

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

FALL 2016

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Evolving Technologies in
Plasma Collection

Overview of Plasma Industry
in Southeast Asia

Ensuring Patient Access to
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A woman with long brown hair, wearing a blue hospital gown, is lying in a hospital bed and smiling while using a tablet. In the background, another patient is lying in a bed, and medical equipment is visible.

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In My View

BY JAN M. BULT, PPTA PRESIDENT & CEO

In July, there were two big events for the hemophilia community. First, there was the meeting of the National Hemophilia Foundation followed by the World Federation of Hemophilia Congress, both in Orlando, Fla.

I have been able to attend these meetings for over 20 years and have witnessed the enormous progress that has been made in this field.

1. The number of persons who require wheelchairs and/or crutches is significantly lower than it was two decades ago. It shows that the treatment levels are getting better and that prophylaxis is effective. The sad thing is that most of the persons who require support are coming from countries where availability of clotting factors is a challenge.
2. The medical expertise is growing enormously. The number of presentations has increased, but more importantly, the understanding of (early) intervention is demonstrated over and over again. Also, the awareness of bleeding disorders in the female population is remarkable. More and more facts about how a deficit in von Willebrand factor affects women are available. It was not long ago when the general perception was that deficits of clotting factor proteins were thought to be a male issue.
3. Though the numbers are still small, there are good results reported with gene transfer in persons with hemophilia B, something that did not exist in the early 90's.
4. The number of available therapies has risen significantly, not only with plasma-derived therapies, but also with recombinant therapies. Relatively recently

we have seen the introduction of long-acting recombinant therapies.

5. There has been an important shift in focus. Decades ago, the focus was very much on safety. Today it is more on the development of inhibitors. It must be stated, however, that safety remains the most important issue for our industry and there is no room for complacency!

The changes are remarkable and very important. At the same time, there is the realization that only 25–30 percent of persons with hemophilia have a form of treatment. That means that the majority of persons with hemophilia in the world have nothing and are facing pain and discomfort—or even worse. The stories of situations where decisions have to be made to determine which person obtains a treatment and which person does not are heartbreaking. Triage is quite normal in emergency situations, but should not be necessary in hemophilia care.

There are several initiatives in place to provide humanitarian aid through the donation of clotting factors. What would be better is if all countries would recognize genetic disorders as a priority in their health care system and provide therapies for their citizens. ●

Jan M. Bult, PPTA President & CEO



PANELISTS DISCUSS:

“Evolving Technologies in Plasma Collection”

AT THIS YEAR’S PLASMA PROTEIN FORUM

BY SONIA BALBONI, PPTA SENIOR MANAGER, SOURCE & STANDARDS

We depend on information technology (IT) in some fashion for virtually every aspect of plasma collection. Information technology can be defined as, “utilization of computing via hardware, software, services, and infrastructure to create, store, exchange, and leverage information in its various forms to accomplish any number of objectives.”¹ In our industry, we use specialized machines to collect plasma, sophisticated freezers for storage, and specialized trucks for transport. Our systems store detailed information about every collection—including volume given, time of donation, and basic donor health parameters. The computers at our centers connect with corporate offices, testing laboratories, and industry databases. In every way, we are connected.

Today, competency in IT is one of the key drivers in the success of businesses in any industry. This is certainly true for plasma collectors. The efficiency of the collection process and the ability to maintain accurate information, are important factors in determining a company’s success. In the past, some of the IT capabilities that were factors in a company’s accomplishments were not possible to implement industry-wide. Reasons for this varied, but they included an organization’s size, its financial profile, or its business model. But now with enhanced technology, and through industry cooperation, capabilities that we did not believe feasible just a few years ago are now available to all companies, regardless of their size or makeup.

At a panel presentation during this June’s Plasma Protein Forum, three experts gave their insight on the value of information technology to a plasma collector.

