Plasma Protein Therapies: Uniquely Different

Current Developments: EU Blood Directive

Interview with Dr. Franz Weinauer

PPTA Q&A with Alvin E. Roth, Ph.D.
Every one of our Fresenius Kabi Plasma Consultants is a seasoned expert in center management and best-practice implementation. Together with our award-winning customer service team, they help your people, your operations, and your business to deliver experiences that go above and beyond your donors’ expectations.
IN MY VIEW

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Enhanced Donor Compensation. Simplified.

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On a regular basis, we see and hear the debate about cost-saving measures for pharmaceuticals. Too often is our sector immediately drawn into this debate because many persons do not understand why we are so different from what we call traditional pharmaceuticals. In this column, I will try to summarize the arguments again.

Plasma protein therapies (PPTs) are used by individuals with rare diseases. Together these individuals make up small patient populations. We are not talking about the blood pressure medications, diabetes therapies, or psychiatric drugs that millions of people use. That in itself is already a differentiator. It has to be understood that the costs for the development and manufacturing of plasma protein therapies are borne by a relatively small group of individuals.

Some recipients of PPTs (e.g., persons with hemophilia) have a choice between therapies. These include plasma-derived clotting factors, recombinant clotting factors, monoclonal therapies, and even gene therapy. Other recipients of PPTs do not have a choice and depend completely on the supply of plasma-derived medicinal products. The manufacturing of these therapies is very different than products made from chemical compounds.

The starting material for PPTs is human plasma from committed healthy donors who are willing to donate on a regular basis to help their fellow citizens. The number of plasma donations needed for a year of therapy is enormous: 130 for immunodeficiency, 900 for Alpha-1 Antitrypsin Deficiency, and 1,200 for the production of factor VIII. It is not difficult to understand that the costs to manufacture therapies from these many donations are substantially higher than the production of a chemical compound.

That is not all. It is just the beginning. There is a strict donor selection procedure that includes medical and diagnostic checks to ensure that the donor is not carrying an infectious agent. After the donation, the plasma is kept in inventory hold for at least 60 days before the manufacturing can start. This adds a level of safety because it allows for post-donation information to be provided. If that information indicates any reason for donor disqualification had the information been available at the time of donation, this added step ensures that the industry can trace this donation and discard the donation before the manufacturing process starts. Having the plasma inventory sitting in storage for 60 days is an enormous financial investment that does not exist for other industries.

Then the manufacturing process starts and involves separation, purification, and viral inactivation, just to name a few requirements. Every step involves robust quality control and quality assurance measures. Even after the distribution to a patient, monitoring and vigilance continues.

Unlike the manufacturing of traditional pharmaceuticals, the time between the plasma donation and distribution to the individual who needs therapy averages between seven and 12 months!

It is good to see that more and more persons are starting to understand why this industry is unique and cannot be subjected to the same cost-containment measures as we see for pharmaceutical drugs. But it is not enough. We need to continue to help more people understand the different nature of our industry.
The European Commission has been assessing the EU’s Blood Directive of 2002 since 2013 with the objectives of understanding the complexities of the sector, evaluating how the Directive functions, and, depending on the outcome, possibly starting the legislative process for the revision of this key legal act. Where do we stand today? What is the outlook?

WHERE DO WE STAND?
Overview: The Blood Directive is currently undergoing an evaluation process, in which PPTA actively participates as one of the EU Commission’s recognized and trusted stakeholders. The evaluation process is in line with the Commission’s Better Regulation Package and aims to assess whether the legislation has achieved its original objectives and whether or not it is still fit for purpose. The evaluation started in 2017 with a road map and continued with a comprehensive EU public consultation as well as a stakeholder event organized by the EU Commission. ICF Consulting Services Ltd. (ICF) has been tasked with conducting an independent study, for which a report was due Summer 2018. The evaluation process is expected to conclude later this year with the publication of the Commission’s final evaluation report, which will be based on information gathered from these various sources. Key findings from the EU public consultation published by the Commission include (among others):

• “Inadequate and/or unclear key definitions in the Blood Directive.”
• “Legislation not up-to-date with scientific, technological, epidemiological, or societal developments and process of updating not flexible or quick enough to adapt to them.”
• “Absence of any provisions for ensuring sufficiency of supply, highlighted particularly by patient groups that see lack of access as key risk to patients.”

Such findings show the stakeholders’ dissatisfaction with the functioning of the Blood Directive and indicates the need for revision.
THE ICF STUDY

At the time of publication, the results of this independent study had not yet been published, but preliminary findings were presented to a validation focus group where PPTA participated. The final ICF study will produce an evidence base that enables the Commission to complete its own final evaluation relating to directive 2002/98/EC and the associated implementing directives, referred to collectively as “blood legislation.” The ICF study findings are addressed in five evaluation criteria: relevance, effectiveness, efficiency, coherence, and EU added value.

