma centres and blood banks in Europe and the USA to fulfil all the requirements. Dr. Werner briefly spoke on the mutual recognition of inspections and concluded that human plasma for fractionation is one of the most intensively regulated source materials for medicinal products.

Finally, Dr. Silvia Domingo (EMA) summarized the EMA experience after three years of implementing the PMF. She described the legal basis of the PMF certification, and reported on the EMA activities from 2003 – 2006 by taking the audience through the EMA PMF website (www.emea.europa.eu/htms/human/pmf/pmf.htm). Dr.

Domingo addressed the opportunities for improvement and the topics which will be discussed during 2007 – 2008 – harmonisation of evaluations by assessors, review of the epidemiological data, implementation of new variation regulations and follow up to industry feedback.



From Left to right: Dr. Sardelli, Mr. Braun, Dr. Domingo, Dr. Zerlauth, Dr. Kurz, Mr. Rossignol, Mrs. Silvester, Dr. Büchel, Mr. Bregeon, Mr. Petrovsky, Dr. Prusa

Plasma Center Visit

In conjunction with the PMF Round Table, PPTA invited the speakers to visit a plasma center located in Vienna. The delegation was provided with a guided tour of the Center and taken through the full donor process. This was an ideal opportunity to inform authorities on today's state of the art plasma collection facilities.

Interview Dr Surjit Singh

By Johan Prevot

Dr. Singh is an Additional Professor of Pediatric Allergy and Immunology, Department of Pediatrics, Advanced Pediatric Centre at the Post Graduate Institute of Medical Education and Research (PGIMER) in Chandigarh, India. He looks after the Pediatric Allergy and Immunology Unit at the said institute. The center is involved in the care of children with immunological disorders. It is the only specialized pediatric immunology unit in India.

Please tell us a little bit more about your hospital and more specifically about the role of the Pediatric Allergy and Immunology Unit at the Advanced Pediatric Centre (PGIMER)?

PGIMER Chandigarh is a federally funded tertiary level medical institute and caters to northern India. It is counted as one amongst the three leading medical institutes in the country. We have a 1400 bed hospital and have advanced facilities for patient care, teaching and research in most medical specialties. As its name implies, ours is a 'not for profit' organization. We have 350 faculty members. The Advanced Pediatric Centre is the children's wing of the institute. It has 250 beds and has many subspecialty units. The Pediatric Allergy and Immunology Unit started in 1993. It has 15 beds and caters to children with immunological disorders. We run 2 outpatient clinics every week. We have one of the largest registries on Primary Immunodeficiency Disorders in India.

In your recent presentation at the International Plasma Protein Congress in Vienna you spoke about the use of immunoglobulins in India. Can you elaborate on their use in PGIMER and the main conditions which you treat with IGs?

Immunoglobulins have been in regular use in our hospital since 1992. We use these products regularly as immunomodulatory agents (for e.g. in Landry Guillain Barre syndrome, Kawasaki Disease, Immune thrombocytopenic purpura) as well as for replacement therapy in immunodeficiency disorders (for e.g. in X-linked hypogammaglobulinemia, Common variable immunodeficiency and Wiskott Aldrich syndrome).



Dr. Singh presenting at IPPC 2007

In your experience, what are the main challenges faced by patients in need of immunoglobulin in India and how can they be overcome?

The main challenge is the cost of these products and the fact that they are not covered by the government and that most people do not have health insurance. At present the cost of 5 Grams of Intravenous Immunoglobulin is in the range of Rs. 2500-Rs. 3000 (i.e. approx. €8-10 per Gram). While these costs are much lower than those in Europe, the products are still unaffordable for the overwhelming majority of our patients. Most of these products originate from fractionation plants in China and Korea.

More expensive products made by European and American companies are also easily available in India but they cost more than five times as much as the products mentioned above.

What are the existing patient organizations in India supporting patients treated with IGs?

We have the Indian Patients Society for Primary Immunodeficiency (IPSPI) which caters to children with immunodeficiencies. This society is affiliated with the International Patient Organization for Primary Immunodeficiency (IPOPI). In addition, at Chandigarh we have a Kawasaki Awareness Society which looks after children with Kawasaki Disease and facilitates the provision of immunoglobulin for affected patients.



Front view of the new outpatient block at the PGIMER Chandigarh .

At PGIMER, it has been our endeavor that no child with Kawasaki Disease should go untreated irrespective of whether the parents can afford the treatment or not.

How are the costs of IG treatment for patients in India covered?

The majority of patients in India do not have health insurance. The costs of IG treatment, therefore, have necessarily to be borne by the parents themselves. We try to involve philanthropic societies and non-governmental organizations for funding such therapies. While this is relatively easy for situations where only one-time treatment is required (e.g. in Kawasaki Disease and Landry Guillain Barre syndrome), it is quite difficult in conditions where treatment has to be provided life-long on a regular basis (for e.g. in hypogammaglobulinemia).