First, Michael Taormina (Biotest) discussed the evolution of IT in industry-wide applications, in particular, the National Donor Deferral Registry (NDDR) and the Cross Donation Check System (CDCS). The NDDR was established to help ensure that donors deferred for reactive HIV, HBV and HCV tests at one facility do not donate at other facilities. Mr. Taormina noted that, in its primitive beginnings, the NDDR was strictly a telephone database. Before 1993, he noted, “the internet was not part of our [industry’s] common vocabulary.” As technology improved and internet use became more widespread, PPTA commissioned a secure website to host the NDDR database. This significantly cut down on donor intake time. Over time, centers’ computer systems continued to become more advanced, and the speed, security, and capabilities of the internet also improved. So in 2010, the NDDR software was upgraded to allow companies to conduct checks with a seamless interface along with other changes that were made to improve the efficiency and integrity of checks.

Mr. Taormina also provided more details about the CDCS, which was implemented early last year. Before 2015, centers used a fax process to check donors for evidence they might be at risk of donating more often than they are allowed. Cross donation can pose a serious risk to a donor’s health, which is



why it is prohibited. A voluntary International Quality Plasma Program standard required the cross donation checks, but the manual process was inefficient, cumbersome, and subject to human error. By 2015, there were several blood/plasma software companies with excellent technological capabilities and strong competencies in developing sophisticated computer programming sequences. A number of these companies submitted proposals to develop a web-based database that could handle the cross donation checks online. Haemonetics was selected to develop and implement the product, and the CDCS was born. This technology has already revolutionized the speed and accuracy in which donor checks are performed. Since the CDCS was implemented one year ago, the technology has already undergone one enhancement. “CDCS has created the opportunity for us to perform a cross donation check every time a donor visits a center, something that would have been unreasonable just two years ago with the faxes,” observed Taormina. Future developments are sure to come as companies’ capabilities continue to advance.

Next, Roger Brinser (BioLife/Shire) discussed the role of IT systems in plasma donor health. Plasma collectors have computer systems that allow them to collect donor data, beginning with the time that they check in, through their screening, examination, and plasma collection. Some companies’ systems are more sophisticated than others; however, all systems provide vital information that helps a company determine whether a donor is qualified to donate. As Brinser stated, “information is used to assess ongoing health and well-being of the prospective donors.” Companies can also review the donor health information in their IT system to find trends, which can help them to improve the quality of their source plasma. “It permits data driven enhancements and improvements to the donor eligibility process,” said Brinser.

The final speaker, Vlasta Hakes (Grifols), discussed how social media can help plasma donor centers connect with donors and communities. Hakes defined social media as any number of “online communication channels for sharing news, audio, video, photos, and content with people in one’s network.” (This would include applications like Facebook, Twitter, and Yelp.) With the click of a button, companies can use social media to instantly access audiences that were previously only available through expensive direct mail or print media campaigns. For Hakes, social media is another tool for the industry to raise awareness about the importance of plasma donation while engaging in meaningful conversations, regardless of the size or resources of the plasma organization.

According to Hakes, social media provides a company with three strategic opportunities in particular:

- Become a key source for credible information about plasma donation;
- Build relationships, educate, generate and improve awareness about plasma donation; and
- Proactively lead the conversation about donating plasma.

“Social media provides us the opportunity to shape the message that we want to tell. It is a key component of our overall awareness and marketing program,” says Hakes.

In the coming years, a plasma collector’s competency in IT will continue to play a large role in its success. Industry will continue to push the envelope on what can be done. Undoubtedly, new breakthrough technologies will emerge, allowing industry to improve its collection processes and more efficiently provide the quality plasma that is necessary for the manufacture of life-saving therapies. ●

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Overview of Plasma Industry in Southeast Asia

BY JULIA FABENS, PPTA MANAGER, INTERNATIONAL AFFAIRS

Southeast Asia, with its population of nearly a billion people and mostly middle-income countries, is forecast by the Organisation for Economic Co-operation and Development to grow at almost five percent over the next five years¹ and holds an increasingly important place in the future of the plasma protein therapeutics industry. For this article, we looked at the Southeast Asian countries of Indonesia, Malaysia, the Philippines, Singapore, and Vietnam, plus Taiwan, to get a clearer picture of the current state of the plasma protein industry in the region. Other countries that are traditionally included in the region are Brunei, Cambodia, East Timor, Laos, and Myanmar, but those countries are not currently involved in any significant plasma collection or fractionation.