Below are the summarized main study findings:

- Blood legislation does “not take sufficiently into account the differences between blood and blood components and plasma for fractionation.”
- It also does not clearly define: Compensation in relation to the VUD (voluntary and unpaid donation) principle” and leaves unclear the scope of the term “self-sufficiency.”
- Blood legislation is “not adequately adapted to changes and developments, and therefore is unable to meet current regulatory needs.” The legislation is “inflexible, especially regarding the consideration of detailed technological requirements, and there is a lack of inclusion of scientific and technological advancements, which can help increase safety and quality.”
- European Directorate for the Quality of Medicines guidelines are considered to be relevant because they are updated biannually, but “some stakeholder[s] feel that they should be created and implemented in a transparent way.”
- EU blood legislation is “not sufficiently adaptable to keep pace with clinical demand and practice, commercialisation, and internationalisation developments in the sector.”
- The directive’s “lack of emergency preparedness plans” is highlighted.
- It is stressed that “more should be done to encourage plasma collection in the EU, in particular by the EU Member States (MSs),” which might be “achieved through an adaptation of the VUD principles or through other measures like a strategic plan for collection capacity.”
- Finally, the summary findings conclude that the EU blood legislation has “added value to the regulation of blood across MSs by setting up common standards for safety and quality, and even helped to facilitate the development of comprehensive legislative framework for the first time for several MSs”; however, “a more uniform
interpretation is needed at a national level across the EU to help increase harmonisation and facilitate mutual recognition of procedures, tests, and products.”

- These findings again stress that the current blood legislation has merit but also addresses important shortcomings that would justify the need to revise this outdated legislation.

It confirms PPTA’s opinion that the Blood Directive is not adapted to scientific and technological regulatory advancements, and it neither addresses a crisis preparedness plan element nor does it keep pace with clinical demand. Thus, the directive needs to be revised.

OUTLOOK
PPTA eagerly awaits the publication of the EU Commission’s official evaluation report, which will be available by the end of 2018. It is understood that this report will focus on the evaluation outcome and will not contain any explicit recommendations for further handling of the EU blood legislation. Therefore, any possible revision of the blood legislation will not be started before the European Parliament elections in May 2019.

The identification of next steps and the possible start of a legislative process regarding the revision of the EU Blood Directive will be reserved for the new European Commission elected in Fall 2019.

Until then, PPTA will continue advocating for a directive revision. Conceptually, there seem to be three possibilities for a revision:

1. Full revision of the EU “Mother” Directive 2002/98/EC with a new structure and new content; or
2. Recasting of the “Mother” Directive 2002/98/EC, keeping the structure but changing the content; or,
3. Amending only the secondary EU Blood Directive legislation, the “Daughter Directives,” with focus solely on regulatory and quality requirements.

There are several signals for openness regarding a potential revision of the directive. These signals come from leading members of the European Parliament and also from the current EU Health Commissioner, Mr. Vytenis Andriukaitis. On World Blood Donor Day, held June 14, 2018, Mr. Andriukaitis published a statement that alludes to potential directive revisions, by stating the following:

“The European Commission is currently carrying out the first formal evaluation of the blood legislation since its adoption in 2002. As there have been many scientific, societal, and epidemiological changes since 2002, I believe that our citizens deserve to benefit from the latest developments in health and safety.”

-Mr. Vytenis Andriukaitis,
EU Health Commissioner

“As there have been many scientific, societal, and epidemiological changes since 2002, I believe that our citizens deserve to benefit from the latest developments in health and safety.”
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Norcross, Georgia 30093

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With eight months until a hard Brexit, the British government is experiencing some turnover and there remain more questions than answers. On July 8, 2018, Brexit Secretary David Davis resigned from the cabinet, citing his lack of enthusiasm for Prime Minister Theresa May’s approach to Brexit. The next day, Foreign Secretary Boris Johnson also resigned from the government over Brexit disagreements. Both Davis and Johnson believe Prime Minister May’s plan is too accommodating to the European Union (EU). Former housing minister Dominic Raab will replace Mr. Davis at the head of the Department for Exiting the European Union (DexEU). Jeremy Hunt has been appointed as the new foreign secretary replacing Johnson.

The United Kingdom’s (UK) Brexit plan (hereinafter “The Plan”) that caused Davis and Johnson to resign was released in a detailed white paper on July 12. The Plan would establish a free trade area for goods. Under this plan, the UK and EU would maintain a common rulebook for all goods, and the UK would commit by treaty to ongoing harmonization with EU rules on goods, covering only those necessary to provide frictionless trade at the border. This would ensure a seamless border between Ireland and Northern Ireland. It would also continue the supply chains for plasma protein therapies.

Item Number 28 of The Plan states, “The UK believes that manufacturers should only need to undergo one series of tests in either market, in order to place products in both markets. This would be supported by arrangements covering all relevant compliance activity, supplemented by continued UK participation in agencies for highly regulated sectors including for medicines, chemicals, and aerospace.”

PPTA is hopeful that the EU is able to work with the UK in ensuring something like the proposed free trade area for goods. In a letter to Brexit negotiators for the EU and UK, we raised concerns with the impact of regulatory changes on the import and export requirements of plasma protein therapies, as well as the regulatory procedures that could cause disruptions to the supply of plasma protein therapies to patients in the EU and the UK.
As a major component of the Abbott total solution, the ACCELERATOR a3600 utilizes true open connectivity to create process efficiencies in the blood and plasma service laboratory by automating inefficient manual processes. Elevate your laboratory’s performance to the next level with personalized solutions to eliminate bottlenecks, improve productivity and reduce labor costs. Contact your Abbott representative to learn more about innovative solutions that can transform the health of your blood and plasma supply.

