

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SUMMER 2016

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Access and Advocacy in the United States

The Global Spread of the Zika Virus,
and How the Past has been a Good Teacher



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SUMMER 2016 Contents

3 IN MY VIEW

4 PPTA: Focused on People with Rare Diseases

10 Access & Advocacy in the United States

12 The Global Spread of Zika Virus, and How the Past has Been a Good Teacher

16 Asia Pacific Society for Immune Deficiencies Inaugural Meeting

20 IPPC 2016: Ethical Aspects of Compensating Plasma Donors

22 IPPC 2016: Patient Voice Inclusion in the Organization of Care

26 IPPC 2016: Blood and Plasma - Visions for a Sustainable System

28 Collecting Hyperimmune Anti-D Plasma in Germany – A Real Challenge

30 EUHASS: Continuation of Patient Registry Series

32 Plasma-based Drug Makes Big Impact for Young Patients

34 INSIDE PPTA

34 The European Plasma Alliance

35 Professor Flora Peyvandi Awarded the 2016 Hilfenhaus Award

36 Meet the Global Operations Team

39 UPCOMING EVENTS

40 GLOSSARY



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In My View

BY JAN M. BULT, PPTA PRESIDENT & CEO

In my view, it is time to rethink some of the terminology that is used whenever it comes to the description of the differences between private and public sector. In the end, the one important thing that matters is how we all—private and public sector—can and must increase the availability of plasma protein therapies to serve the many patients in the world whose lives depend on these therapies.

About 15 years ago, when several private sector manufacturers were having difficult times and some companies were in the red, they became “not-for-profit,” but not on purpose. Since then we have seen the beginning of many improvements e.g., manufacturing, yield, capacity, and efficiency. In the last decade we have noticed that many blood banks are having difficult times and are losing money. Though they are not-for-profit, losing money is definitely not the preferred option. So, if profit is important to all, what is the issue when we talk about for-profit or not-for-profit?

What is the not-for-profit business model? According to the website of the International Plasma Fractionation Association (IPFA) there are two important factors:

- No financial gain will flow to external stakeholders.
- Any financial surplus will be reinvested in research and facilities.

This is really interesting! Our (private sector) member companies invest more than ever in research and facilities and are providing the super majority of the plasma protein therapies in the world. And, in addition, they are able to give a return to their investors — a win-win situation for all!

At the same time, we see another interesting development. Because of the financial losses within the blood bank sector, there is a desire to convert blood donors to plasma donors. This plasma will generate revenue for the blood banks because the plasma will be sold to manufacturers. That will improve the profitability of the not-for-profit blood banks. It sounds ironic, doesn't it?

We notice that some not-for-profit companies now have their own commercial plasma collection centers in both the U.S. and Europe. There is nothing wrong with that,



it just makes sense to realize the potential of source plasma collection and that recovered plasma alone will not bring the volumes that are needed.

So, in conclusion, both sectors—public and private—need to make a profit for their sustainability, both sectors use source and recovered plasma, both sectors agree that growth has to come from source plasma collection and most importantly, both sectors agree that the main focus must be to improve access to care to patients!

Let us stop playing with words, let us work more towards the sustainable supply of life-saving therapies for all patients in the world. ●

Jan M. Bult, PPTA President & CEO

PPTA: FOCUSED

BY WILLIAM MURRAY

ON PEOPLE WITH RARE DISEASES



No other group of therapies for rare diseases comes from the human donor; it is this connection, through plasma, that creates the signature of the industry's commitment to the ultra-rare condition.

For over two decades, PPTA has been **the leading global trade association representing manufacturers of plasma-derived and recombinant therapies** (collectively, plasma protein therapies). PPTA staff have the expertise and substantial policy experience to understand how to position rare diseases in the global environment. Plasma protein therapies, such as: blood clotting factors that treat individuals with hemophilia and other bleeding disorders; alpha-1 proteinase inhibitor that treat alpha-1 antitrypsin deficiency, C1-esterase inhibitor life-saving for individuals with hereditary angioedema (HAE) and immunoglobulin necessary to sustain the quality of life for individuals with primary immunodeficiency (PID) disorders are ultra-rare diseases.

PLASMA

The human factor: Donors, treaters, regulators, industry workers, and people with rare diseases are who make the industry unique and are the driver for the industry's commitment to treating rare diseases. Plasma is more than a raw material for plasma protein therapeutics; it is the foundation of this commitment to the treatment of rare disease. With more than forty years of experience in advocating for plasma collection, the plasma donor, and people with rare diseases, PPTA takes a multidisciplinary and global approach to working toward the strategic goal of ensuring the availability of safe, high quality plasma for fractionation.

This strategy takes shape through a number of different contexts and tactics. As an example, through an active and vigorous voluntary standards program unlike any other, the industry constantly seeks global solutions for the betterment of itself and to improve the plasma donation experience. As another example, through the Association's industry profile efforts in North America and Europe, we address stakeholders, the regulatory community, and the public at large via multiple channels, including the International Plasma Awareness Week, a point of significant partnership with the rare disease community. Working in a variety of disciplines, including legislative and regulatory advocacy, we ensure a consistent message and effort across a complex landscape of issues.

PPTA's strategic goals drive our efforts to build awareness of the global importance of plasma as the foundation of plasma protein therapies. These efforts include regulatory advocacy, free trade advocacy, access to care advocacy, and communications around the world.

Recently, PPTA defended the industry's interests by filing an amicus brief in ongoing litigation regarding the status of U.S. plasma collection centers under the Americans with

Disabilities Act. PPTA's brief asserted that a plasma collection center is not a "place of public accommodation," as this designation is inconsistent with the rigorous donor screening process. Although anyone is welcome to offer to donate plasma, some donors are necessarily turned away due to health reasons, including screening criteria set forth in United States Food and Drug Administration (FDA) regulations and PPTA standards. The brief presented an important opportunity to educate both the judiciary and the disability community on the often misunderstood process of plasma donation, including its central role in the lives of individuals with rare, life-threatening plasma protein deficiencies.

REGULATORY

PPTA engages with regulators about access to safe therapies for people with rare diseases in a number of settings including annual FDA-industry liaison meetings, European Medicines Agency (EMA) staff meetings, and industry conferences and workshops. PPTA utilizes these interactions to update regulators on industry initiatives, including providing assurance that plasma protein therapies are safe in the face of emerging or re-emerging pathogen threats. In recent years, industry has provided data on continued safety from, among others, vCJD, WNV, SARS, HEV, and Chikungunya. PPTA further engages regulators via FDA-industry co-sponsored public workshops and participation in EMA workshops. Recent workshops have addressed thromboembolic and hemolytic events in IG patients, immunogenicity of therapeutic coagulation proteins, and the value of patient registries in the regulatory processes. PPTA also liaises between patient advocacy groups and regulators on access issues, including through FDA's Patient-Focused Drug Development initiative.

PPTA partners with the FDA and the National Institutes of Health (NIH) to hold public workshops that address issues of importance to the plasma protein therapy industry. For example, at the FDA-PPTA-NIH public workshop, “Risk Mitigation Strategies to Address Potential Procoagulant Activity in Immune Globulin Products” (Rockville, Md., May 17-18, 2011), PPTA presented an analysis of spontaneously reported thromboembolic events for patients under intravenous immunoglobulin treatment (2005-2010). Workshop outcomes included increased awareness of complexity of events, including the role of procoagulants and host factors; understanding of fractionation processes with respect to procoagulant presence and removal; and status



of assay development, feasibility for validation, and use in manufacturing environments. At the FDA-PPTA-NIH public workshop, “Strategies to Address Hemolytic Complication of Immune Globulin Infusions” (NIH, Bethesda, Md., Jan. 28-29, 2014), PPTA presented a patient qualitative case analysis of industry adverse events – the largest such dataset to date. PPTA took the lead to publish manuscripts developed from the proceedings as a supplement to the journal *Transfusion* in July 2015. Most recently, the FDA-PPTA-NIH-NHF (National Hemophilia Foundation) public workshop, “New Methods to Predict the Immunogenicity of Therapeutic Coagulation Proteins” (NIH, Bethesda, Md., Sept. 17- 18, 2015), focused on genetic determinants of immunogenicity, uses of genetic information, preclinical assessments of immunogenicity, studies of previously untreated patients, and immune tolerance induction. At PPTA’s invitation, FDA attended a meeting of the Association’s industry-wide immunogenicity task force (April 5, 2016) to discuss workshop outcomes and next steps.

In 2012 and 2014, comments to FDA, PPTA supported the inclusion of rare diseases, for which member companies

provide life-saving and life-supporting therapies, in the Agency’s listings of potential candidates for the Patient-Focused Drug Development initiative. After receiving comments, FDA chose 24 disease areas, including hemophilia A, hemophilia B, von Willebrand Disease, other heritable bleeding disorders such as factor I, V, VII, X, and XI deficiencies, and platelet disorders; PPTA facilitated dialogue between the Agency and patient advocacy groups leading up to the FDA public meeting on these disorders (FDA campus, White Oak, Md., Sept. 22, 2014). Alpha-1 antitrypsin deficiency (meeting held Sept. 29, 2015) and hereditary angioedema (meeting to be held by Sept. 30, 2017) have also been chosen by FDA as candidates for the Patient-Focused Drug Development initiative.

PPTA also participates in EMA workshops including a recent workshop on hemophilia registries (EMA, London, U.K., July 1-2, 2015), which focused on how data collected in these registries could be utilized in a more coordinated and efficient fashion to inform patients and clinicians, regulators, and the general public on possible ways to maximize the benefit to public health. Patient registries have the most perceived value in rare diseases.

PPTA continued to educate stakeholders and increase support for its European Data Program. A major achievement was securing an opportunity to deliver a Data Program presentation to EMA’s Blood Products Working Party. The presentation was a targeted and highly effective means of introducing the key regulators of plasma protein therapies in all 28 E.U. Member States to this important drug shortage preparedness mechanism on which rare disease communities, particularly PID and hemophilia patients, rely.