Broadly, domestic recovered and source plasma collection is still tightly controlled by government authorities or government designated National Blood Centers and/or national Red Cross organizations. Most blood and plasma is collected from non-remunerated donors or replacement donors, with a small amount of remunerated whole blood donation still taking place.

A few of the high-income countries in the region—e.g. Singapore, Taiwan, and upper-middle income Malaysia—have contracted for toll fractionation, while two of the upper-middle income countries—Malaysia, again, and Thailand—are

advancing agreements to build domestic fractionation facilities. The lower-middle income countries reviewed—Indonesia, the Philippines, and Vietnam—have tested the waters, but only the Vietnam project has gained any traction thus far.

Thailand is an upper-middle income country with great interest in the plasma fractionation industry. National Blood Center operates a small plasma fractionation facility, capable of fractionating around 10,000 liters of plasma per year, and mainly produces albumin, hepatitis B immunoglobulins (IG) and rabies IG.² The Thai Red Cross and Green Cross Corporation (a Korean pharmaceutical company) signed an agreement to build a 200,000 liter fractionation facility slated to begin production of intravenous immunoglobulin (IVIG), albumin, and factor VIII this year.^{3,4} Plasma collection in Thailand is performed by the National Blood Service and consists mainly of recovered plasma, with some source plasma and some replacement donation.

Malaysia is another upper-middle income country with an interest in plasma fractionation. The country currently uses external fractionation for the production of FVIII, FIX, IVIG, and albumin. A Malaysian company has contracted with a European company for help constructing a 300,000 liter facility originally slated for completion in 2017,⁵ but limited

discernible progress has been made. Plasma for fractionation is collected in a limited number of centers in Malaysia, under the auspices of the National Blood Center. The National Blood Center Production and Fractionation Unit is responsible for receiving, processing, and delivering plasma for fractionation.

Singapore is a small island nation with a high-income economy located off the tip of the Malaysian Peninsula. Given its small size, traditional plasma fractionation facilities are not feasible, but other fractionation arrangements have been made. Toll fractionation is carried out and a Singaporean company built a 20,000–30,000 liter facility that will process plasma using a filtration membrane once it is fully operational.⁶

Taiwan, another high-income island nation, follows Singapore's model and sends plasma for fractionation by an external fractionator. In 2014, the country sent just over 31,000 kilograms of plasma for production of albumin, IVIG, FVIII, and FIX.⁷ According to the Taiwan Blood Services Foundation (TBSF), in addition to supplying Taiwan, the TBSF was able to donate 3,840 vials of FVIII to the World Federation of Hemophilia. Plasma is collected by the TBSF Blood Center in Taipei and donors are not remunerated.

In Vietnam, a local firm is currently building a 300,000 liter facility with technology transferred from a private European manufacturer. The facility is slated to begin producing albumin, IVIG, FVIII, and prothrombin complex in 2018.⁸ An earlier fractionation project was attempted, but abandoned due to a lack of quality plasma.⁹ The National Institute of Hematology and Blood Transfusion, which collects plasma along with the Vietnamese Red Cross, introduced nucleic acid testing last year, with the goal of expanding to all blood collection facilities by the end of 2018. Some blood donors receive up to 180,000 dong (\$8), though most are unpaid.¹⁰ The government has set a goal of 100 percent non-remunerated blood donation by 2020.

Indonesia may get a domestic fractionation facility, as well. In 2013, the Indonesian Red Cross and an Indonesian company agreed to cooperate on a fractionation facility¹¹ but again, there has been limited discernible progress. Indonesia was chosen as the pilot country for the World Health Organization/European Commission project for enhancing the availability, safety, and quality of blood products in low- and middle-income countries. As part of that study, a 2013 audit of Indonesian blood centers by three international fractionators revealed some “critical deviations” from established best practices, but the project did give Indonesia a clear path toward producing plasma suitable for fractionation. The Indonesian Red Cross collects the vast majority of blood in Indonesia, from non-remunerated and replacement donors.