In your opinion, what is the future for IG treatment in India?

The prices of intravenous immunoglobulin have been consistently showing a downward trend and this is very encouraging. At present most companies are procuring the product from China and Korea and marketing it in India. However, it is likely that some of these companies will soon set up fractionation plants in India itself. Once that happens, I am sure the prices would fall further. Another important aspect is that we hope the government will

recognize the essential nature of immunoglobulins by facilitating access to these therapies and covering treatment costs for patients in need. We are optimistic about the future and are hopeful that our patients with hypogammaglobulinemia would continue getting their therapy as prescribed.



Global Developments in the Supply of Ig

By Charles Waller

What is immunoglobulin?

Human blood contains immunoglobulins of both a protective and immunomodulatory nature. Polyvalent Human Immunoglobulins are prepared from pooled plasma and contains a distribution of antibodies which reflects that in normal human blood. Adequate doses of this medicinal product restore protection against bacterial and viral infections in those with immune deficiencies and provide immune modulation for patients with some diseases caused by auto-antibodies.

Antibodies are Y-shaped proteins that bind to antigens with both upper arms of the Y. Under normal conditions, an antibody binds to an antigen (a bacterium, virus, or other pathogen) and “tags” it for destruction. Antibodies themselves do not harm the bound antigen; instead, they activate the immune system to remove the pathogen. IVIG is composed primarily of IgG, one of five classes of antibodies, which include IgG, IgM, IgA, IgE, and IgD. IgG is the predominant immunoglobulin in plasma and the main source of humoral immunity.

Uniqueness of plasma protein therapies

It is important to recognize that life saving plasma protein therapies, including immunoglobulin, are unique and should not be considered as a typical pharmaceutical product. Some things that make them unique include: use of unique starting material (human plasma) and not a chemical compound, the manufacturing process is complex and can take between 6 and 8 months, manufacturing is costly and very capital intensive, regulatory requirements are extremely robust and the therapies are used by relatively small patient populations. It is important that decision makers understand why these therapies are so unique. It is understandable that authorities want to control health care costs, it is also important to recognize that cutting costs without a full appreciation of how these therapies differ from traditional pharma, may result in undesired (and very negative) consequences for the patients that depend on them.

International clinical need for immunoglobulin has risen annually for the last decade. In the last year the rate of increase seems to have accelerated and while plasma

donations and investment in manufacturing capacity have continued to increase, the long lead time between the donation of plasma to the finished product is resulting in challenges to meet demand. PPTA and its members are committed to meet needs of people whose lives depend on the immunoglobulin and other plasma derived medicinal products. Significant investments have enabled the production of immunoglobulin to increase every year. This article provides some information on significant factors that affect this situation.

Predicting the need of immunoglobulin is something of a challenge, but a supply linked to clinical need and not to product availability must be the goal to which health care providers and governments should focus their decision-making.

There is also an important ethical aspect. Plasma-derived therapies are manufactured from human plasma donated by committed regular donors. It is ethically necessary that this plasma fulfils its potential and can be used as widely as possible and without being subject to arbitrary requirements that lack a scientific basis. Thankfully there are governmental organizations that are playing an important role in providing the basis for harmonization and leadership that is extremely important for biological pharmaceuticals.

There is a shared obligation for all plasma protein therapy stakeholders - regulators, politicians, physicians, patients and industry - to ensure that the full potential of the donated plasma is realized. Industry is working on improving yields and opening new plasma centers. Today, more immunoglobulin is being distributed by the private sector manufacturers than ever before. Regulatory alignment will be another important step in the optimization of resources. It is an ethical duty in the context of plasma-derived medicinal therapies that regulations and policies do not impose new and unnecessary barriers which are wasteful and that could endanger patient access to care. PPTA will continue to work hard to ensure that serious impact on patients can be avoided.

The cost of producing plasma-derived therapies is high compared to chemical-based medicines, with the plasma itself and the related cost of production typically

accounting for about 70% of the purchase price compared to less than 20% for chemical based pharmaceuticals (see Chart 1). Consequently, each manufacturer must individually endeavor to carefully balance production with estimated demand and must maximize the number of therapies it can derive from each litre of plasma. Additionally, manufacturers need to ensure that the “yield” of each therapy from each liter of plasma is maximized. In the last five years significant investment has helped double the amount of immunoglobulin extracted from a liter of plasma to an average of over 3.5 grams per liter.