FREE TRADE

Knowledge about the needs of people with rare diseases has grown exponentially over the past several years, uniting the efforts of PPTA, our member companies, and the global patient community. This has placed a renewed emphasis on the strategic goal of free trade, working toward the elimination of trade barriers and discriminatory practices to achieve open access to all therapies globally.

PPTA is committed to improving access for patients in jurisdictions outside of the United States and European Union. For example, our efforts in China include workshops and outreach designed to demonstrate the safety and quality of source plasma and plasma protein therapies to treat patients. Indeed, one of the greatest challenges for PPTA’s member companies has been recognition of rare diseases as an object of concern and priority for health care systems and policymakers.

PPTA’s uniquely situated operations and expertise –in Europe, in North America, and in Asia—allows the industry



Knowledge about the needs of people afflicted with rare conditions **has grown exponentially** over the past several years, **uniting the efforts of PPTA, our member companies, and the global patient community.**

to pursue a consistent and harmonized advocacy effort. Our work includes not only specific areas of potential regulatory relief in different countries, but in advocating for better solutions for people with rare diseases, rather than trade restrictions. We've been an active participant in the ongoing high-level negotiations between the European Union (EU) and U.S. in the Transatlantic Trade and Investment Partnership (TTIP), informing policymakers of the importance of plasma therapies and explaining the ways in which improved trade relations could benefit our industry's operations and, therefore, improve access and availability to patients.

PPTA is using the ongoing negotiations concerning the TTIP trade deal as an opportunity to bring the regulatory convergence priorities of both the plasma protein therapies and source plasma collection industries to policymakers on both sides of the Atlantic. PPTA continues to engage in advocacy outreach to

the key regulators responsible for both trade (USTR and DG Trade) and pharmaceuticals (FDA and EMA) on three priorities: mutual recognition of inspections, U.S. acceptance of European plasma, and "global sufficiency" for source plasma. These efforts will ensure that the needs of niche, rare disease therapies will not be overlooked as the TTIP negotiators also address the broader "Big Pharma" agenda.

ACCESS TO CARE

Rare diseases are a key health priority in the EU as described in our last Source magazine.¹ However, as we see the countries adopting national rare disease plans, there is still a long way to go for improving the care of the patients suffering from these diseases. Plasma protein disorders are rare diseases and if we take a closer look into the access to care situation of these diseases, we can certainly highlight the following difficulties currently encountered by the rare plasma disorders patient community:



- The continuous pressure and new measures on pharmaceuticals expenditures not considering the specificities of rare plasma disorders (with recent examples in Greece, Cyprus, Romania, U.K., etc.).
- The need for education of the medical community about rare plasma disorders during the course of their training.
- The difficulty in establishing a harmonized database and data resources to foster research.
- The differences in treatment levels and reimbursed indications by country.
- The never-ending discussions to establish hemophilia treatment centers (e.g., in Belgium).
- Delayed diagnosis.
- An increased clinical demand requiring that more plasma is collected as starting material.

Interestingly, one important aspect of plasma protein therapies is that they have proven to be very effective as shown during the numerous years of clinical use and experience. However, a classical difficulty in the field of rare diseases is to ensure that the specific coverage needed to treat a specific disease is supported, and this is where resources start to compete.

So on one side, although very good rare disease policy developments are occurring, it should not be denied that counterproductive moves are happening at the same time, worsening access to care for plasma protein therapies. Here again we take the example of countries implementing new cost containment measures that do not take into consideration the specific nature of plasma protein therapies and their cost-effective treatment. This is like improving the thermal insulation of the walls of a house while removing the windows at the same time.

However, the efforts of the EU in the rare disease area should be noted, particularly the initiatives to improve collaboration among experts.

EU INITIATIVES

There is an effort to encourage specialized health care providers to establish European Reference Networks (ERNs). The goal of the ERNs is to allow the top specialists from across Europe to coordinate their efforts to tackle complex or rare medical conditions that require highly specialized health care

and a concentration of knowledge and resources. The call for grant applications to become an ERN with a grant was begun on March 15 and is running until June 21, 2016, the non-grant application will run from June 23 up to July 22, 2016.

Every year, the EU chooses a specific subject to encourage debate and dialogue within and between European countries. The aim of the European Year is to raise awareness of a certain topic. A European Year can also send a strong political signal to gain a commitment from EU institutions and Member State governments that the subject will be taken into consideration in future policymaking. The European Organisation for Rare Diseases (EURORDIS) has launched a campaign to get the year 2019 assigned to Rare Diseases. With all the new orphan drugs in development and the need for more coordination in the different aspects of rare diseases, this is certainly a theme that would be useful in attracting the attention and action of decision makers.

There are several initiatives to improve the care for rare diseases but it's important to maintain what has been achieved so far. The role of PPTA is to support patients and patient advocacy groups in having their voices heard. By representing the industry, PPTA is in the position to bring the messages to decision-makers who need the industry's consolidated view and expertise.

COMMUNICATIONS & ADVOCACY

PPTA has a robust global communications and advocacy operation dedicated to raising awareness of rare diseases and ensuring that people with rare diseases have access to the life-saving therapies they need. PPTA maintains six websites in three languages to educate people and help them find ways to donate much needed plasma. We conduct outreach to patient groups and stakeholders around the world and work with them to ensure patients have access to the therapies they need to sustain and improve their lives. Through its numerous conferences, PPTA engages with industry, regulators, legislators, patients, and others to improve access to care. PPTA's quarterly magazine, *The Source*, keeps its members and other stakeholders apprised of the latest developments in rare diseases and plasma protein therapies.

Access to high quality health care is of fundamental importance to all people around the world. On a global scale, policymakers are faced with tough decisions regarding access to plasma protein therapies. Recently, downward budget pressures have caused public and private payers to more closely scrutinize their expenditures, coverage, and utilization of specialty drugs treating rare diseases. As proposals are debated in Congress, Parliament, and Health Ministries, maintaining access to care for people with rare diseases should be in the forefront. Before any solution is identified, remember that a one-size-fits-all policy does not work. It is critical to focus on the importance of patient access to

No other group of therapies for rare diseases comes from the human donor; it is this connection, through plasma, that creates the signature of the industry's commitment to the ultra-rare condition.

plasma protein therapies and to highlight why these essential therapies are vastly different from synthetic pharmaceuticals. The economics of manufacturing plasma protein therapies and that of traditional pharmaceutical medicines could not be more different. Where new developments for chemical pharmaceuticals come from new molecules or molecule improvements, developing new plasma protein therapies from proteins found in human plasma is significantly different. Innovation in the plasma protein therapeutics space is significantly different and more intense because of research, clinical trial requirements, upfront investment, and regulatory compliance. These differences must be taken into consideration when policy makers are deciding issues that will impact access to plasma protein therapies. For patients with rare diseases, plasma protein therapies are high value and high impact; access is a necessity to sustain quality of life.

There are hundreds of plasma proteins circulating throughout the body. However, only a small number of those proteins are manufactured into medicines that save and improve the lives of patients with chronic, rare genetic disorders. Some of those proteins can be used to manufacture blood clotting factor, immunoglobulins, antithrombin III, fibrinogen, alpha-1 antitrypsin, C1-esterase inhibitor and albumin. Only plasma proteins that are present in the human body can be identified, analyzed, manufactured, purified and, finally, administered by a physician to a patient for clinical purposes. These plasma proteins can be manufactured through either a plasma fractionation process or the use of recombinant technology (biotechnology).

Many individuals suffer from the inability to produce these proteins in sufficient quantities and therefore require replacement or augmentation therapies depending on the degree of the deficiency. The diseases treated with plasma protein therapies can be life-threatening and are chronic, rare conditions. PPTA is the Association poised to address the access to care challenges facing patients with these rare diseases who depend upon access to plasma protein therapies to sustain and improve their lives. •

WILLIAM MURRAY, PPTA Director, Global Communications

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ACCESS & ADVOCACY IN THE UNITED STATES

BY BILL SPEIR

In the last twelve months, PPTA government affairs staff has successfully advocated for changes to Alabama and Nebraska laws to allow 18-year-olds to donate plasma. The age of majority is 19-years-old in both states, therefore legislation creating an exception to donate plasma at 18 was needed.

Legislatures in both states found our advocacy message compelling enough to create the exception. Our advocacy message was simple: Plasma is often referred to as the “gift of life” because it is the essential starting material for plasma protein therapies that help patients worldwide with rare, chronic conditions to live healthier, productive, and fulfilling lives. Our advocacy partners, including member companies and the American Plasma Users Coalition (A-PLUS), assisted us in our successful efforts.

These successes are only a few of our advocacy efforts. We also strive to ensure patients have access to their medically appropriate therapy from quality specialty pharmacies at the site of service that is preferred by the physician and the patient. In the United States, the constant balancing act between best price and best cost creates a tension in the health care system that often negatively impacts patient access.

Our advocacy message in this arena focuses on the following principles approved by the PPTA North America Board of Directors:

1. Plasma protein patients require access to the highest quality care.
2. Plasma protein patients require timely and medically appropriate access to care and to all brands of plasma protein therapies.

3. Reimbursement for plasma protein therapies must be adequate to sustain access.
4. Plasma protein patients require access to transparent networks, as well as timely and medically appropriate access to the right provider at the right site of care.

These principles are based on the guidelines and recommendations of physicians that treat plasma deficient patients. The National Hemophilia Foundation’s Medical and Scientific Advisory Council (MASAC) and the American Academy of Allergy, Asthma & Immunology (AAAAI) have published advice for the best treatment of individuals with hemophilia and primary immune deficiency. PPTA frequently uses these publications in our advocacy efforts.