Currently, the majority of plasma recovered from whole blood by the Indonesian Red Cross is discarded, according to the Market Research Bureau.

The Philippines has shown interest in a domestic fractionation industry, but has not yet made substantial progress toward that goal. There is currently no domestic fractionation capability, though an agreement was signed in 2011 to look into building a membrane filtration plasma processing facility¹²—that project has yet to materialize. Blood in the Philippines is collected by the Philippine Red Cross, regional blood centers, hospital-based blood banks, and local government units. Some plasma is used to make cryoprecipitate in hospitals, but limited clotting factor is available in the country.

There are still several factors preventing the region from becoming more integrated with the global plasma industry, including lack of quality plasma, low diagnosis rates of the life-threatening diseases that plasma protein therapies (PPTs) treat, and a lack of awareness of patient need. Bright spots can be seen here however—the number of patient registries are increasing and patient groups are forming, such as the Southeast Asian PID network (SEAPID) and many local PID groups. Hopefully with the support of industry, regulators, patients, and physicians on the ground, the development of the PPT industry in SE Asia can continue down a path of further integration to best serve the thousands of patients living there. ●

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The EU Clinical Trial Regulation (Regulation 536/2014) – A New Way Forward for CLINICAL TRIALS IN EUROPE

BY: DOMINIKA MISZTELA, PPTA MANAGER, REGULATORY POLICY EUROPE

BACKGROUND: FROM DIRECTIVE TO REGULATION—REGULATORY FRAMEWORK FOR CLINICAL TRIALS IN THE EUROPEAN UNION

The EU Clinical Trial Regulation 536/2014 (Regulation) (Regulation 536/2014)¹ was approved by the European Parliament on April 16, 2014 and came into force on July 16, 2014. It is expected that it will be fully implemented toward the end of 2018.

Unlike previous legislative pieces governing clinical trials (CTs) in the European Union (EU)—such as EU Clinical Trials Directive 2001/20/EC (Directive)², Directive 2005/28/EC³, International Good Clinical Practice⁴, and Declaration of Helsinki on ‘Ethical Principles for Medical Research Involving Human Subjects’⁵—this is a Regulation, which makes it legally binding for the 28 Member States (MSs) of the EU (EU-28)⁶ (see sidebar). It aims to harmonize submission, authorization, and conduct of CTs across Europe and, ultimately, facilitate access to innovative and novel medicines for patients and maintain the competitiveness of the EU market for research and innovation.

The Regulation will replace the EU Clinical Trials Directive 2001/20/EC (Directive), which has been the regulatory basis of CT conduct in Europe since 2004, and seeks to address several criticisms of the Directive. Unlike the Regulation, the Directive provided the legislative goals for CTs in the EU but left the actual requirements to the individual MSs. It earned criticism from industry, academia, and patient groups due to its divergent and inconsistent interpretation across different MSs. The legislative

intent of the Directive permitted and, in many instances, required each of the EU-28 MSs to have additional, country-specific legislation and guidelines that transposed the Directive into national requirements and governed the conduct of CTs on a national level.

Due to this, over the past years, the conduct of CTs required growing staffing and administrative costs in order to comply with complex and seemingly bureaucratic regulatory requirements, rising insurance fees, and a growing number of novel medicines, which resulted in significant delays in trial set-up and start, as well as a decrease in the number of CTs approved and conducted in the EU.⁸

CLINICAL TRIAL PORTAL AND DATABASE—AN ‘ALL-IN-ONE’ APPROACH

Under the Regulation, EU member states will remain responsible for the authorization and oversight of CTs under the Regulation. However, the practical aspects of the Regulation were made possible through two main technical developments, which were put in place and will be maintained by the European Medicines Agency (EMA)⁹:

- A single submission portal, to allow the ‘physical’ submission, authorization, and supervision of CTs, and
- A CT database, which will hold the results of the CTs.

The Regulation will apply six months after both the submission portal and the database have been declared functional by EMA.¹⁰

The single submission portal will streamline the CT application (CTA) procedure, as it will only require one set of documents (also called a CTA dossier) to be submitted for a CTA for several potential participating countries. The dossier will be prepared and assessed according to harmonized requirements, and a single decision on key aspects of the trial (see below) will be given. Thus, in theory, by having streamlined requirements, using a single application dossier, submission of a trial in up to 28 potential participating countries could be performed at the same time.