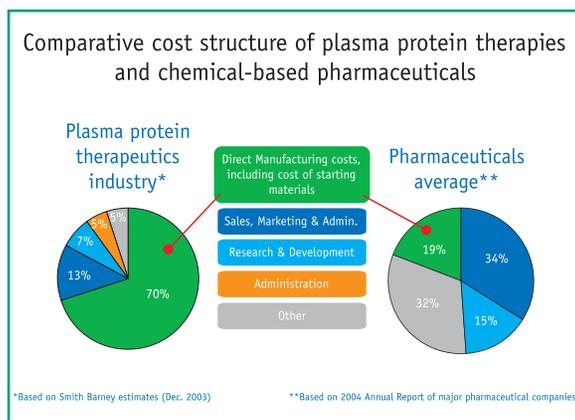


Chart 1

Today, we see the demand for many plasma-derived therapies increasing. This is particularly true for immunoglobulin. An invaluable tool in the immunologist’s armory has been used for some years to treat various immune deficiencies and other conditions for which there is no other therapy, including Kawasaki Disease and Guillain Barre Syndrome (GBS).

GBS is the first recognized neurological condition for which is the first line of therapy. Physicians, encouraged by the work with GBS have used immunoglobulin with increasing success in other neurological conditions and the medical literature on the subject is growing. Most recently at the 10th International Conference on Alzheimer’s Disease in Madrid July 2006¹, the important role of immunoglobulin in the treatment of Alzheimer’s disease was recognized. According to investigators, it appears that IVIg provides reinforcements that help immune cells carry misfolded proteins out of the brain. A pilot study involving seven Alzheimer patients produced such impressive results that the National Institute on Aging (part of the U.S. Department of Health and Human Services) is organizing a larger study². Charts 2 and 3 shows how PPTA member’s investments have helped deliver record quantities of immunoglobulin to meet patients needs in the last six years in Europe and the US. It should be noted that the data for the EU 15 countries refers to the pre 2004 European Union

and excludes production of the public sector fractionators and contract fractionated products which may double the Immunoglobulin actually distributed according to Chart 2. It must be acknowledged that it is vital that as few barriers as possible stand in the way of the free movement of plasma-derived therapies from one well regulated and controlled region or country to another. Countries that insist on local variations in their regulatory requirements impose an unnecessary risk on their fellow citizens. The burden of this falls on the patients and physicians from these countries. If the domestic plasma is negatively impacted, there may be no other plasma that meets the national requirements, even though it complies with requirements of the rest of the worlds leading authorities.

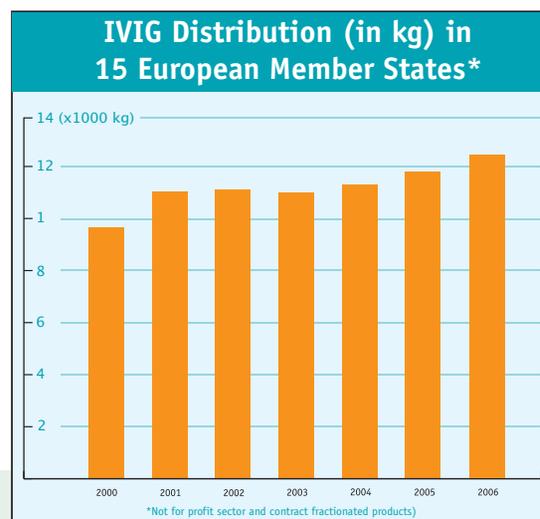


Chart 2

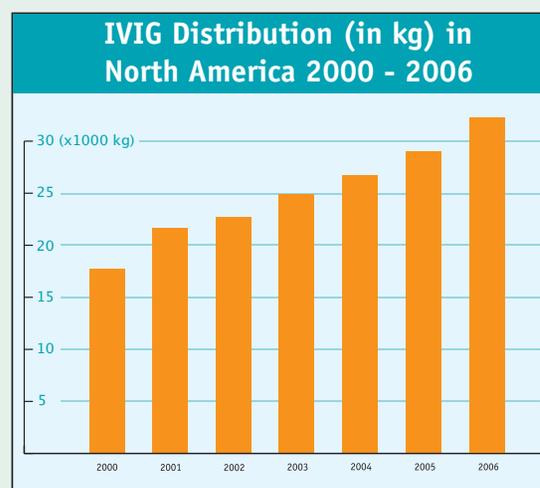


Chart 3

¹ <http://www.alz.org/icad/overview.asp>

² <http://www.nia.nih.gov/alzheimers>

Role of Nurses in PID Care

THE ROLE OF THE NURSE IN RECOGNISING AND CARING FOR PATIENTS WITH PRIMARY ANTIBODY DEFICIENCY DISORDERS

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Primary immunodeficiency disorders (PID), of which there are over 120 different types, are important diseases because of their effects on the patient, the healthcare delivery systems, and society, when they are not diagnosed and successfully treated. Experience over the past 30 years has shown that the earlier the PID is diagnosed and the sooner that appropriate and adequate treatment is commenced, then the better the chances of tremendous improvements in morbidity, mortality, and in health-related quality of life occurring.