For example, MASAC Recommendation 159 states that: “Clotting factor therapies are neither pharmacologically nor therapeutically equivalent” and that: “The benefit of limiting products to one within a class, such as one recombinant factor VIII concentrate, solely for the purpose of cost containment is not supported by present clinical practice or by published data.” We recently shared this information with the state of Georgia, which is considering a policy that is completely contrary to the MASAC recommendation that there is no clinical “benefit of limiting products to one within a class.” Georgia Medicaid’s proposal would create a preferred drug list that would have one preferred product for factor VIII recombinant, one for factor IX recombinant, and one for von Willebrand factors.

State Medicaid programs develop preferred drug lists to drive patients to the therapy or therapies in a therapeutic class that provides them with lowest cost in that therapeutic class. In theory, some recipients will be asked to switch from a



therapy that works for them to a therapy that may not be as effective. Some states may even require some Medicaid recipients to fail first on a preferred product before the recipient may access a non-preferred product. This may seem wise on paper since it appears to save the state money while providing Medicaid recipients with access to a therapy that is in the same therapeutic class. However, this wisdom, which may work for some therapeutic classes, is not wise for the classes of plasma protein therapies.

PPTA is working with our stakeholders to ensure that Georgia Medicaid recipients who are stable on a therapy are able to remain on their current therapy. This is known as grandfathering. The proposed Georgia policy states that they will allow grandfathering. We are also advocating for clear clinical guidelines that prescribers may use to gain an exemption from the preferred drug list if they think a non-preferred therapy is the most medically appropriate.

The third principle, “Reimbursement for plasma protein therapies must be adequate to sustain access,” is the reason for our advocacy efforts around state implementation of a recent federal rule change. In January 2016, the federal Medicaid program finalized a rule (CMS-2345-FC) that changes the way Medicaid reimburses pharmacies including the specialty pharmacies that provide plasma protein therapies to Medicaid recipients.

This final rule will require all state Medicaid programs to adopt, by April 1, 2017, a reimbursement methodology for pharmacy providers that will pay pharmacies, including specialty pharmacies, based on their actual acquisition cost plus a professional dispensing fee.

States have begun the process of amending their state plan amendments, statutes, and/or regulations to meet the new rule requirements. PPTA is taking this opportunity to advocate for reimbursement methodologies that are sufficient to ensure patients have access to their medically appropriate plasma protein therapy. This involves collaboration with patient organizations and specialty pharmacies.

The federal rule has been a long time coming and PPTA began addressing this change before the rule was proposed. PPTA created the State Patient Access Coalition (SPAC) in 2009, a coalition of clotting factor manufacturers and specialty pharmacies, to address the need to protect patient access since Medicaid leaders were even then discussing a desire to change pharmacy reimbursement. The SPAC advocacy efforts focus on sharing with decision-makers all the activities that specialty pharmacies must perform to properly provide blood clotting factor to individuals with bleeding disorders according to the standards in MASAC recommendation 188.¹

Access to plasma protein therapies requires coordinated and constant advocacy efforts. PPTA remains diligent in promoting advocacy based on sound principles that lead to the best outcomes for individuals that rely on plasma protein therapies. ●

BILL SPEIR, *PPTA Senior Director, State Affairs*

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¹ National Hemophilia Foundation. MASAC Recommendations Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders. (MASAC Document #188).



The Global Spread of Zika Virus, and How the Past has Been a Good Teacher

BY THOMAS R. KREIL

The recently accelerating spread of Zika virus (ZIKV) around the globe¹ will be recorded as another reminder of the literally countless infectious agents that exist.

Beyond being the day-to-day challenge for members of a narrowly specialized scientific community, these infectious agents go largely unnoticed from a public health perspective. This is true particularly if they cause no or generally mild and self-limiting diseases quite unlike e.g. the Ebola virus. Only when an increasing number of infection cases occur in an area of advanced socio-economic status, supported by a more vigilant health care system, and subject to higher attention of the press, rare yet clinically more impactful manifestations of these infections may also become apparent. For ZIKV, larger numbers of infections in more recently affected areas such as French Polynesia, and now South and Central America and the Caribbean have been paralleled by an increased incidence of Guillain-Barré syndrome (GBS) cases, as well as fetal malformations described as microcephaly.²

These events have been a sufficient reason for ZIKV to dominate both the lay and scientific press, much like other arthropod-borne viruses such as Dengue, West Nile, and Chikungunya did when they reached the United States at epidemic scale during the last two decades. Similar to those earlier arrivals, the first cases of ZIKV transmission through blood transfusion recently reported have triggered widely-debated questions about the safety of the blood supply, followed by purely mechanistically related concerns about the safety of plasma-derived medicinal products.

This time though, the responses of all concerned stakeholders were different. The United States Food and Drug Administration (FDA) was fast to issue Guidance for Industry,³ recommending to establishments collecting blood and blood components that, if provision of blood components for transfusion collected in areas without active ZIKV circulation per information from the Centers for Disease Control and Prevention (CDC) was not possible, the traditionally successful measures such as deferral of at-risk donors and testing with an FDA-licensed test, when available, be implemented. As an alternative though, the use of pathogen inactivation was encouraged for blood components where an FDA-licensed option existed, an innovation that had not been available during similar situations in the past. Consistent with the appreciation of pathogen reduction, and as ZIKV *is likely cleared by the existing viral inactivation and removal methods that are currently used to clear viruses in the*

manufacturing processes for plasma-derived products, the guidance document recommendations do *not apply to the collection of Source Plasma*.⁴ This statement is entirely compatible with the conclusion of an assessment about plasma product safety versus ZIKV as performed by the PPTA Pathogen Safety Steering Committee, i.e. Zika virus is not a concern for the safety of plasma protein therapies.⁵

When the first cases of West Nile virus infections were detected in the U.S. in 1999, the development of similar FDA guidance was a more complex process, arguably with a similar outcome in the end: a testing requirement for transfused blood components without pathogen reduction capacity and the contrary for plasma for fractionation after the inactivation capacity of manufacturing processes for the respective virus had been verified.⁶

As a Danish proverb states, prediction is difficult, especially when dealing with the future – which is most certainly true for infectious agents. Whether ZIKV will

Estimated range of *Aedes aegypti* and *Aedes albopictus* in the United States, 2016*



***Aedes aegypti* mosquitoes are more likely to spread viruses like Zika, dengue, chikungunya than other types of mosquitoes such as *Aedes albopictus* mosquitoes.**

- These maps show CDC's best estimate of the potential range of *Aedes aegypti* and *Aedes albopictus* in the United States.
- These maps include areas where mosquitoes are or have been previously found.
- Shaded areas on the map do not necessarily mean that there are infected mosquitoes in that area.

*Maps have been updated from a variety of sources. These maps represent CDC's best estimate of the potential range of *Aedes aegypti* and *Aedes albopictus* in the United States. Maps are not meant to represent risk for spread of disease.

Figure 1, source: CDC Zika Vector Surveillance and Control, Accessed April 1, 2016 at www.cdc.gov/zika/vector/index.html

establish a continuous presence in the U.S., similar to West Nile virus after its introduction into an, until then, naive continent in 1999, is unclear, although, according to the CDC, mosquitos of the type that can spread ZIKV are found in many of the States (Figure 1). Still, it may be worth keeping in mind that some predictions about the potential spread of the Chikungunya virus into the U.S. have not yet materialized. Beyond ill-understood environmental factors, the answer to the question may also depend on whether currently ongoing vaccine development efforts will be successful. The potentially causative correlation of exposure to ZIKV and the development of GBS may turn out to be a challenge for vaccine development though, which would result in injecting ZIKV material, either the whole-killed or live-attenuated virus, into many healthy individuals. On the other hand, because plasma-derived intravenous immune globulins are clinically indicated for the treatment of GBS it may turn out to be demanding to provide enough of the potential treatment for an increased incidence of the disease.

It is gratifying to see that all the work invested into addressing the advent of infectious disease agents of potential impact to the safety of the blood supply in the past has offered a solid foundation for a swift and solution-oriented response to ZIKV this time, with all members of the community – patients firstly, but also regulators and industry – working

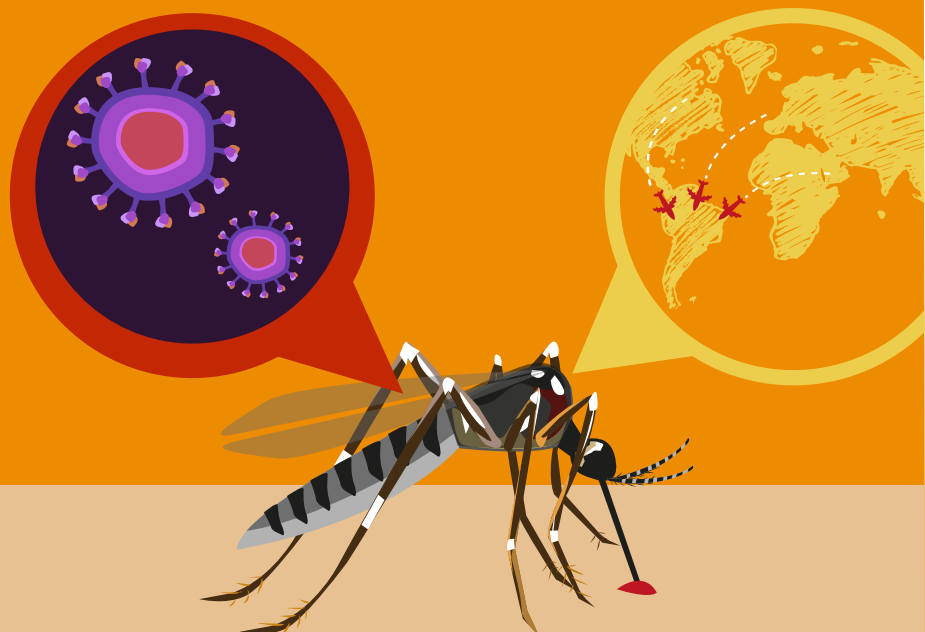
through facts rather than losing time speculating. And while there is no doubt that little separation between animal virus reservoirs and humans particularly in less developed geographies combined with global trade and travel will result in the future emergence of viruses, the current experience with ZIKV has – again – reinforced the robust virus safety margins of plasma derivatives, as well as a great collaborative spirit amongst the community concerned. ●

THOMAS R. KREIL, Ph.D., Associate Professor of Virology, Chairman, PPTA Pathogen Safety Steering Committee Senior Director Global Pathogen Safety, Baxalta

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It is gratifying to see that all the work invested into addressing the advent of infectious disease agents of potential impact to the safety of the blood supply in the past has offered a solid foundation for a swift and solution-oriented response...