EXPERIENCE PERSONALIZED INNOVATIONS
The EU responded to our letter with a reassurance that the withdrawal of the United Kingdom from the EU is not going to impact the EU’s regulatory framework for substances of human origin and medicinal products. The European Commission is working closely with other EU institutions and bodies, as well as Member States, to ensure preparedness. Stakeholders have also been made aware of the need to prepare for the withdrawal.

In response to our call for future cooperation between the EU and the UK to ensure patients have access to their medically appropriate therapies, the EU mentioned the key principles set out in the guidelines of the European Council of March 23, 2018, on the framework for the future EU-UK relationship. In this document, the heads of state and government of the EU hope to maintain the UK as a close partner in the future but caution that the UK shall not have the same rights and enjoy the same benefits as a Member State.

This response warns of the need to prepare for a scenario where the two parties cannot come to an agreement and the UK becomes a third country. In preparation for this, the European Medicines Agency (EMA) has already advised stakeholders to move all UK-based activities to EU/European Economic Area (EEA) countries, including:

- Transfer of all Marketing Authorization (MA) activities, including orphan designation: Any MA Application (MAA) Orphan medicinal product UK-designation must be transferred to an EU/EEA Orphan Designation-Holder;
- Transfer of all safety activities: The Qualified Person for Pharmacovigilance will need to be located in an EU/EEA country;
- Transfer of all manufacturing and quality/batch release activities: Any active substance manufactured in the UK will be classified as an imported substance; any finished product manufactured in the UK will be classified as an imported finished product and will thus need to have an authorized importer established in EU/EEA. The UK site of batch release will need to be transferred to the EU/EEA, and any clinical/bioequivalence/biosimilar and other studies done with substances sourced in the UK can only be used in the MAA if the MA is granted before March 30, 2019; any batch of finished product must be certified by a Qualified Person within the EEA before being released for placing on the market in the EEA or for export. Certification can only be performed by a Qualified Person of the manufacturer and/or importer who is identified in the Marketing Authorization and is located in the EEA.

In response to our call for FUTURE COOPERATION between the EU and the UK to ENSURE PATIENTS HAVE ACCESS TO THEIR MEDICALLY APPROPRIATE THERAPIES, the EU mentioned the key principles set out in the guidelines of the European Council of March 23, 2018, on the framework for the future EU-UK relationship.
Products and services for hemostasis research

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- Customized blood collection tubes
- R&D assay services

HTI is a manufacturer of highly-purified, native plasma proteins and associated products involved in the hemostatic system. Development of assays for research or destined for cGMP testing is also offered.

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- Stability & release testing
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How is the German blood and plasma donation system structured?
The particularity of the German system is that it is based on four organizations for the collection of blood, blood components, and plasma for manufacturing medicinal products. These four entities include:
• German Red Cross organizations
• Hospital blood banks
• Private (independent) collectors
• Industry collectors

The German Transfusion Act, together with the provisions of the Drug Law, regulates the production, quality, and licensing of all medicinal products, including blood products. The German Medical Association, in cooperation with the Paul-Ehrlich-Institut, establishes the guidelines for the collection, manufacture, and use of blood and blood components. In Germany, women can donate blood up to four times each year, whereas men can donate up to six times annually. Since the revision of the guidelines in 2017, plasma can now be donated up to 60 times a year.

Do these four organizations work together?
Yes, these four collecting entities coexist and work together. They are all members of the German umbrella association Arbeitsgemeinschaft Plasmapherese e.V. (ARGE). The mission of the ARGE is to promote safe plasma collection practices in Germany, with a focus on donor health and product safety to ensure patient access to safe products according to the current state of science and technology, in addition to scientific assessment of various aspects of plasmapheresis.

The ARGE currently has 21 members operating 92 plasma collection centers in 15 German states. The collaboration among the organizations is also reflected in the ARGE Board, which is composed of one representative from each of these collecting entities. The chair is currently Dr. Kirsten Seidel from CSL Plasma, and I serve as vice chair.

Together we work on different projects and have supported scientific studies such as SIPLA I and II on the safety of long-term intensive plasmapheresis in donors. This coexistence makes the German system work well and helps to ensure patients access to care.

Is Germany confronted by ethical concerns because donors are compensated?
The particularity of the German system is that it allows a fixed-rate monetary donor compensation. This is the so-called “Aufwandsentschädigung,” a German term that designates the fixed-rate compensation for expenses.
It is anchored in the German Transfusion Act and is in line with the voluntary and unpaid donation principle. The establishments are free to choose if they compensate their donors or not.

In the case of whole blood donation managed by Red Cross blood centers, there is no need for remuneration at all as we drive with our mobile teams to a location close to the donor. On the other hand, our experience has shown that plasma donation without compensation is not working. Donors have to come to the plasma center, the process is lengthy, and donors have to be committed, so it is justified to compensate plasma donors for their time and inconvenience. Otherwise, the supply with plasma products would not be achievable and thus justifies ethically the compensation.