This paper sets out to show that the nurse is a crucial link in recognising whether or not a patient has a potential PID, and for setting in motion the referral of that patient for the appropriate tests and treatments. This happens because

nurses are often the initial link with the patient and family, and therefore she/he is often in the unique position of being the first healthcare professional to suspect the presence of a PID. In addition, once the child or the adult is diagnosed, it is most often the nurse that is responsible for educating patients/families who need replacement therapy with immunoglobulin G (IgG) and to give continuous support in order for the patient/family to comply with the lifelong therapy. If the patient should need hospital care, it is the nurse who spends most time with the patients and families.

In some EU countries nurses, after additional education, are allowed to prescribe drugs. Discussions to implement nurse prescriptions are ongoing also in other countries. For example, in the UK, qualified Nurse Independent prescribers are able to prescribe any licensed medicine, including immunoglobulins, for any medical condition that is within their competency. Discussions are ongoing in other countries for the same system to be in place. So it can be seen that the PID nurse is a key person involved in the care of a patient with PID.

Primary immunodeficiency disorders (PID) is a group of chronic diseases, with over 120 genetically distinct subtypes identified^{1,2} - although more causes of these primary immunodeficiencies are continually being identified¹. Some of these primary immunodeficiency disorders are very rare, whilst others are very common. In the European Union alone, about 1,900,000 individuals are calculated to have some form of PID, most often an antibody deficiency³. Depending upon the type of PID, some cause a few or even no symptoms. Alternatively, others can cause major problems, and some can even be fatal.

Nurses important in helping to recognise PID

As far back as 1982, the World Health Organisation (WHO) pointed out that nurses are extremely important in regards to the health of the population because they have the qualifications and the experience to find 'ill-health'. In addition they make up the largest single professional group in the healthcare system and they can be found in all different societies and specialties⁴.

Because PID can cause so many different signs and symptoms, then nurses in all areas of health care can play a vital role in the detection of patients with these disorders⁵⁻⁷. Nurses are often in the front line of health care and will see patients at an early stage of their illness, often with minor problems. Therefore they are often the first person to look at the symptoms and to consider the possibility that there may be a primary immunodeficiency underlying the presenting problem⁶. This is very important, because as the EU PID consensus statement, following the important EU PID Consensus Conference in Frankfurt, 2006, emphasises, the importance of early diagnosis, and state that early identification of PIDs will save lives, improve health, quality of life, and lifespan in identified patients, as well as allowing for genetic counselling and prenatal diagnosis within the family³. In addition, the EU PID consensus statement recognises that, at the moment, there is a lack of awareness of PIDs by front-line healthcare workers, including nurses³.

Nurses spend more time with patients and their families than do any other group of health care professionals. Consequently they are in a position to consider not only the physical signs and symptoms, but also other problems that may lead them to consider the possibility of a PID.

Of particular importance in the diagnosis of a PID is the role of the children's nurse. The reason for this is that most PIDs are present from birth, and so it is essential that children's nurses are aware of this group of disorders⁷.

Why is it important for nurses to recognise PID early?

The earlier a PID is diagnosed and adequately and appropriately treated, the better the long-term prognosis and health-related quality of life (HRQL) for the patient. Any delay in diagnosing and treating a PID will mean that the body can become seriously damaged by recurrent infections, or the patient could even die^{8,9}. Diagnosing and correctly treating patients will significantly improve the HRQL among both adult patients^{5,10-13} and children^{11,12,14} and also lower the costs both for society^{15,16} and for the patient/families¹⁷. In particular, the use of subcutaneous IgG (SCIG) self-infusions at home has been shown to be appreciated by the patients and their families^{10-16,18}, to improve treatment satisfaction¹⁸⁻²⁰, and to lower costs^{5,15-17}.

How does the nurse recognise/diagnose PIDs?

There are several cardinal signs that indicate the possibility of a PID^{7,21}, namely:

- recurrent infections
- chronic infections

infections in unexpected organs and/or at an unexpected age, e.g. recurrent otitis media in adults infections by opportunistic microorganisms (i.e. organisms that are not usually a problem to a healthy person, but are able to take the opportunity of a weak/absent immune system to cause problems, e.g. cytomegalovirus) incomplete response to an infection (i.e. only able to partly fight an infection) reflected by a reduced inflammatory response being manifested, for example, in the lack of normal infection inflammatory parameters in blood tests incomplete clearing of an infection after the use of antibiotics or antiviral/-antifungal drugs.