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Asia Pacific Society for Immune Deficiencies Inaugural Meeting

BY JULIA FABENS & JOSHUA PENROD

In a positive step for primary immunodeficiency (PID) patients in Asia, the Asia Pacific Society for Immune Deficiencies (APSID) held its inaugural congress April 29 - May 1 in Hong Kong.

APSID was formed with the following goals: to care for and cure patients with PID; to share PID experiences to promote collaboration and education; to improve PID management through understanding its genetics and pathogenesis; and to advocate and advance the care of PID patients through engaging governments, patient organizations and industry.¹ PPTA's President & CEO, Jan M. Bult, and Vice President, Source and International Affairs, Joshua Penrod, were in attendance.

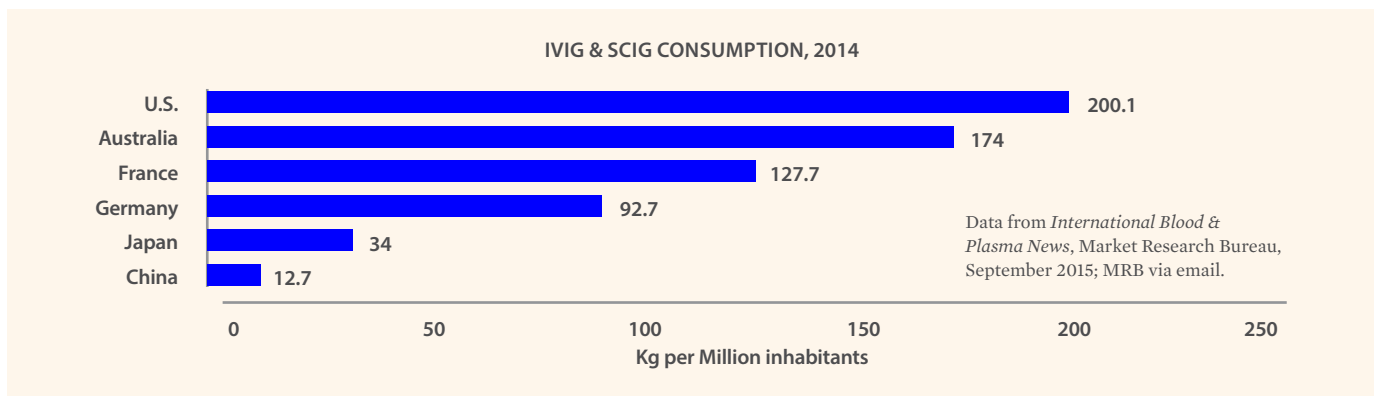
Speakers at the conference included representatives from major international patient groups and leading Chinese physicians and medical experts, as well as leaders in the field from around the world. The overseas organizing committee included representatives of 14 countries, most of which are in the Asia Pacific region.

One of the groups represented at the congress is the recently-formed Primary Immunodeficiency League, based in Hong Kong. Unlike many Western countries, China does not have a strong advocacy culture and patients with primary immune deficiencies historically have not been well

represented in China. While Hong Kong does hold special status in China, the momentum this new group creates will hopefully be able to start increasing the recognition of patients with PID and the challenges they face in China broadly.

PID identification and treatment levels in China fall well below those in the West. Experts suggest that there may be 50,000-100,000 patients with PID in China, and very few of those have been diagnosed.² The low diagnosis rates in China are due to several factors including a lack of data, low clinician awareness of PID, and limited screening (screening for PIDs is not included in the current newborn screening panel).³

Low treatment levels are partly attributable to the fairly limited availability of immunoglobulins (IG), which treat PIDs. There are a number of Chinese manufacturers of plasma protein therapies but they are not currently producing the amount of IG required to treat the expanding patient population. IG manufactured outside of China is barred from entering the country by a provision known as Article 49, so the shortfall cannot be made up through imports. Consumption rates of IG is far lower in China than in other countries,



according to data from the Marketing Research Bureau; China has less than one-tenth of the usage that one finds in the United States.

In the last few years, however, some progress has been made to recognize and treat these patients. There are now several PID centers in China, notably the Children's Hospital at Chongqing Medical University and several run in cooperation with the Jeffrey Model Foundation. A fledgling PID registry has been developed in partnership with the Children's Hospital at Chongqing Medical University but there is no comprehensive nationwide registry. In conjunction with the conference, APSID hosted a Spring School designed for young clinicians training in immunology and infectious diseases and held its inaugural conference featuring thought leaders in the treatment of primary immune deficiencies from around the world.

The two-day main program covered many areas of both specific and general relevance to PID treatment, ranging from gene therapy to stem cell replacement to construction of registries and clinical findings relating to the administration of immune globulin.

The opening session of the program laid out the framework for the APSID organization and its history. Also included were presentations that demonstrated the value of other, similar programs in other areas of the globe. Dr. Andrew Cant of the European Society for Immunodeficiencies described its history and development and Ms. Jose Drabwell, President, International Patient Organisation for Primary Immunodeficiencies, discussed the important role of patient advocacy in the efforts to increase the awareness of rare conditions such as PID. This theme was then picked up by Geoffrey Yu, Vice President of the PID League, based in Hong Kong and celebrating its one-year anniversary. The PID League is the first of its kind in Asia – a patient advocacy group giving PID patients a voice in advocacy, diagnosis, and treatment.

Following this introductory panel were several panels exploring the scientific and clinical elements of the spectrum of immune disorders. DNA typing and other genetic links among conditions were explored, with clinical outcomes that showed the importance of early diagnosis and treatment. This dovetailed as well with the importance of clinical and medical education pertaining to the conditions and several speakers

emphasized the importance of the free exchange of scientific information in order to secure best practices for improved patient outcomes.

Another panel focused on the development of registries in different areas of the world, including Europe, North America, Southeast Asia, and Japan. Critical to the success of the registry efforts include appropriate resourcing and awareness of the conditions. While the registries had several areas in common, there were several clear differences, the most notable being the comparative length of time and intensity at which a registry has been working. Newer registries, such as those in the developing world, are not yet adequately resourced in terms of financial backing or human resources. There is cause for optimism as time goes on, however, as the registries themselves contemplate and encourage greater awareness of PID and related conditions.

Clinical care of PID patients also occupied a large part of the discussion time during the meeting. These presentations included comparisons of treatment in both the developed and developing world, along with considerations of experimental technologies and techniques, such as stem cell transplants and gene therapy. Of great interest was a presentation by Dr. Xiaodong Zhao, who carefully described the usage of IG in a single, large hospital system in mainland China. Out of the many categories of conditions for IG treatment, those patients presenting with PID occupied less than five percent of IG usage. Dr. Zhao impressed upon the audience the need for appropriate clinical awareness and IVIG availability.

All told, this inaugural APSID event was a success and a significant step forward in describing a region that is not yet well understood in terms of clinical need. In addition, it fostered greater communication among several different groups of those interested in the realm of diagnosis and treatment of PID and related conditions. ●

JULIA FABENS, *PPTA Manager, International Affairs*

JOSHUA PENROD, *PPTA Vice President, Source & International Affairs*

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IPPC 2016: ETHICAL ASPECTS OF COMPENSATING PLASMA DONORS

BY SONIA BALBONI

Rare diseases are a key health policy priority in the European Union (EU) due to the limited number of patients and scarcity of relevant knowledge and expertise regarding particular diseases. Patients with rare diseases, some of them suffering from primary immune deficiency and other conditions treated by plasma-derived medicinal products, often spend years of uncertainty waiting for their disease to be diagnosed, and for an appropriate treatment to be found.

Three experts convened at Barcelona's International Plasma Protein Congress (IPPC) to review the ethical aspects of compensating plasma donors.

Typically, this type of debate involves two sides arguing whether it is ethical to remunerate donors. This time, however, Professor James Stacey Taylor turned the question upside down. He argued that it is, in fact, unethical to not offer donors the chance to be compensated. Per Professor Taylor, it is wrong to prohibit compensation in an environment where the product is scarce. Such is the case for plasma, said Taylor. In his view, without enough plasma to meet patients' needs, governments are essentially blocking access to care when they disallow compensated donation. He hinted that individuals are in fact exploited when they are asked to donate plasma without remuneration, if the individual soliciting the plasma is, in fact, capable of offering compensation. Professor Taylor suggested that, in today's reality, there is a place for both compensated and non-compensated donation. For him, it is more ethical to foster a system where both compensated and

non-compensated forms of donation co-exist. Allowing both possibilities, he says, gives the donor control over their choice.

Dr. Stuart Youngner, MD, considered the bioethical aspects of the compensation issue. He argued that payment for plasma donation is ethical in a well-regulated environment, where all stakeholder interests are taken into consideration. Professor Youngner reviewed the typical arguments against compensated donation and, using an established framework, refuted the value of each claim. He asserted that the evidence does not support the arguments against remuneration of plasma donors. He also concluded that continued quality improvement keeps industry vigilant for plasma safety and other moral principles relating to plasma collection.