**Do you think that there is crowding out happening in Germany, meaning do you think there is a loss of blood donors due to the activities of private sector plasma collectors?**

In the past years, Germany has observed a decrease in blood donations. However this is related to the progress in surgical practices and the introduction of patient blood management. These decreasing figures have been observed in many European countries, even in countries that do not have plasma collection programs in place.

It should also be mentioned that blood and plasma donors are not part of the same donor population. We observed that blood donors are on average older than plasma donors.

When we were preparing the opening of our plasma donation center in Würzburg, we sent letters to 1,200 of our blood donors (blood group AB) in the area, and in the end, we only got a handful of persons who actually came to donate. This is probably also related to the donation frequency. On average, a blood donor donates twice a year. A plasma donor donates on average 20 times a year. This is a whole different commitment and explains why it is more appealing to the younger population.

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### PLASMA FOR FRACTIONATION

*German plasma donation in years by collecting entity. Figures of the Paul-Ehrlich-Institut.*

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The ARGE Board met with Municipal Councilor of Kassel Renate Fricke (fourth from left) at the 2017 ARGE Congress in Kassel (Germany). Board members (from left to right) are Dr. Franz Weinauer, Bavarian Red Cross; Dr. Kirsten Seidel, CSL Plasma; Prof. Dr. Cornelius Knabbe, Hospital of the Ruhr-University Bochum; and Dr. Matthias Eberhard, TMD Kassel.
PPTA was honored to feature Nobel Laureate Alvin E. Roth, Ph.D., at the 2018 Plasma Protein Forum in June, where he gave a keynote address on issues surrounding compensated plasma donation, and how those issues connect to his work on “repugnant markets.” Dr. Roth was the co-recipient of the 2012 Nobel Memorial Prize in Economic Sciences “for the theory of stable allocations and the practice of market design.”

Dr. Roth is a market designer. He redesigned the way medical students are matched with residency positions, worked with several large cities to design school-choice systems, and designed the New England Kidney Exchange, which finds pairs of incompatible would-be kidney donors and recipients who can cross-donate. Consider a husband willing to donate to, but incompatible with, his wife, and a sister willing to donate to, but incompatible with, her brother. If the husband (donor) from the first pair and brother (recipient) from the second are compatible, then that transplant can be made instead. Dr. Roth’s system allows long chains of such transplants to be made and has saved thousands of lives.

During his presentation, Dr. Roth gave several examples of “repugnant markets,” in which a person is willing to sell something, but a third party does not agree that the sale should be allowed, which could describe how some feel about compensated plasma donation. Kidney donation is a favorite topic, and one in which he has done extensive work. He also points to lending money for interest as an example, showing how something once considered a sin is now part of the foundation of a modern economy. He explained that often, activities that are completely acceptable become repugnant once money is introduced, citing prostitution and surrogacy, among other markets.

Dr. Roth shared words of wisdom for the plasma protein therapeutics industry. He pointed out that the concept of repugnance can enter into a market if social support for that market is not preserved. In the case of compensated plasma donation, we do not see many opponents in the United States, particularly as compared to the number internationally, but Dr. Roth warned that continued emphasis on the health and safety of donors is paramount to ensuring ongoing support for and expansion of compensated donation.

After the Forum, PPTA followed up with Dr. Roth to get some of his impressions.
Thank you for joining us at the 2018 Plasma Protein Forum. Could you share with our readers your main takeaways? Was there anything that surprised you?
I was surprised at the diverse interests of the Forum participants, and the range of talks presented, from those focusing on specific diseases (angioedema, cirrhosis), to specific countries (Canada, China), and the diversity of speakers (industry experts, physicians, even economists).

Your work addresses “repugnant markets,” which you define as markets where a person is willing to sell something but a third party does not agree the sale should be allowed. That certainly describes how some feel about compensated plasma donation. You also point to markets where repugnance has been eliminated. What factors changed in these markets, and do you see opportunities in this industry for similar changes?
Some things that once attracted significant repugnance but are now widely admired include: interest-paying bank deposits (and the payment of interest more generally), insurance, and treatments for infertility (such as IVF [in vitro fertilization]). Attitudes toward each changed slowly. I think the changes in attitude were primarily a result of growing familiarity with the benefits each of these things brings and increasingly successful efforts to communicate these benefits.

Certain governments and international bodies promote the idea that compensated plasma donation is unacceptable. Have you seen any examples of repugnance being either created or eliminated in markets through government interference?
Because familiarity may reduce repugnance in cases where the transactions in question have widespread benefits, governments can maintain repugnance by passing laws forbidding certain practices. (And to the extent that this creates black markets run by criminals that produce bad outcomes, this can help reinforce repugnance for the underlying transaction.) For example, surrogacy is fully legal in California, but prostitution is illegal, while the reverse is true in Germany. To the extent that the legal markets are sufficiently regulated, witnessing their success can further reduce repugnance. Of course, even legal markets that produce bad outcomes can become repugnant through familiarity. Thus participants in legal markets have an interest in making sure they produce good outcomes and that these are communicated.