The close relationship between nurses and their patients and families allows for them to observe all aspects of the patients and families and to bring their knowledge and experience into helping them, in this case by making a potential diagnosis of a PID.

Patients presenting with the signs and symptoms that a PID is present must be referred for further tests. To actually diagnose the type of PID can vary from the simple to the quite complicated, involving many different health-care specialists, depending upon the type of primary immunodeficiency²¹. However, for the patient, almost all of these tests just require a blood sample²².

What is the treatment?

This depends upon the underlying cause of the primary immunodeficiency disorder, and its severity. The majority of PIDs are usually concerned with a deficiency in one particular arm of the immune system known as the B-cell system – or antibody deficiency. An immunodeficiency in this part of the immune system means that the patient is unable to produce sufficient, or indeed any, antibodies (otherwise known as immunoglobulins). There are many primary antibody deficiencies (PADs), including X-linked agammaglobulinaemia (which presents in male infants) and

common variable immunodeficiency (which often does not present itself until adulthood),²³ The first line therapy for children or adults suffering from PAD is IgG administration intravenously²⁴⁻²⁷ or subcutaneously^{2,5,11-16,18,28-30}. In particular, rapid SCIG selfinfusions (20 ml/hour) have a very excellent safety profile, result in high and stable serum IgG levels, and are easy to handle^{2,5,11-16,18,19,28-30}. This makes this method very suitable for self infusions at home by the child/parent or adult patient^{2,5,11,16,18-20}.



Figure 1: The subcutaneous infusions using the abdomen and/or thighs.



Figure 2. Also elderly can easily learn the subcutaneous administration rout.



Figure 3. Using subcutaneous self-infusions at home make the patient mobile and flexible to do what he/she wants (doing paper work, reading, watching TV, cooking, having dinner with the family, playing).

Other treatments for these patients include the early prescribing of antibiotics and other antimicrobial drugs and in some patients the use of prophylactic antibiotics⁷. It is further of utmost importance to educate these patients to achieve good nutrition⁵ and to encourage them to cease indulging in behaviours that are harmful to their health, e.g. smoking¹⁶.

The role of the nurse after diagnosis

The role of the nurse once the child or the adult has been diagnosed include:

- continuous support of the patient and the family - the psychological and social aspects of the nursing care should be considered and special emphasis put on the care support of the entire family^{31,32}
- monitoring and following-up of IgG replacement therapy and the home-therapy
- initiation of medical investigations when needed, education in self-care and early warning signs of infections

An essential nursing responsibility is to set up special patient and family educational and training programmes before transferring patients to home-therapy whether given subcutaneously or intravenously^{16,33}. From research about patient education it is known that it is not sufficient just to train the patient in a technical skill such as using infusion equipment. It is also important to educate the patient and family about the disease itself and why the therapy is important.

A PID educational and training program should include the three following components^{16,33}:

- “Know-that knowledge” including information and education about primary antibody deficiencies (diagnosis, aetiology, prognosis, therapy), the aim and importance of immunoglobulin therapy, infections, systemic adverse reactions, and what to do if a such a reaction occurs at home
- “Know-why knowledge” including knowledge and understanding about how one’s own behaviours affect the disease, therapy and daily life, e.g. smoking habits, nutrition, self-care and prevention, as well as behaviour changes such as allowing time for the weekly infusions
- “Know-how knowledge” including the training and knowledge of how to use the necessary equipment and how to insert the needle, etc. (“skills”).

With the rapidly increased body of knowledge that exists within the PID area, another important responsibility of the nurse is to follow and implement new research findings in the clinical setting and care of the patients and families. It is important to stimulate more PID nurses to become researchers and perhaps to work towards a PhD degree. The first steps in that direction have been taken in the UK with a Master’s immunology degree programme for nurses and other health professionals³⁴. A similar course is also being discussed in Sweden.

A new role as a drug prescriber

In some countries, like the UK, following successful completion of specialist education, the nurse has the right to prescribe certain drugs that a patient may need, among them immunoglobulins. In Sweden district nurses, as well as nurses within elderly care services, are allowed to prescribe specially listed drugs and equipment after taking on an additional course in pharmacology³⁵. However, a discussion is ongoing to allow all nurses the right to prescribe drugs, including nurses working within hospitals, and also to open up the Swedish international book of drugs (FASS)³⁶ to nurse prescribers³⁷.

International collaboration among nurses

The International Nursing Group for Immunodeficiencies (INGID) is dedicated to the education and support of all nurses throughout the world who are working with children and/or adults with PIDs. This is achieved by regular conferences, a dedicated website³⁸, and regular editions of the INGID e-Journal and the INGID e-Newsletter. The international group is very much involved in the EU PID project mentioned earlier, because, as identified above, nurses have such an important role in identifying, educating and supporting the patients. Finally, other international nursing collaborations are presently working to improve the lives of individuals suffering from PIDs³⁹.