Attorney Cristiana Spontoni considered the ethical aspects of special labeling on final products. She asked whether it was ethical to assign different labeling to products that were sourced using compensated donations. She questioned whether such labeling stigmatized the product, leading patients to believe products made with compensated donations are less acceptable than other products.

Ms. Spontoni discussed some specific cases in Europe. In France, for example, there is a proposal to add to product packaging an emblem reading, “Ethical Label.” Products would receive this label if they were manufactured using only “non-compensated, anonymous” donations. Products without the label would not be available for sale in the public hospital market. According to Ms. Spontoni, only one product on the market, which happens to be manufactured in France, would meet the criterion. Ms. Spontoni also explained the newest proposal from France, which would require a special label on all European products, indicating the country of origin for source plasma.

Ms. Spontoni noted this type of protective discrimination is not new. In the Netherlands in 2004, for example, a statutory obligation was made to require that a product’s outer package indicate whether the therapy was made from paid or unpaid donors. Due in part to PPTA’s advocacy, however, the Dutch initiative was lifted. Most recently, per Ms. Spontoni, the Italian Ministry of Health launched a contest for the



In France, for example, there is a proposal to add to product packaging an emblem reading, “Ethical Label.”



Per Professor Taylor, it is wrong to prohibit compensation in an environment where the product is scarce.

creation of a special label for plasma-derived medicinal products that exclusively use plasma collected in Italy from voluntary unpaid donations. Thus, companies not collecting exclusively in Italy using voluntary unpaid donations would be excluded from use of the label. The French and Italian cases are still pending. ●

SONIA BALBONI, PPTA Senior Manager, Source & Standards

An article¹ in a previous edition of The Source detailed the actions PPTA took to address the labeling issues in France, Italy, and Europe. Since publication of that article and following the PPTA actions, the French government has not taken any further steps. The question about the proposed special EU label regarding the origin of the manufacturing steps of pharmaceutical products, however, remains active, and PPTA is taking measures to follow up. PPTA is also actively opposing the Italian ethical labeling matter. We sent a communication to the Italian Ministry of Health and to the Permanent Representation of Italy to the European Union, outlining the arguments. Future developments on these issues will be reported in this magazine.

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IPPC 2016: Patient Voice Inclusion in the Organization of Care

BY NICK HICKS

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This was a very engaging and motivating session in which three patient representatives gave inspiring but different perspectives on patient inclusion in care, including a speaker from a non-related plasma protein user group on policy best practice within oncology.

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PLUS VISION AND PATIENT INVOLVEMENT IN EUROPEAN REFERENCE NETWORKS

Johan Prevot, Executive Director International Patient Organisation for Immunodeficiencies (IPOPI)

PLUS (Platform of Plasma Protein Users) addresses the unique needs of patients with rare diseases that use life-saving plasma protein therapies. It is a multi-stakeholder collaboration of which IPOPI is one of the founding organizations, representing the views of more than 110,000 European patients. PLUS has undertaken a focused call for action addressing fundamental issues such as equitable access, improving diagnosis rates, the establishment of patient registries, and the recognition of the unique nature of plasma protein therapies in national policies.

“PLUS is a great example of the value of strong patient groups in positively affecting health policy and important access decisions,” said Mr. Prevot.

Central to the success of the PLUS initiative are the consensus conferences, which identify ongoing issues and develop consensus statements and action plans to tackle the specific issues via multi-stakeholder working groups. These consensus statements, along with the supporting dialogue and rationale, are submitted for peer review prior to publication.

High on the current agenda are the necessary amendments to the EU blood directive. PLUS believes the amendments should state that the needs of patients should determine the optimal collection of blood and plasma. The May 24, 2016 meeting will seek consensus on potential improvement to parts of the Directive and, in particular, the need for clarification of what should apply to blood and blood collection and what should apply to plasma and plasma collection.

“The increasing clinical demand for plasma [proteins] is a major health issue for patients; we need to make the general public aware of the importance of plasma collection using all available sources, whether it is by compensated donation or not,” Mr. Prevot noted.

IPOPI is also currently involved in setting-up the ERN on Rare Immunological and Auto-Inflammatory Disorders whose aim is to improve the quality and safety of and access to highly specialized health care for these rare conditions. Professor Andrew Cant, European Society for Immunodeficiencies, is coordinating the project. IPOPI has been providing logistical support to the ERN from the start of the process and is providing the patient voice in the discussions. The ERN will provide a clear EU-endorsed framework to optimize access to diagnosis services, specialist treatments, and will also facilitate cross-border health care through a patient-centered approach. Another notable achievement was the publication of the Principles of Care in Frontiers of Immunology, which sets out clear guidance on what is best practice throughout the primary immunodeficiency (PID) patient journey.

PATIENT PERSPECTIVES IN SPAIN

Laura Miralles Paya, Association Espanola de Deficits Inmunitarios Primarios (AEDIP)

Having successfully overcome various life challenges before finally being diagnosed in 2014 with PID, Laura gave an insightful and personal reflection of her own PID journey. “While geographical differences exist, the principal problem in Spain is late diagnosis due to insufficient resources and lack of co-ordination between centers,” said Ms. Miralles Paya. While the problems facing PID patients in Spain are common to some other countries, their combined effect has a significant detrimental effect on patient care.

Spain is decentralized into 17 regions each having its own diagnosis management policy, resulting in significant inequalities in care and treatment. PID is a high burden disease with significant long term economic impacts due to hospitalization and sickness rates. The health care infrastructure is not set up to address the unique challenges imposed by PID.

AEDIP’s mission statement is to “Improve the quality of life of PID patients.” At present, many regions do not have an immunology service in their hospitals. Patients are faced with



Jana Pelouchová (ECPC), Laura Miralles Paya (AEDIP), and Johan Prevot (IPOPI) participate on a panel at the 2016 IPPC.

“The increasing clinical demand for plasma [proteins] is a major health issue for patients; we need to make the general public aware of the importance of plasma collection using all available sources, whether it is by compensated donation or not.”

additional logistical challenges if they need an infusion and their hospital is not a reference center. A PID registry exists but is not updated. The real incidence of PID is probably double that currently reported. Consequently, PID is not treated as a major health issue. In all regions within Spain, there is a lack of a common strategy for PID. There are clinical immunology services in several regions but for various reasons these are uncoordinated, sometimes due to lack of knowledge or sub-optimal contact between the immunologist and the unit hospital manager. There is need for a standard policy to improve the current system of PID management. The changes in immunology training given to doctors reflect the lower importance given to PID when compared to other diseases. AEDIP is committed to improving the current situation by raising awareness of PID and creating a health care infrastructure that provides the level of care needed for sufferers of the condition. “The current focus of health management is short term without an overall vision, and needs to change, so that it is on the patient,” says Ms. Miralles Paya.

An IPOPI initiative, in which AEDIP is becoming involved, is to improve early diagnosis of severe combined immune deficiency (SCID) in Spain where survival is currently between 45-65 percent compared to the U.S. where survival rates are above 90 percent. This initiative, which kicked off with two

IPOPI parliamentary events in Paris and Rome in 2014 and 2015 respectively, attracted experts, Members of the European Parliament, Ministry of Health representatives and national pediatric organizations. The program uses a patient-centered approach and will play a pivotal role in creating a clear EU endorsed framework to optimize cross-border collaboration and care.

ACCESS TO CARE ISSUES FOR RARE CANCER PATIENTS

Jana Pelouchová, European Cancer Patient Coalition (ECPC)

With her opening statement of, “Nothing about us, without us”, Ms. Jana Pelouchová gave an enlightening talk on some of the significant patient centered policy initiatives in which the ECPC has been involved leading to improved care and access for rare cancer patients. She was diagnosed with a rare form of chronic myeloid leukemia (CML) in 2002, which Ms. Pelouchová described as “a lonely cancer because you are robbed of people going through the same thing.”

The ECPC represents 383 cancer patient groups in 44 countries. Run by patients for patients, the ECPC has four main roles: advocacy, capacity building, partnership, and research. All of the patient focused policies relate to one or more of these strategic pillars. The ECPC has given cancer patients a stronger voice through European-focused initiatives like the RARECAREnet project whose aim was to build an informative network providing comprehensive information on rare cancers. The aims of RARECAREnet, which is funded by the European Commission health program, were focused on improving diagnosis of rare cancers, creating equal access

to care and treatment, and creating standardized care for rare cancers across Europe.

The RARECAREnet approach is a classic example of effective capacity building; linking European and national initiatives through the use of new technology, and mobilizing national patient groups who developed a list of treatment centers in collaboration with Orphanet and national health ministries, using online surveys. A standard questionnaire on topics such as center identification and the way rare cancers were diagnosed and treated was prepared. The patient groups had previously been validated by a combination of email and telephone follow-up using native language speakers, to ensure they were still functioning. Out of the original 275 patient groups, 145 responded to the request for information. Information supplied by the groups was put into a dedicated web-based database divided into sections.

“The project team undertook a gap analysis to identify existing information and what was needed and what was of interest as sometimes they are not the same thing,” said Ms. Pelouchová. The results were tabulated and showed that patients were interested in clinical symptoms, diagnosis, treatment, centers of treatment, economic support, social life reintegration, and clinical trials.

RARECAREnet is part of the Joint Action on Rare Cancers, a collaboration between member states authorities, cancer institutes, and patient organizations and will create guidelines for the development of ERNs for rare cancers. ●

NICK HICKS, *Communitaire advocacy healthcare communications*

A woman with long brown hair is lying in a hospital bed, smiling and looking at a tablet computer. In the background, another patient is lying in a bed, and medical equipment is visible.