ASSESSMENT PROCESS

The assessment of the CTA will be performed in two parts. Part I will be jointly assessed by all MSs involved; however, only one designated national body or agency will formally provide feedback to the applicant, with one contact point, with one fee to be paid for the entire review. As a result, a single opinion will be issued on the CT protocol, Investigator's Brochure (IB) or Summary of Product Characteristics (SmPC), Investigational Medicinal Product (IMP) information, safety, pharmacovigilance, and risk-management provisions.

Part II of the CTA will be assessed by individual MSs based on national requirements. These include ethical aspects—such as set-up of ethics committees, duration of the assessment process, and conditions to be fulfilled by the applicant, including submission language, types and number of documents to be submitted, data protection and data retention requirements, specifics of patient information and patient recruitment, and assessment of liabilities and insurance needs in the case of

damage(s). In addition, local site requirements, such as qualifications of investigator(s) and set-up and requirements for clinical trial sites, as well as country-specific manufacturing, labeling, and import requirements for the medicinal product will be considered.

Depending on preferences of the CT applicant, part I and II can be reviewed in parallel; or part I can be reviewed first, followed by part II. This will be decided by the applicant prior to the submission and is expected to shorten the timelines for the review—if, for instance, the applicant chooses a parallel part I and II review option. The entire review process will take between 106–156 days for advanced therapy medicinal products, such as genetically modified IMPs and cell-therapies.

The CT database will contain information on all CTs conducted in the EU, including details on authorization procedure(s), start of trial, suspension(s), temporary hold(s), or early termination(s) as well as details on informed consent, general conduct of trials, and safety information.

OTHER MAIN CHANGES BROUGHT ON BY THE REGULATION INCLUDE:

- Direct safety reporting into EMA's EudraVigilance database, instead of submission of safety information to each individual MS participating in the CT;
- Co-sponsorship will be permitted, recognizing the complexities of current CT conduct often requiring multiple stakeholder involvement;
- Risk-based monitoring, whereby the sponsor, based on the risks and interventions associated with the CT, will determine the extent and nature of monitoring needed;
- Introduction of the definition of so-called 'low-interventional' or 'low-risk' trials, if the IMP is used within existing license or its off-license use is standard practice and the safety profile of the IMP is known (so further risk-adaptation), resulting in accelerated application process and the number of requirements and conditions to be fulfilled as well insurance costs; and
- Public access to CT results.

PUBLIC ACCESS TO CT DATA AND TRANSPARENCY

Public access to CT results is one of the most debated aspects of the Regulation. The Regulation requires that data from clinical study reports are public; under the Directive these have largely been viewed as commercially confidential information (CCI). Exceptions for public disclosure will apply to personal data, communication between MSs during the assessment of the CT, as well as certain commercial information to protect the intellectual property rights and/or Marketing Authorization (MA) status of the medicinal product, in accordance with predefined 'redaction' requirements—and unless public interest prevails to disclose this information.¹¹

Overview of EU Legislative Framework

The EU legislative framework is implemented into national laws of the individual MSs through several different legal acts⁷:

- **Regulation:** Binding legislative act; it must be applied in its entirety into national regulation(s) in the MSs.
- **Directive:** Direction-giving legislative act; its application and transposition (and to which extent) into the national law(s) is decided by the relevant MS.
- **Opinion:** Non-binding; official statement issued by an EU Institution in response to a particular question, for example from one of the MSs.
- **Recommendation:** Non-binding, official response issued by an EU Institution following a particular question. This type of response allows and is most commonly used by official institutions to express their preferred point of view or action.

CT results will have to be publicly available in the CT database within one year after the end of the CT. These will include results summaries in lay language, pharmacovigilance, and protocol data, as well as details on CT application process, such as rejections, patient withdrawals, and temporary halt(s). Final study reports submitted as part of MA will need to be uploaded in the CT database within 30 days, similar to rejections or withdrawals of MAs. Sponsors will face penalties, if they fail to comply with these requirements.