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2007 Congressional Update

By Jay Greissing and Jon McKnight

The Federal Affairs division of PPTA North America is active on a variety of legislative and regulatory fronts as the first session of the 110th Congress approaches the summer months. Priorities for PPTA include educating policy makers of the unique characteristics of plasma derived and recombinant analog therapies (collectively, plasma protein therapies) and differentiating them from traditional pharmaceutical and biotechnology medicines; finding a permanent and comprehensive solution to the ongoing IVIG patient access dilemma; and addressing the industry's concerns with transparency and product diversion issues revolving around the government mandated 340B Drug Pricing Program.

IVIG Patient Access Issues Continue

According to data by consumer organizations, the current intravenous immune globulin (IVIG) provider reimbursement shortfall continues to affect patient access to this lifesaving medicine. Changes in Medicare provider payment rates to the Average Sales Price (ASP) plus 6 percent reimbursement methodology in January 2005 in the physician setting resulted in patient migration to the hospital outpatient setting. Because physicians were reimbursed at a rate lower than their purchase price, it has been reported that many were unable to perform IVIG infusions for their patients because it became economically unsustainable. Beginning in January 2006, a similar occurrence with Medicare reimbursement in the hospital outpatient setting has taken place where it has led in some instances to increased Medicare beneficiary IVIG access problems.

The Medicare beneficiary IVIG access issue has still not been addressed by the Department of Health and Human Services (HHS) despite two pending agency-directed IVIG marketplace studies. The first study was initiated because various consumer organizations have reported that Medicare beneficiaries were experiencing significant difficulties accessing IVIG in their preferred site of service, the Committees on Energy and Commerce and Ways and Means of the United States House of Representatives jointly

requested in the summer of 2005 that the HHS Office of Inspector General (OIG) examine the current IVIG marketplace. In addition to assessing manufacturer pricing, this study is examining the role of distributors, Group Purchasing Organizations (GPOs), and physicians in the IVIG distribution channel. At press time of this article, we understand that the OIG study is still pending and has not been released.

HHS commissioned a second study during the summer of 2006 for the purpose of better understanding the IVIG marketplace and elevating access and reimbursement concerns expressed by patients and physicians. The HHS Office of the Assistant Secretary for Planning and Evaluation (ASPE) contracted with Eastern Research Group to examine supply, distribution, demand, and access issues associated with IVIG. On September 28, 2006, ASPE conducted a Town Hall meeting for the purpose of obtaining public comment on IVIG access problems. An overwhelming majority of those patients and physicians commenting at the meeting argued that inadequate Medicare reimbursement for IVIG is the chief reason for IVIG access problems for Medicare beneficiaries. PPTA also made a statement (see www.pptaglobal.org) at the Town Hall meeting.

Almost two years have passed since the OIG began its investigation and the crucial report has yet to be released. To exacerbate the situation, many policymakers are unwilling to provide a legislative or regulatory remedy until these important studies are published. In the meantime, while the IVIG community eagerly awaits the release of the OIG report and the ASPE study, critical patient and physician surveys have been conducted by the Immune Deficiency Foundation (IDF) that overwhelmingly illustrate a continuous and ongoing patient access problem that has not subsided and restricts the ability for patients to acquire their medicines at the most appropriate site of service.

Since the inception of the new ASP plus 6 percent provider payment rate mandated in the 2003 Medicare Modernization Act, PPTA and its member companies as well as stakeholders in the IVIG community have been reaching out to Congress and HHS to advocate for an immediate and

comprehensive solution to restore Medicare beneficiary access. PPTA will continue to work with Congress, HHS and the Centers for Medicare and Medicaid Services (CMS) in finding a comprehensive and permanent solution that will address the dilemma and return patients to the most appropriate site of service for their lifesaving IVIG treatment.

For more information, please contact PPTA North America Federal Affairs staff: Jay Greissing, Esq., Director, Federal Affairs at jgreissing@pptaglobal.org or Jon McKnight, Esq., Manager, Federal Affairs at jmcknight@pptaglobal.org

340B Drug Pricing Program:

On February 9, 2007, House Committee on Oversight and Government Reform Chairman Henry A. Waxman (D-CA) conducted a hearing on allegations of waste, fraud, and abuse in pharmaceutical pricing. In his opening remarks, Chairman Waxman criticized manufacturers for overcharging the entities that are eligible for discounted drugs for its patients under the Public Health Service Drug Pricing Program, also known as the 340B Program.