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IPPC 2016: Blood and Plasma

VISIONS FOR A SUSTAINABLE SYSTEM

BY WILLIAM MURRAY

The 2016 International Plasma Protein Congress closed with a session featuring industry leaders talking about the future of blood and plasma donation. The session featured Dr. Kari Aranka, Executive Director of the European Blood Alliance; Dr. Stephan Walsemann, Chairman of the European Plasma Collectors Committee; Dr. Paul Strengers, Executive President of the International Plasma Fractionation Association; and Mr. Jan M. Bult, President & CEO of the Plasma Protein Therapeutics Association (PPTA).

David Bell of Grifols, Inc. and Chairman of the PPTA Global Board set the tone for the session by asking, “How do we provide for a sustainable system that meets the need of the patients that we truly have the obligation to help and protect?”

While each speaker presented unique and often differing opinions on how to best manage a sustainable system of blood and plasma donation, there were some common themes that emerged. First and foremost is that everything is done for the benefit of the patients. Secondly, is the realization that the human factor is what makes the blood and plasma industry unique. This applies to not just patients but also donors, which leads to another common point – the industry is dependent upon citizens willing to give their blood and plasma so that those in need receive blood, plasma, and plasma protein therapies that are often

life-saving. Finally, all agreed that donor safety, patient safety, and safe, high-quality therapies are, and must remain, the focus of the industry.

Where there were differing opinions, much discussion centered around the issues of compensation for donors and self-sufficiency. These issues are intertwined and some of the presenters believe that plasma should be collected without compensation and, further, there should be more balance between the United States, which currently supplies the vast majority of the plasma used for therapies around the world, and the rest of the world, specifically Europe.

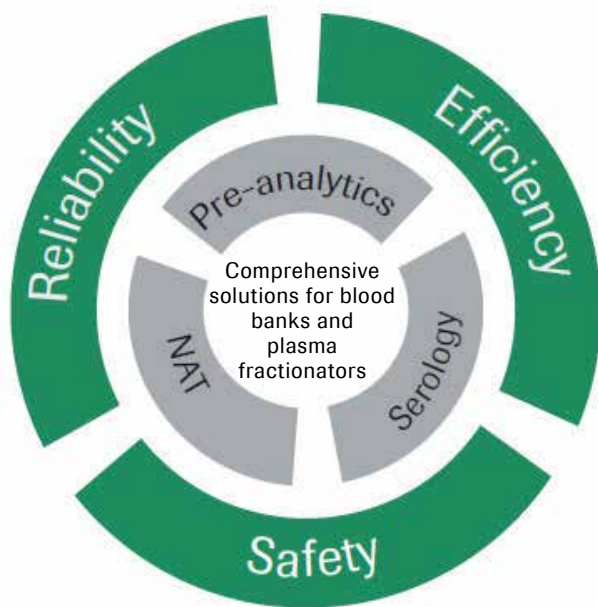
On the other side of the argument, Mr. Jan M. Bult closed the session by noting that the industry has been driven by patient access and maintaining the safety of donors and therapies. Mr. Bult noted that every country has the obligation to ensure the health of its citizens but self-sufficiency should not be dogma and, indeed, many countries no longer pursue self-sufficiency after realizing it was neither practical nor even possible. Mr. Bult noted, as did other presenters, that there is insufficient access to therapies for many patients around the world for various reasons. It is only through cooperation between the non-profit and for-profit sectors that patients around the world can receive the life-saving therapies they need. •

WILLIAM MURRAY, PPTA Director, Global Communications



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Collecting Hyperimmune Anti-D Plasma in Germany – A REAL CHALLENGE

BY ALEXA WETZEL

When a pregnant woman has Rh-negative blood and the baby in her womb has Rh-positive blood there is an Rh incompatibility, which can have serious consequences for the infant. The pregnant women are treated with Rh immune globulin during and immediately after pregnancy to prevent sensitization to the D antigen. It works by binding any fetal red blood cells with the D antigen before the mother is able to produce an immune response and form anti-D IgG.¹

More than 70 years after World War II, there are no more D negative war veterans who were immunized after receiving D positive blood transfusions.

Fifty years ago, Germany started to administer anti-D prophylaxis to D negative women who had just delivered a D positive baby, thus preventing the formation of antibodies in the women that could harm future pregnancies. Therefore, D negative women who were immunized as a result of their pregnancies are at least 70 years old and too old for plasma donation.

Today donors have to be specifically recruited and actively immunized several times with D positive red blood cells. This is currently done only in the United States and leads to Europe's dependence for the sufficient supply of hyperimmune anti-D plasma.

The legal basis for anti-D plasma collection in Germany is the "Guidelines for the production of plasma for specific purposes (Hyperimmune plasma) - first revised version (2000)," developed by the scientific advisory board of the

German Chamber of Physicians in collaboration with the Paul Ehrlich Institut.

The following are important issues to consider:

COLLECTING RED BLOOD CELLS FOR IMMUNIZATION

In Germany, red blood cells for immunization are licensed products. Currently, due to the extremely complex application requirements, only two blood transfusion services have a license.

One of these blood transfusion services would be able to collect red blood cell concentrate from its own donors for the production of hyperimmune plasma and proceed to cryo-conservation, storage in quarantine, thawing, resuspension, and transport to plasma collection centers. Even the requested compatibility analysis of the red blood cell donor and the recipient plasma donor could be carried out. However, the condition that red blood cell donors have to be immunized for Hepatitis A/B (a requirement of the German guideline) cannot be fulfilled by any of the transfusion services because they do not have the license.

INSURANCE COVERAGE

The guidelines state, “Physicians and centers that operate according to these guidelines have to ensure sufficient insurance coverage.” Blood and plasma donors are insured through a general accident and travel insurance that includes also prepared actions (such as damage in relation with donation). However, to date it seems that there is no insurance company in Germany that would insure participants of an anti-D program. This implies that such a program would not be feasible.

FINDING THE RIGHT PLASMA DONORS

The entire donor pool of a plasma collector has to be screened for D type. Statistically, about 15 percent of the donor pool would be D negative. By excluding premenopausal women from potential donors, approximately nine percent of donors would remain. This means that from a donor pool of 10,000 reliable, regular donors, a maximum of 900 would be potentially available as candidates for an anti-D program.

HEPATITIS A/B VACCINATION OF PLASMA DONORS

The recipients of the red blood cells have to be vaccinated for Hepatitis A (HAV) and Hepatitis B (HBV) according to the German guideline and the successful immunization for HAV and HBV has to be proven before donation.

This requirement leads to a delay of the anti-D program of at least six months and to a loss of 3.5 percent of the potential participants. It is to be expected that 30 of the 900 candidates would be non-responders to HAV or HBV immunization.

COMPENSATION

§ 10 of the German Transfusion law states that a donation has to be unpaid. The donor may receive compensation related to the time and effort and to the type of donation.

However, the level of effort is completely unclear in this context. The maximum amount of compensation for a plasma donation has been fixed at €25 in Germany. The outcome of the procedure is not without a certain amount of risk. In addition to the risk associated with forming an anti-D antibody, the donor could develop unwanted antibodies against other antigens.

NON-RESPONDER RATE

According to literature and experiences with anti-D programs in the United States, the non-responder rate is about 50-60 percent, even after several immunizations.

It could be inferred that if every 10th potential participant of the remaining 870 donors agreed to donate, it is to be expected that of 87 immunized donors approximately only 48 donors, of the initially 10,000 donors, would build up a sufficient antibody titer.

NON-RESPONDER

Donors that were not able to build anti-D have to be deferred from further blood and plasma donation because of the multiple immunizations and related sensitization. Regrettably, these donors are usually part of a very committed and reliable donor population. Because potential donors have to be educated about the risks, many of the candidates might be afraid of a permanent deferral and refrain from participating in the program.

SUCCESS RATE AND DEMAND

For the period of the program, to avoid needless deferrals, donors with sufficient titers have to be asked not to travel outside the European Union and not to get pierced or tattooed.

Assuming that donors will not get sick for a longer period or deferred for a low iron-level or an insufficient total protein level, a potential average volume of 15 liters (about 20 plasma donations) per year per donor might be collected. Such a program would allow the collection of about 720 liters of anti-D plasma per year. This volume would allow the production of more than 10,000 doses of anti-D prophylactic medication.

In Germany, around 675,000 babies are born per year and the estimated demand would be about 200,000 doses per year. Additional doses could be needed for miscarriages and other pregnancy related complications. To be able to meet the demand in Germany, more than 100 donors are needed to regularly donate plasma. This can only be reached if at least 40 collection centers in Germany start their own anti-D programs.

CHALLENGES

The production of anti-D plasma requires much planning and effort. The support of the fractionators is also needed to justify the immunization of donors and the potential related health risks. It could be problematic to inform committed donors after two years of participation in a program that their contribution is no longer favored simply because a contract has come to an end.

The collection of anti-D plasma is also very expensive. It is impossible to make an exact cost estimate because of all the unknown factors and the amount of potential compensation.

CONCLUSION

In order to have a successful hyperimmune anti-D plasma collection program in Germany, a revision of the hyperimmune guideline would be needed. Finding adequate insurance coverage and defining appropriate compensation for donors would be a first step. The support and collaboration of the fractionators is also indispensable. But, of course, nothing would be possible without the contribution of committed donors. ●

ALEXA WETZEL, PPTA Senior Manager, Source Europe

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European Haemophilia Safety Surveillance: CONTINUATION OF PATIENT REGISTRY SERIES

BY MIKE MAKRIS AND ESTELLE GILMAN

The development of adverse events to treatment has affected the hemophilia community more than any other factor. Incredibly, prior to 1985 when viral inactivation was introduced, the chance of being infected with hepatitis C with a first exposure to FVIII concentrate was 100 percent. Clotting factor concentrates these days are a lot safer but adverse events continue to occur.

Adverse event reporting strategies are available in almost every European country, yet most adverse events to clotting factor concentrates have not been reported to these systems in the past. There are many reasons for the failure to report but the main two are the belief that the events (e.g. inhibitors) are well known and not required to be reported, and secondly, busy clinicians who lack the time to report the amount of required information.