“SOME THINGS THAT ONCE ATTRACTION SIGNIFICANT REPUGNANCE BUT ARE NOW WIDELY ADMIRER INCLUDE: INTEREST-PAYING BANK DEPOSITS (AND THE PAYMENT OF INTEREST MORE GENERALLY), INSURANCE, AND TREATMENTS FOR INFERTILITY (SUCH AS IVF [IN VITRO FERTILIZATION]).”
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- **Strengthen donor relationships** by simplifying and accelerating data collection, reducing wait times and accelerating donor flow through the center.
Recognizing Excellence at the Plasma Protein Forum

Held annually, the Plasma Protein Forum (PPF) is an ideal occasion to recognize excellence and dedication to the plasma protein therapeutics industry. At this year’s PPF, David Bell, Chair of the PPTA Global Board of Directors and Chief Innovation Officer at Grifols S.A., used his time at the podium to present two individuals with well-deserved appreciation for careers spent advancing the industry’s goals and PPTA’s mission to promote the availability of and access to safe and effective plasma protein therapeutics for all patients in the world.

Mr. Bell called Jan M. Bult, President & CEO of PPTA, to the stage and presented him with PPTA’s Lifetime Achievement Award in recognition of his decades of service to PPTA and its industry members and the patients who benefit from access to PPTs.

Upon receiving the award, Mr. Bult noted his appreciation to the Global Board of Directors and offered gratitude to the entire PPTA staff for their work in support of PPTA’s mission. Shortly thereafter, Mr. Bell presented the 2018 Dr. Otto Schwarz Award to Mr. Shinji Wada, Vice President, Japanese Affairs, Grifols S.A. The Dr. Otto Schwarz Award is presented only to those individuals whose efforts and commitment have significantly advanced the plasma protein therapeutics industry.

PPTA congratulates Mr. Bult and Mr. Wada on their greatly deserved recognition and is pleased to offer sincere gratitude for their unwavering support of the plasma protein therapeutics industry throughout their long and distinguished careers.
Inside PPTA: Health Policy in Action

INTERNATIONAL AFFAIRS
Canada was a major focus for the PPTA International Affairs team in early 2018. In late April, British Columbia banned compensated plasma donation despite PPTA’s outreach to members of both parties, including the British Columbia Minister of Health, and outreach by patient advocates. Directly on the heels of the ban, Health Canada’s Expert Panel on Immune Globulin Product Supply and Related Impacts in Canada publicly released a report on its findings. PPTA and its members were afforded an opportunity to comment and did so, with positions grounded in science, quality, donor and patient safety, and availability. The yearlong evaluation was focused largely on the merits of compensated plasma donation. Though the report is favorable to the concept of compensated plasma donation, it proposes troubling demand-management strategies, which could restrict access to IG.

We continue to prepare for the second Plasma Protein Industry Summit at the Parenteral Drug Industry Congress. The summit will feature PPTA members and staff, as well as leading experts and Chinese and U.S. regulatory officials, and will focus on areas of possible cooperation. It will also feature discussions of how Chinese industry and PPTA might be able to find common ground on some existing differences.

EUROPE
PPTA’s Health Policy staff has continued working on several projects at the European level, such as the new Health Technology Assessment (HTA) Regulation. After its engagement in the Industry Association Stakeholder Pool earlier this year, PPTA recently participated in a meeting of European trade associations with the EU Health Commissioner Vytenis Andriukaitis regarding the current draft HTA Regulation. The meeting’s goal was to exchange views and concerns on the production of Joint Clinical Assessments (JCAs) and their governance. PPTA supported the idea of voluntary participation from industry in the JCAs to establish best practice, as well as a mandatory uptake of JCAs by EU Member States. PPTA also highlighted the role of trade associations as stakeholders and the need to clarify issues related to HTA methodologies.

Another current major topic at the European level is the reform of the pharmaceutical incentives by the European Commission. Earlier this year, PPTA provided its comments during the consultation period and, recently, a study on the legal aspects of the Commission’s Supplementary Protection Certificates, and has published a proposal for regulation on those certificates for medicinal products. Finally, with regard to advocacy at the European institutions level, PPTA reached out to some members of the European Parliament and advocated that they formally put forward a Parliamentary Question (PQ) in support of the need for more plasma in the EU. The answer from Health Commissioner Andriukaitis to the PQ provided fertile ground for PPTA’s future advocacy efforts. The discrepancy between current levels of plasma collection in the EU and the growing need for plasma-derived medicinal products (PDMPs) were acknowledged, and the importance of PDMPs for the treatment of EU patients was recognized.

NORTH AMERICA
The upcoming 2018 fall midterm election is one of the most momentous midterms in recent memory, as future control of the House and Senate are both unknown. Republicans previously expected to strengthen their majority in the Senate this midterm due to the 33 seats up for re-election, but when considering historical outcomes of midterm elections, the Democratic party now has an opportunity to regain control of both chambers of Congress. For Democrats to control the Senate, they only need to gain two seats and they now have a potential path to win 24 seats in the House and regain full control of Congress. This would establish a divided government for Trump’s final two years of his term, limiting his ability to pass any major legislation through Congress, forcing the White House to consider implementing policy reform via executive orders and other administrative procedures.