Section 602 of the Veteran's Health Care Act of 1992 enacted the 340B Program. Throughout the duration of the program, the Health Resources and Services Administration (HRSA) issued guidance in *Federal Register* notices and Dear Manufacturer letters. HRSA, however, has not codified any of these guidelines in the Code of Federal Regulations, which has led to some confusion among covered entities regarding their responsibilities under the program.

The 340B Program requires drug manufacturers to enter into a pharmaceutical pricing agreement with HRSA to provide discounted prices on "covered outpatient drugs" to a list of "covered entities" in order for payment to be available under Medicaid or Medicare Part B for such outpatient drugs. Although participation in the 340B Program is voluntary for entities that fall within the definition of a "covered entity," such as comprehensive hemophilia treatment centers (HTCs) and disproportionate share hospitals (DSHs), participation is mandatory for all PPTA members that provide therapies to both Medicaid and Medicare Part B beneficiaries. In order to qualify as a covered entity,

those HTCs must receive a maternal and child health services block grant under Section 501(a) (2) of the Social Security Act. According to the HRSA Web site, 85 of approximately 145 HTCs receiving these grants are currently participating in the 340B Program.

According to the statute, a covered entity may only obtain covered outpatient drugs at the mandated drug discount price under the 340B Program for those who qualify as patients of such a covered entity. Specifically, the 340B Program prohibits covered outpatient drugs purchased at this price by a covered entity from being resold or otherwise transferred "to a person who is not a patient of the entity." HRSA recently concluded "it is possible that some 340B covered entities may have interpreted the definition too broadly, resulting in the potential for diversion of medications purchased under the 340B Program." This "diversion" of products purchased at this 340B ceiling price occurs when covered entities attempt to artificially expand their patient population. Although Chairman Waxman is currently focused on whether these covered entities are obtaining product at the appropriate discount, his committee must also examine the potential fraud and abuse perpetuated by covered entities through product diversion.

In recent months, HRSA has not only issued two proposals in the *Federal Register* that should strengthen the 340B Program, but also issued one proposal that would expand the program without implementing proper safeguards to protect the program from potential fraud and abuse. Among these proposals, however, HRSA has not yet implemented any of the recent recommendations of the Office of Inspector General (OIG) of the U.S. Department of Health and Human Services (HHS). Such recommendations include improving oversight to ensure covered entities are charged at or below the 340B discounted price by manufacturers, providing technical assistance to program participants, and obtaining consistent data to calculate the 340B ceiling price. HHS is also encouraging HRSA to provide guidance on the "penny price policy," which calls for manufacturers to charge a penny multiplied by the drug's package size when faced with a negative 340B ceiling price. HHS believes published guidelines are necessary to ensure

compliance, which should reduce overpayments for products by covered entities.

PPTA has issued letters of support for both HRSA's efforts at strengthening the definition of a patient under the 340B Program, as well as HRSA's decision to propose a requirement that HTC's under the 340B Program file a report on patient factor replacement program (FRP) participation, FRP program revenue, FRP program costs, FRP program net income, and use of such income. Both proposals should help alleviate problems of product diversion within the program.

PPTA, however, also sent a letter urging HRSA to limit its expansion of the contract pharmacy program under the 340B Program, as well as to refrain from such expansion unless proper safeguards are in place. While PPTA supports the intent of the contract pharmacy program to "facilitate program participation for those eligible covered entities that do not have access to appropriate 'in-house' pharmacy services," the association believes that without appropriate oversight and audit requirements, expansion of the contract pharmacy program may increase the risk of product diversion and duplicate discounts.

Improving the 340B Program will require considerable effort from both Congress and HRSA. At the February 9, 2007 hearing, John E. Dicken, Director of Health Care at GAO, testified to the "inadequacies" of HRSA's "oversight of the 340B drug pricing program, a lack of transparency in the 340B prices, and overpayments to drug manufacturers." The recent findings and recommendations by both GAO and

the HHS OIG will likely exacerbate the scrutiny of the 340B Program by both Congress and the Administration.

Because covered entities such as DSH hospitals and HTC's are important venues for some individuals to obtain life-saving plasma protein therapy treatments, PPTA is determined to improve the program by continuing to propose policies that will help eliminate potential for fraud and abuse. PPTA's efforts are focused on preserving the Congressional intent of enabling these covered entities "to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services." PPTA will work to educate Chairman Waxman and his staff, as well as other interested Members of Congress on the significant issues surrounding the 340B Program that could adversely affect a very vulnerable patient population.

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Meet PPTA Staff



Background

My name is Sophie Van Puyvelde. I am 33 years old. I come from Belgium and I am based in the PPTA Europe offices in Brussels. I have two little girls, Leandra (5 years old) and Elena (4 years old). This year will be my 8th year working at PPTA.

Tell us about your background?