The European Haemophilia Safety Surveillance (EUHASS) was developed with the backing of the European Association for Haemophilia and Allied Disorders (EAHAD), the health professionals' organization and the European Haemophilia Consortium (EHC), the patients' organization in Europe. The intention was to develop a simple, electronic system that covered all inherited bleeding disorders and all clotting factor concentrates. The events that are reported are acute and allergic reactions, transfusion transmitted infections, inhibitors, thrombosis, malignancies, and deaths.

Centers can report all events as they occur or they can report quarterly. At the end of each quarter, all centers confirm

they have reported all their events or that they have not had any events during the period. Once a year, centers report the number of patients they have registered with each inherited bleeding disorder as well as the number of people treated with each concentrate during the year.

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EUHASS produces three types of reports:

- a) Quarterly reports summarizing events that have been reported during each three month period.
- b) An annual, more detailed report for each full year of surveillance.
- c) Product-specific reports for each concentrate.

EUHASS was created on Oct. 1, 2008 and has been funded mainly by two grants: one from the European Commission for 60 percent of the cost and the other from pharmaceutical manufacturers for the remaining 40 percent of costs. The European Commission funding ended in May 2015 and, since then, the project continues with just pharmaceutical industry funding. The following 10 manufacturers provide equal financial support to the project: Baxalta, Bayer, Biotest, CSL Behring, Grifols, Kedrion, Novo Nordisk, Octapharma, Pfizer, and SOBI/Biogen; LFB and BPL provide a smaller level of support.

Among the observations arising from EUHASS monitoring is the absence of transfusion transmitted infections by the currently used products. A total of 144 acute or allergic reactions were reported of which 15 were anaphylactic and 93 of the events occurred within 30 minutes of concentrate administration. EUHASS accepts reports of inhibitors in previously untreated patients (PUPs), previously treated patients (PTPs), and mild patients. In contrast to some recent publications, EUHASS did not find a significant difference in PUP inhibitor rates between plasma-derived and recombinant concentrates or between second- and third-generation recombinant products. An unexpected finding was the reporting of 160 new thrombotic episodes which had previously been thought to be very rare events in this population. Finally, EUHASS collects events of malignancy and death because of the possibility that these may be related to concentrate treatment. The most common malignancy reported is hepatocellular carcinoma and the



Among the observations arising from EUHASS monitoring is the absence of transfusion transmitted infections by the currently used products.

most common cause of death is liver failure, both of which are secondary to the hepatitis C infection that the clotting factor concentrate treatment transmitted 30 years earlier.

The success of the EUHASS strategy is primarily due to its simplicity, large size, prospective nature, and the fact that a single plan covers all products and all different inherited bleeding disorders. Among its weaknesses are the limited nature of the data collected, the anonymous nature of the reports, and the lack of central testing of inhibitors or audit of the data reported by the centers.

While EUHASS is a European-based system, an almost identical system that uses the same software and methodology is now running in Canada and is called CHES (Canadian Hemophilia Surveillance System). There is agreement that in the future EUHASS and CHES will produce combined reports. ●

MIKE MAKRIS AND ESTELLE GILMAN, *University of Sheffield, U.K.*



Plasma-based Drug Makes Big Impact for Young Patients

BY DAVID ARNOLD AND JOHN MCVEY

At five and a half months old, Ayden suddenly stopped feeding, became lethargic, and soon began to go limp. One pediatrician had a hunch that it could be infant botulism—a rare but potentially life-threatening disease with only one treatment option—BabyBIG®, a public service drug. After a single dose by IV, Ayden began a dramatic recovery.

BabyBIG® is produced every five to seven years as part of a joint effort with about 10 different entities. Baxalta was approached almost 10 years ago to be involved due to its expertise in plasma fractionation and specialized production facility in Los Angeles, Calif. Feeling strongly about its responsibility to contribute its expertise and capabilities for these patients, the company quickly stepped up to help.

Three times since 2008, the company has manufactured the drug in bulk and collaborated with partners to accomplish the donor boosting and collection of plasma from donors who have been vaccinated to raise their anti-botulinum toxin antibody levels. Baxalta coordinates with the plasma collectors to secure this hyper-immune material, then carries out the plasma fractionation, stability, and final container release testing. Some employees involved in the project put their normal jobs on hold for more than 18 months during these campaigns to focus solely on the complex process.

For the most recent BabyBIG campaign, a new investigational recombinant botulinum vaccine for boosting antibody levels in volunteer plasma donors needed to be qualified per an FDA requirement, a change that added time to the overall process of producing a new lot of BabyBIG®.

Once immunized donors were located, Baxalta reached out to Blood Centers of the Pacific (BCP) and The Interstate Companies (IBBI). BioLife Plasma Services, the plasma

collection arm of Baxalta, briefed BCP on source plasma collection requirements and, together, these teams quickly fulfilled the necessary volume requirements. There is a very limited supply of the plasma and new plasma units cannot be collected again for several years, so there is no margin for error. Given the limited pool of qualified donors, the collected plasma is a precious resource.

With plasma supplied by IBBI and BCP, Baxalta has produced two lots of bulk BabyBIG®, each enough to treat the roughly 120-150 patients who are diagnosed annually. While the company is reimbursed for costs in producing BabyBIG®, it makes no profit from it. The treatment is distributed to families for free; the fee for the medicine is paid by the hospital, which is reimbursed by third-party insurers. Treatment with BabyBIG® shortens hospital stay for an affected baby by almost one month, much of which would have been time in the intensive care unit. BabyBIG® treatment reduces hospital costs by almost \$100,000 per patient, and since its introduction into clinical practice, BabyBIG® use has cumulatively resulted in more than 75 years of avoided hospital stays and in more than \$100 million in avoided hospital costs.

Baxalta is inspired knowing that with BabyBIG®, children like Ayden can fully recover from infant botulism. Ayden's mother, Cherie says, "I do feel that having BabyBIG® accessible helped. Since then, she's been healthy and we feel really fortunate." •

DAVID ARNOLD, L.A. Plant Manager, Baxalta

JOHN MCVEY, Head of Quality and Regulatory Affairs for BioLife Plasma Services

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- Immunoassay development

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Inside PPTA

● The European Plasma Alliance

BY ALEXA WETZEL



The discussion on strengthening the role of the European Plasma Collectors Committee

(EPCC) as a recognized stakeholder was one of the key points at the last PPTA Source Planning Meeting in Memphis, Tenn. In order to increase the stature of the EPCC in Europe and to increase industry participation in various forums, it was thought that a new brand and identity would be necessary. In order to move forward, the committee members suggested a name change. Three alternative names were shortlisted by the group and the name “European Plasma Alliance” (EPA) was adopted by the majority.

History

The creation of the EPCC goes back to 2001. The purpose was to advocate for plasma collection in Europe and to foster patient access to plasma-derived medicinal products (PDMPs). Fifteen years later, the role of the committee is still to provide input to European and national regulatory authorities and policymakers and to improve the landscape for plasma collection in Europe. At the time of its foundation, plasma collection took place predominantly in Germany and Austria. Today, our members are also engaged in the collecting of source plasma for industrial manufacturing in the Czech Republic and Hungary.

The committee, led since 2014 by chair Dr. Stephan Walsemann (KEDPLASMA/Kedrion Group), is an alliance of 14 European Source Member companies that operate a total of 97 plasma collection facilities with 69 in Germany, 17 in Austria, seven in Hungary, and four in the Czech Republic. In 2015 they collected more than 2.4 million liters of plasma for the manufacturing of life-saving plasma protein therapies.

Projects

In the past year the committee focused on a variety of projects emphasizing plasma collection in Europe. Among these projects was an effort focused on a non-harmonized value-added tax (VAT) system in Germany. According to



Dr. Stephan Walsemann has led the European Plasma Alliance, formerly the European Plasma Collectors Committee, since 2014.

some German States, plasma for manufacturing is exempted from the VAT, much like blood and blood components for therapeutic use. PPTA, in collaboration with a tax specialist, developed an industry position paper on this issue with the objective of starting a dialogue with local tax authorities to discuss that plasma for manufacturing is a starting material for industrial use and as such should be applicable to VAT. It quickly became clear that this was not only a German issue and that the VAT application for plasma was part of a gray area in several European Member States under the umbrella of the VAT Council Directive 2006/112/EC.

At the same time, the Finance Court of Kassel in Hesse, Germany convened an oral hearing regarding the VAT status of source plasma. During the course of the hearing, the Chairman of the Court quoted at length from the PPTA position paper in contrast to the arguments made by the tax authorities. As a result, the Court decided to suspend the proceedings and referred the matter to the European Court of Justice for clarification on certain matters of VAT interpretation. The final ruling is expected by year-end.

In light of a potential review of the current European Blood Directive 2002/98, PPTA's European Health Policy

Steering Committee invited representatives of the EPA to participate in different workshops on how to differentiate blood for transfusion and plasma for further manufacturing in order to provide input for a new legislative proposal.

Due to the difficulties in finding physicians to work in collection centers in some areas of Germany, the EPA supported a project that would allow physicians to delegate some tasks to qualified medical staff. Outreach activities to politicians resulted in a draft legislative proposal that was handed to members of the German parliament.

PPTA and EPA representatives met with staff of the European Commission to discuss industry's priorities in the Transatlantic Trade and Investment Partnership (TTIP) negotiations. One of these priorities is to achieve regulatory convergence toward U.S. acceptance of European plasma. Currently, concerns regarding variant Creutzfeldt-Jakob Disease, as well as a number of technical differences in the regulations governing plasma collection, function as a de facto ban on European plasma from the U.S. market. The U.S. policy has also created a negative perception of European plasma that has led to bans from other markets.