Considering this year’s midterm election, the priority on Capitol Hill has been on campaigning, rather than on hearings, policy, or legislation. However, health care reform has remained a focus for the Trump Administration, with the publication of the “American Patients First” Blueprint. PPTA responded to the Secretary of Health and Human Services’ Request for Information with comments currently posted on the association’s website. The comments urged the Secretary not to move reimbursement for plasma protein therapies (PPTs) in Medicare Part B to Part D and objected to the inclusion of PPTs in a flawed Competitive Acquisition Program. PPTA remains committed to its mission to promote the availability of, and access to, safe and effective plasma protein therapies for all patients in the world.
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Walking the Halls of Congress

By Chase Thomas, Assistant Director, Government Relations, PPTA

On Thursday, May 17, PPTA held its annual Capitol Hill Fly-In, where the Association brings together producers of plasma protein therapies, patients with rare diseases, and patient group representatives to “walk the halls” of Congress. The Fly-In provides the plasma protein therapy community an opportunity to inform Congress about the unique nature of these therapies and the chronic diseases they treat and to discuss policies that preserve access to safe and effective treatments. With the Trump administration releasing its “Blueprint to Lower Drug Prices,” on May 11, the 2018 congressional Fly-In was a timely opportunity to remind staff in Congressional offices of the value of plasma protein therapies. Further, the 2018 Fly-In came on the heels of the North America launch of the “How Is Your Day?” campaign and the February 2018 publishing of the economic analysis by Bates White Economic Consulting, “Key economic and value considerations in the U.S. market for plasma protein therapies.”

Congressman G.K. Butterfield (D-NC), who serves on the Energy and Commerce Committee and is co-chair of the Congressional Rare Disease Caucus, provided opening remarks and received a leadership award from the PPTA North America Board Chair Paul Blanchfield.

After Rep. Butterfield’s remarks and a quick briefing on Federal Government Relations issues by Tom Lilburn, Senior Director of Government Relations, the 32 participants departed the Reserve Officers Association and met with more than 50 congressional offices.
During our congressional meetings, PPTA stressed the importance of preserving policies that uphold necessary patient protections established in the Affordable Care Act. Specifically, we continue to advocate for protecting the “four pillars,” which are essential for preserving patient access to plasma protein therapies:

1. No exclusion for pre-existing conditions
2. No annual or lifetime caps
3. Ability for dependents to remain on parents insurance plans until age 26
4. Limit on maximum out-of-pocket expenses

In addition, PPTA advocated to ensure new insurance plans protect patients, as there is growing concern that administration efforts with association health plans, Medicaid waivers, and short duration plans could allow health plans with no prescription benefits and none of the above patient protections. States are implementing “non-insurance” health plans that could eliminate access to plasma protein therapies. Fly-In participants urged that Congress must exercise oversight to ensure adequate access protections for these extremely vulnerable patients.

PPTA appreciates everyone who was able to participate in the Fly-In this year and would like to encourage those interested in attending next year to please contact Chase Thomas, Assistant Director of Government Relations, at cthomas@pptaglobal.org. We hope to see you in Washington, D.C., for the 2019 congressional Fly-In next spring!
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PPTA attended and was pleased to exhibit for the first time at the World Federation of Hemophilia (WFH) World Congress, held May 20–24, in Glasgow, Scotland. The conference was attended by nearly 5,000 people with hemophilia, as well as family members and medical professionals who treat various aspects of patient health. The purpose of the exhibition was to highlight the recently launched “How Is Your Day?” global education campaign and to draw attention to PPTA’s voluntary industry standards programs.
In addition to sharing information about the campaign and the standards program during the event, PPTA representatives were able to personally meet with individuals affected by hemophilia and hear their emotional stories and testimonies. After spending several months gaining the support of national and international stakeholder groups, the WFH conference was PPTA’s first opportunity to share the “How Is Your Day?” initiative directly with patients, caregivers, and advocates. The response was overwhelming, as people from all over the world eagerly shared their stories and the impact of access to clotting factor on their lives. For example, Ekawat lives in Thailand and described his father carrying him to the hospital for infusions until he was 10 years old. Today, thanks to clotting factor, he runs his own graphic design company and is able to keep up with his two young and active children. Additionally, Mayda, who is an oral surgeon, understands how incredibly important access to clotting factor is for people living with bleeding disorders.

PPTA will continue discussing and promoting the “How Is Your Day?” campaign at patient meetings in the coming months and looks forward to learning from individuals whose lives are improved by having access to plasma protein therapies. Ultimately, the campaign’s goal is to showcase these stories to improve awareness of the diseases treated by these therapies, with the goal of working with legislators and payer sources to increase access globally for these unique medicines.

As Alain Weill, President of the World Federation of Hemophilia, stated, “Treatment for all: This is our global vision. I would love to have the situation, where, if we were to ask anyone, anywhere in the world, ‘How is your day?’ they would be able to answer ‘Fine!’ That would be a key achievement.”

PPTA’s standards exhibit educated attendees about its two voluntary standards programs, the International Quality Plasma Program (IQPP) and Quality Standards of Excellence, Assurance and Leadership (QSEAL) voluntary standards programs. Staff explained to attendees the level of commitment that companies invest in the programs, including high-level participation on the committees that develop and oversee the programs, meeting monthly to ensure that the requirements represent state-of-the-art safety protocols, efficiency, and reliability for plasma collection and manufacturing. Staff members, who welcomed visitor questions, also explained how the IQPP and the QSEAL programs work jointly to promote the quality and safety of source plasma and of the final therapies.