I studied in Lisbon, Portugal where I learned to speak Portuguese. Afterwards I found a job in tourism in Lisbon and the Algarve. Being fed up with too much sun, I then chose to move to London where I found an administrative job at America Online. I moved back to Belgium for family reasons and found a job at PPTA as National Affairs Assistant.

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

My career at PPTA has evolved and I am now Manager, Office and Events. As Office Manager I am responsible for the smooth running of the office. I also work with our accountant and I assist in HR matters and organizing travel for PPTA staff.

As Events Manager, my main task is the organization and co-ordination of our annual International Plasma Protein Congress. This year's event was successfully held in Vienna

and I look forward to next year's Congress. This is one of the most challenging aspects of my job and can be a stressful period but I really enjoy it. Additionally, I am also responsible for organizing internal PPTA events such as the annual PPTA Europe Planning Meeting.

What is most rewarding about working in this industry?

I really enjoy working at PPTA because we are a small and very close team with a strong family atmosphere. At the end of the day I also feel rewarded as I know we work to help patients establishing a communication with our industry. I enjoy my contacts with our member companies and feel they appreciate the work we deliver.



PPTA welcomes Kara Flynn as the Association's new Director for Global Communications. In this role, she is responsible for building and promoting the image and awareness of the plasma protein therapeutics industry globally and for developing and maintaining a positive image with multiple stakeholders including consumers, health care leaders, innovators and the public.

The World Health Organization (WHO) will hold a special expert meeting on Essential Medicines for Children in Geneva Switzerland on 9-13 July 2007. One of the objectives of the meeting will be to establish a WHO List of Essential Medicines for Children along the lines of the WHO List of Essential Medicines.

The European Commission, the Japanese Ministry of Health Labor and Welfare and the Pharmaceuticals and the Pharmaceuticals and Medical Devices Agency agreed on confidentiality arrangements to exchange confidential information.

The Medicare physician payment formula was a leading topic at both the Senate Committee on the Budget and the House Committee on Ways and Means Subcommittee on Health hearings on the President's 2008 Budget for the Department of Health and Human Services.

In Japan, the Blood Council has approved the Donation Promotion Plan, Donation Plan and Demand and Supply Plan.

The US Food and Drug Administration (FDA) has posted information regarding the potential risk of Variant Creutzfeldt-Jakob Disease (vCJD) from Plasma-Derived Products. They state that the risk of vCJD to patients who receive US licensed pdFVIII products is most likely to be

extremely small and that risk from other plasma derived products, including Factor IX, is likely to be as small or smaller.

The EDQM and the Health Products and Food Branch (HPFB) of Health Canada have agreed to sign a memorandum of understanding that would see the official incorporation of Certificates of Suitability granted by the EDQM into the evaluation of drug substances by the Therapeutic Products Directorate (TPD) of the HPFB.

Immunoglobulins have been reinstated into the World Health Organization's (WHO) Essential Medicines List. The WHO recently published the 15th edition of its Essential Medicines List on its website. Immunoglobulins had been removed from the list in 2003 by the WHO and previous individual requests for reinstatement from concerned stakeholders had failed in 2005. Since then, PPTA had embarked on a joint campaign with all concerned stakeholders and a joint application requesting the reinstatement of immunoglobulins on the WHO List had been submitted in October 2006 by the International Patient Organization for Primary Immunodeficiencies (IPOPI) and the International Union for Immunological Societies (IUIS). The joint application received worldwide support from more than 200 stakeholder organizations and individuals when it was posted for public review on the WHO website late last year.



Calendar of Events

May 3-6

WFH 10th Musculoskeletal Congress
Stresa, Italy

May 8-12

5th Plasma Product Biotechnology Meeting
Hotel Hermitage-Biodola Elba, Italy

May 15-16

IPFA International Workshop on the Safety and
Supply of Blood and Plasma Products
Kyoto, Japan

May 18-20

XX Annual Conference of the European
Hemophilia Consortium
Parma, Italy

June 5-6

The Plasma Protein Forum (PPTA)
Hyatt Regency Reston, USA

June 14-15

14th IPFA/PEI Workshop on Surveillance and
Screening of Blood Borne Pathogens
Warsaw, Poland

June 23-27

XVII Regional Congress of the ISBT, Europe
Madrid, Spain

July 6-12

XXI ISTH Congress
2007 – Geneva, Switzerland

September 14-16

40th Biannual Congress of ESPHI (European
Society of Pediatric Hematology and
Immunology) Athens, Greece

September 24-25

5th WFH Global Forum on the Safety and Supply
of Treatments for Bleeding Disorders
Montreal, Canada

September 28-30

The 3rd World Congress of Alpha1 Patients
Rome, Italy

November 10-13

XVIII Regional Congress of the ISBT, Asia
Hanoi, Vietnam



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