In 2016, the committee will continue its efforts to strengthen the role of the EPA as a recognized stakeholder and become an active player in the European landscape.

EPA's chairman was invited to participate in the last Platform of Plasma Protein Users (PLUS) Consensus Meeting to present the industry's viewpoint on the potential Blood Directive Revision. He also participated in an animated panel discussion at the last International Plasma Protein Congress (IPPC) that featured some of the foremost leaders in the industry to present the visions for a sustainable system in Europe.

Source plasma donation is an important activity that contributes to saving lives. For many patients with rare diseases, PDMPs are the only therapies available to treat their chronic conditions. Unfortunately, diagnosis and treatment is still suboptimal in many European Member States and patients have limited or no access to plasma protein therapies. It is therefore important to raise awareness of stakeholders that more plasma for manufacturing is needed to mitigate the current limitations. ●

ALEXA WETZEL, PPTA Senior Manager, Source Europe



Professor Flora Peyvandi Awarded the 2016 Hilfenhaus Award

Each year, PPTA recognizes an individual who has made a significant contribution to patient access to safe plasma protein therapies with the prestigious Hilfenhaus Award.

This year's award was presented to Professor Flora Peyvandi, M.D., Ph.D. Since 1998, Professor Peyvandi has more than 300 studies published in well-reputed specialized journals and she has been the recipient of more than 40 project grants funded by Italian and International organizations. Her fields of research are clinical and basic science in rare bleeding disorders, von Willebrand disease, inhibitor in hemophilia and physiopathogenesis of microangiopathies.

PPTA has recognized industry leaders such as Dr. Peyvandi with the Hilfenhaus award since 1998. The award is named in honor of Dr. Joachim Hilfenhaus, a well-respected virologist who unfortunately passed at a young age in 1996. Dr. Hilfenhaus was the first Chairman of the Industry Experts Working Group on viral safety and he worked for Behringwerke in Marburg, Germany.

The 2016 award was presented by Dr. Oliver Schmitt, CSL Behring, and Chairman of the European Board of Directors. ●

MEET THE

Global Operations Team

As the global trade association representing the collectors of source plasma and the manufacturers of plasma protein therapies,

PPTA has a dedicated team that provides support to PPTA staff and Association members around the world. PPTA's Global Operations team has a diverse set of skills that keeps the Association running and meets the needs of our membership and vendors. These skills include human resources, legal affairs, membership, information technology, finance, and facility management to name just a few.

The Global Operations Team guides the Association through its annual external audit of the U.S. and European financial statements and ensures that the Association is following best practices for financial reporting. The Association also uses Automatic Clearing House Monitoring system to prevent bank fraud.

Because the Association operates globally and has staff in Europe, it has unique challenges, especially around eCommerce. The Global Operations Team makes certain that the Association uses the most secure eCommerce systems available including Payeezy-First Data for all credit card transactions in the U.S. and OGONE-Ingenico Payment Services in Europe to address fraud and risk management.

Safeguarding the assets of the Association is a crucial goal of the Global Operations Team. In addition to the systems and software mentioned above, the team monitors financial issues and events to protect the Association's cash reserves held in financial institutions. The Global Operations Team works with PPTA leadership on the annual budget, which is approved by the PPTA Global Board of Directors.

PPTA's Global Operations Team is the backbone of the Association. PPTA runs like clockwork because of the hard work, expertise, and focus of this team.



John Delacourt
Vice President,
Legal Affairs
& Global Operations



Cathy Izzi
Senior Director;
Human Resources &
Administrative Services



Mike McCormick
Director, IT & Facilities



Charon Smith
Senior Manager, Accounts
Receivables/Payables



Gene Stroshane
Controller

Patient Notification System

*for the plasma-derived and
recombinant analog industry*

www.PatientNotificationSystem.org
1.888.873.2838

The Patient Notification System (PNS) is provided at no cost and is a confidential, 24-hour communication system providing information on plasma-derived and recombinant analog therapy withdrawals and recalls.

The system was created to provide consumers with a single, convenient, and confidential source for up-to-date withdrawal and recall information. Led by the Plasma Protein Therapeutics Association (PPTA), the Patient Notification System was developed by the manufacturers of plasma therapies with direct input from consumers.

HOW THE SYSTEM WORKS

Anyone interested in participating registers with the Patient Notification System and provides general contact information, including their preferred methods of notification. Registrants have the opportunity of being notified by:

E-mail, telephone, or fax - which ever is most convenient for them. Please consider e-mail as your method of notification for the following reasons:

- >> E-mail is instantaneous
- >> E-mail is trackable
- >> E-mail is accessible, even on travel

If a therapy is withdrawn or recalled, the company involved immediately contacts Stericycle Inc. which then directly notifies the registrant. Every effort will be made to notify registrants within 24 hours. Each registrant will also receive a letter by first-class mail to ensure receipt of the information.

For current information on therapy recalls or withdrawals. To maximize the usefulness of the system, it is important for consumers to keep accurate infusion logs and record the lot number, therapy name, and manufacturer for all therapies used.



To make accessing the PNS site easier for users, the Association has developed a QR code which is a machine-readable code and will allow users to scan a barcode with a smart phone and immediately be taken to the PNS website to register.

In addition, consumers can go online to:

www.patientnotificationsystem.org

or call a 24-hour, toll-free number:

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(1-888-873-2838)

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Upcoming Events

CONFERENCES & SYMPOSIUMS

June

- 14 – 15** Plasma Protein Forum
Washington, D.C., U.S.
- 24 – 26** 25th Annual Alpha-1 National Education Conference
Miami, Fla., U.S.

July

- 8 – 10** Immune Thrombocytopenia (ITP) Conference
Orlando, Fla., U.S.
- 21 – 23** National Hemophilia Foundation (NHF) 68th Annual Meeting
Orlando, Fla., U.S.
- 24 – 28** World Federation of Hemophilia (WFH) 2016 World Congress
Orlando, Fla., U.S.

August

- 23 – 26** 2016 National Ryan White Conference on HIV Care and Treatment
Washington, D.C., U.S.

September

- 21 – 24** 17th Biennial Meeting of the European Society of Immunodeficiencies (ESID)
Barcelona, SPAIN
- 23 – 24** Guillain-Barré Syndrome (GBS)/Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) Foundation International Symposium 2016
San Antonio, Texas, U.S.

October

- 7 – 9** European Haemophilia Consortium (EHC) Annual Conference
Stavanger, NORWAY
- 9 – 15** International Plasma Awareness Week (IPAW)
- 11** PPTA Business Forum (Members only)
Orlando, Fla., U.S.
- 22 – 25** American Association of Blood Banks (AABB) Annual Meeting
Orlando, Fla., U.S.

November

- 5 – 6** 12th Annual Symposium on Primary Immunodeficiency
Newport Beach, Calif., U.S.

JUNE 13, 2017

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PPTA's 25th Anniversary Special Event

GLOSSARY OF TERMS

AAAAI – AMERICAN ACADEMY OF ALLERGY, ASTHMA & IMMUNOLOGY

AEDIP - ASSOCIATION ESPANOLA DE DEFICITS IMMUNITARIOS PRIMARIOS

A-PLUS – AMERICAN PLASMA USERS COALITION

APSID - ASIA PACIFIC SOCIETY FOR IMMUNE DEFICIENCIES

BCP – BLOOD CENTERS OF THE PACIFIC

CDC – CENTERS FOR DISEASE CONTROL AND PREVENTION

CHESS – CANADIAN HEMOPHILIA SURVEILLANCE SYSTEM

CML – CHRONIC MYELOID LEUKEMIA

EAHAD – EUROPEAN ASSOCIATION FOR HAEMOPHILIA AND ALLIED DISORDERS

ECPC – EUROPEAN CANCER PATIENT COALITION

EHC - EUROPEAN HAEMOPHILIA CONSORTIUM

EMA – EUROPEAN MEDICINES AGENCY

EPA – EUROPEAN PLASMA ALLIANCE

EPCC – EUROPEAN PLASMA COLLECTORS COMMITTEE

ERN – EUROPEAN REFERENCE NETWORK

EU – EUROPEAN UNION

EUHASS – EUROPEAN HAEMOPHILIA SAFETY SURVEILLANCE

EURORDIS – EUROPEAN ORGANISATION FOR RARE DISEASES

FDA – UNITED STATES FOOD AND DRUG ADMINISTRATION

GBS – GUILLAIN-BARRÉ SYNDROME

HAE – HEREDITARY ANGIOEDEMA

HAV – HEPATITIS A VIRUS

HBV – HEPATITIS B VIRUS

HTC – HEMOPHILIA TREATMENT CENTER

IG – IMMUNOGLOBULIN

IPFA – INTERNATIONAL PLASMA FRACTIONATION ASSOCIATION

IPOPI – INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

IPPC - INTERNATIONAL PLASMA PROTEIN CONGRESS

JARC - JOINT ACTION ON RARE CANCERS

MASAC – MEDICAL SCIENTIFIC ADVISORY COUNCIL

NHF – NATIONAL HEMOPHILIA FOUNDATION

NIH – NATIONAL INSTITUTES OF HEALTH

PDMP – PLASMA-DERIVED MEDICINAL PRODUCT

PID – PRIMARY IMMUNODEFICIENCY

PLUS – PLATFORM OF PLASMA PROTEIN USERS

PPTA – PLASMA PROTEIN THERAPEUTICS ASSOCIATION

PTP – PREVIOUSLY TREATED PATIENT

PUP – PREVIOUSLY UNTREATED PATIENT

SCID – SEVERE COMBINED IMMUNE DEFICIENCY

SPAC – STATE PATIENT ACCESS COALITION

TTIP - TRANSATLANTIC TRADE AND INVESTMENT PARTNERSHIP

VAT – VALUE-ADDED TAX

ZIKV – ZIKA VIRUS

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