Visitors also learned about the roles of the National Donor Deferral Registry (NDDR), the Qualified Donor Standard, the Inventory Hold Standard, and other requirements of the programs that help to ensure plasma safety and product safety. Staff described how the audit programs work and the requirements for a center or facility to receive IQPP or QSEAL certification, respectively. Furthermore, staff gave conference attendees an overview of the standards designed to protect donor health, including the newer IQPP Fluid Administration Standard and the Donor Adverse Events Recording Standard. PPTA staff also answered questions about the debate in some countries surrounding compensated donation.

Staff had the opportunity to meet patients from all over the world, as far away as Africa and the Middle East and as near as the conference’s hometown of Glasgow. Each patient had a unique story to share. Many detailed their long journey toward achieving a diagnosis, which in some cases did not come until they were almost adults. Others conveyed the difficulty they had in obtaining therapy within their countries and, when therapies were available, the hours-long journeys they had to undertake to reach a facility where the therapy could be administered.

For some individuals, treatment meant simply the administration of cryoprecipitate, and even for that, only when a bleed occurred. Others in some regions had access...
to plasma protein therapies, but only thanks to grants and donations by generous individuals or companies. Parents discussed the anguish they felt in trying to get the right treatment to their children. One mother recounted how her son had to relocate to another country to be treated because therapy was not available in their homeland.

These patients’ stories, coupled with the promotion of the “How Is Your Day?” initiative and the sharing of information about the IQPP and QSEAL standards programs, helped reinforce for all those in attendance PPTA’s mission “to promote the availability of and access to safe and effective plasma protein therapeutics for all patients in the world.”

“I HOPE THAT ALL PATIENTS IN MY COUNTRY CAN HAVE ACCESS TO PLASMA PROTEIN THERAPIES SO THEY CAN HAVE A BETTER QUALITY OF LIFE; AND WHEN OTHERS ASK THEM, ‘HOW IS YOUR DAY’, THEY CAN SAY, ‘ALL IS WELL. BETTER THAN WELL, IN FACT!’”

Mayda, odontologist treating individuals with bleeding disorders

“MY FATHER CARRIED ME TO THE HOSPITAL IN BANGKOK, 90 MINUTES EACH WAY, FOR INFUSIONS UNTIL I WAS 10 YEARS OLD. TODAY, ACCESS TO CLOTTING FACTOR ALLOWS ME TO OWN MY OWN GRAPHIC DESIGN COMPANY AND PLAY WITH MY KIDS.”

Ekawat, living with Hemophilia
“How Is Your Day?”
Comes to Washington, D.C.

Gathering more than 300 attendees from across the globe, the Plasma Protein Forum in Washington, D.C. brings together experts from the plasma protein therapeutics (PPTs) industry, policymakers, as well as people living with rare, chronic conditions whose lives have been impacted by PPTs. For many patients, having access to PPTs has been life-changing and has allowed them to lead a healthy, active life by giving them the ability to do things some would conceive as “normal” such as riding a bicycle. In celebration of the profound impact of PPTs for so many, Forum attendees were invited to become a part of the “How Is Your Day?” initiative by riding a stationary bicycle and racing against the time of other attendees. With many attendees taking their turn on the bicycle, PPTA is grateful for all the support received for the “How Is Your Day?” initiative.

We invite you to follow, like, and engage with “How Is Your Day?” on Facebook and Twitter @HIYDglobal and online: www.HowIsYourDay.org.
MEET THE PPTA

Conference Planning Team

Throughout the year, PPTA hosts several events that draw members, policymakers, industry representatives, patient organizations, and representatives from various government agencies worldwide to hear from key speakers about current issues affecting the plasma protein therapeutics industry. The planning and organizing of these meetings is done solely by PPTA staff, which helps reduce the registration cost for attendees and provides an opportunity for PPTA to actively engage with registrants and sponsors. The conference planning team consists of staff members from a range of divisions, all of whom bring their expertise to their respective tasks to ensure each meeting is well-organized and runs efficiently. PPTA is grateful to the following staff members for their central roles in organizing our meetings:

MELANIE CONRADS, OFFICE ASSISTANT, EUROPE
PPTA hosts the International Plasma Protein Congress (IPPC) in Europe every spring, gathering more than 300 attendees. Melanie is the primary contact for IPPC’s organization and is responsible for all meeting logistics, including securing the venue, assisting speakers, and organizing the various committee and stakeholder meetings that take place during the week of IPPC. Melanie is always available to ensure PPTA meets the needs of all attendees.

LAUREN FREESE, EXECUTIVE ASSISTANT
The Plasma Protein Forum (PPF) is PPTA’s annual North America meeting. Lauren is the staff person with primary responsibility for ensuring attendees, speakers, and other VIPs receive hotel rooms at the host hotel and also partners with colleagues to secure conference rooms for the additional meetings which take place prior to and during PPF.

RACHEL LIEBE, ASSISTANT MANAGER, COMMUNICATIONS
To host a successful meeting, it is essential for PPTA members and other meeting attendees to become aware of, and stay informed of, our events and all their updates. Rachel uses her years of experience in graphic and web design to design all of the print, digital, and web-based content to market our meetings, provide signage, and to recognize each meeting’s sponsors and exhibitors. She also develops and manages the IPPC and PPF mobile apps, which allow attendees to receive updates about the program, learn more about speakers and sponsors, and also locate exhibitors throughout the exhibit hall.

MICHELLE MASON, COORDINATOR, GLOBAL REGULATORY POLICY/QSEAL ADMINISTRATOR
Sponsors and exhibitors are essential meeting supporters, as the fees paid help offset meeting costs, ultimately keeping expenses as low as possible for PPTA’s members and other event attendees. Michelle works closely with numerous companies to secure their support as sponsors and/or exhibitors and also ensures all their respective materials are shipped and arrive at each host hotel in time for each event.
KIMBERLY SEROTA, ASSISTANT MANAGER, GOVERNMENT RELATIONS
The PPTA North America team brings together producers of plasma protein therapies, patients with rare diseases, and patient group representatives each year for its annual Capitol Hill Fly-In in Washington, D.C., providing the community an opportunity to discuss policies that preserve access to safe and effective plasma protein therapies. Kimberly is essential to ensuring participants have a positive experience, securing meetings with Congressional offices and providing timely material to each office visited. This year, Kimberly managed the introduction of a Fly-In app to the participants; the app provided easy access to schedules, background information, and meeting materials.

In addition to managing the Fly-In, Kimberly also supports the PPF by coordinating with speakers, securing presentations, and lending support to the team whenever needed. This year she successfully promoted PPF and its speakers by integrating them into the North America division’s Facebook and Twitter platforms.

CHARON SMITH, SENIOR MANAGER, ACCOUNTS RECEIVABLES/PAYABLES
The attendee experience begins at registration, so PPTA has worked to make the registration process as seamless as possible by implementing a secure, cloud-based management system for event registration and payment processing. Charon manages the registration process and assists registrants with any potential issues as they arise.

BOBBI STACKMAN, ADMINISTRATIVE ASSISTANT, SOURCE
As the Forum’s primary staff contact, Bobbi is responsible for the meeting’s logistics, including hotel selection and speaker coordination.

SARA STEFANELLI, COMMUNICATIONS ASSISTANT, EUROPE
When registering for an event, attendees look for a robust and exciting agenda. Sara works with the PPTA Leadership Team to develop the IPPC agenda, communicating all updates with team members. Patient involvement is important for a successful event, so Sara reaches out to local patient groups to extend an invitation for the IPPC and organize the stakeholder meeting, regularly held in conjunction with the Congress.

PPTA staff is happy to answer questions and address concerns and we are always looking to improve the attendee experience, so please feel free to share your feedback! Be sure to check the PPTA website for future events.
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<td>September</td>
<td>IRIS (Association de Patients Déficits Immunitaires Primitifs) [French PID patient group] 20th Anniversary</td>
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<td>October</td>
<td>2018 European Haemophilia Consortium Conference</td>
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<td>Brussels, Belgium</td>
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<td>International Plasma Awareness Week (IPAW)</td>
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<td>National Hemophilia Foundation (NHF) 70th Bleeding Disorders Conference and Annual Meeting</td>
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<td>AABB Annual Meeting</td>
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<td>National Organization for Rare Disorders (NORD) Rare Diseases and Orphan Products</td>
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<td>PPTA Business Forum [Members only]</td>
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<td>Atlanta, Georgia, United States</td>
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<td>18th Biennial Meeting of the European Society for Immunodeficiencies (ESID)</td>
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<td>November</td>
<td>Guillain-Barré Syndrome (GBS)</td>
<td>Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) 15th Biennial Symposium</td>
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<td>Third International BioProcessing Asia Conference (see next page for details)</td>
<td>12 – 15</td>
<td>Langkawi, Malaysia</td>
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<td>March 2019</td>
<td>PPTA International Plasma Protein Congress (IPPC)</td>
<td>19 – 20</td>
<td>Amsterdam, the Netherlands</td>
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<td>May 2019</td>
<td>International Plasma and Fractionation Association (IPFA)/Paul-Ehrlich-Institut (PEI) 26th Workshop on “Surveillance and Screening of Blood-borne Pathogens”</td>
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<td>June 2019</td>
<td>PPTA Plasma Protein Forum</td>
<td>18 – 19</td>
<td>Reston, Virginia, United States</td>
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The clinical need for plasma protein therapies is increasing year after year and there is no reason to believe that this will stop. At the same time there are interesting developments with the introduction of new therapeutic modalities such as gene therapy, gene editing, and targeted monoclonal antibody therapies that will allow more personalized and precision medicine. These developments may lead to a shift from large scale plasma product manufacturing to cell culture based products for the corresponding medical indications. The question then becomes “What is on the horizon?”

This session investigates the current and future plasma derived products landscape and will highlight developments in Asia. Contributions also look into the cellular production of plasma proteins, not least to see how new modalities will impact the traditional economics of plasma fractionation. Product labeling is a new dimension in many areas such as personalized medicine, but also helpful to prevent counterfeiting. Another focus is on the experiences in Asia with respect to to plasma fractionation plant development, plant expansion and specific challenges in the plasma products manufacturing landscape in Asia.
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