Up until now, all trials conducted in any EU country had to be registered in the EU Clinical Trial Database (EudraCT¹²), and a summary of trial results had to be submitted to the EU Clinical Trial Register¹³ within 12 months of the end of the trial.

The resulting ‘transparency’ of CT results through the Regulation has largely been welcomed by the public, but remains a concern for some—such as rare disease patients—since a high level of data protection for participating subjects will need to be assured as these will be easier to identify through the public nature of the results.

Also, public access to CCI which could potentially be present in IBs, SmPCs, formulation, assay technologies, and methods for novel products submitted as part of the CTA dossier could make this information potentially prone to loss or theft of important intellectual property.

As part of the implementation of the Regulation through EMA’s policy 070^{14, 15} data for CTs, which have been approved by EMA through the Centralised Application Procedure (CAP)¹⁶ as of 2015 will also have to be retrospectively uploaded into the CT database.

TRANSITION:

Until the Regulation comes into force (depending on the functionality of the submission portal and the database)¹⁶, all CTs in the EU will be conducted in accordance with the Directive. The Directive will, however, still apply three years from the day the Regulation became effective in the following instances:

- CTs submitted before the entry into force of the Regulation; and
- CTs submitted within one year after entry into force, if the sponsor opted to have the CT assessed under the Directive.

PPTA and its member companies support the EU Clinical Trial Regulation and its aim to harmonize, streamline, and facilitate the regulatory environment for CT conduct in Europe.

We believe that the provisions set out in the Regulation will improve the submission and conduct of multinational CTs and the Regulation will help to streamline and improve administrative processes, reduce cost, and improve current best practice in set-up and management of CTs. ●

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- 11 External guidance on the implementation of the European Medicines Agency policy on the publication of clinical data for medicinal products for human use: http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2016/03/WC500202621.pdf
- 12 EU Clinical Trials Database (EudraCT) : <https://eudract.ema.europa.eu/>
- 13 EU Clinical Trials Register : <https://www.clinicaltrialsregister.eu/>
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PPTA and its member companies support the EU Clinical Trial Regulation and its aim to harmonize, streamline, and facilitate the regulatory environment for CT conduct in Europe.



EU Blood Directive: What Are the Latest Developments?

BY BRUNO SANTONI, PPTA EXECUTIVE DIRECTOR, EUROPE

Several activities are taking place within the context of the current Blood Directive 2002/98/EC and its potential revision. Indeed several stakeholders, including PPTA, are engaged in the reflection around the current functioning of the directive. This column provides an overview of the most recent developments.

It was on Jan. 21, 2016 that a delegation from the European Commission (EC) visited the Biotest AG manufacturing site in Dreieich, Germany and a CSL Plasma collection center in Frankfurt, Germany. The meeting was organized by PPTA in order to provide an opportunity for the EC to see the functioning and organization of our sector. Throughout the day, companies showcased their operations and sites and presented the challenges they face within the current regulation. A key point that was addressed was the need to develop an environment that fosters more plasmapheresis in the EU so that increasing clinical need can be met. All participants of the meeting expressed their great satisfaction with the information exchange that was made available. The reports of the meeting are published by the EC on their website.¹ Indeed, as part of their transparency goal, the EC is now publishing reports of meetings they hold

with stakeholders. This is a useful source of information that allows the industry to follow the positions expressed by the different stakeholders to the EC. It's important, though, to note that at this stage there is no formal consultation process by the EC regarding the Blood Directive. A full process of evaluation of the existing legal framework is likely to start before the end of the year. This will include public and targeted consultations and PPTA will, of course, be part of the interactions and dialogue.

PPTA is also advocating to be invited as a stakeholder at one of the next National Competent Authorities meetings that the EC (DG SANTE B4) is organizing with blood sector representatives of EU countries. Being at this meeting would allow PPTA to present and discuss key aspects of the sector directly with national representatives who are best informed on the specific needs of their country.



Delegation of EU Commission at the CSL Plasma collection center in Frankfurt, Germany.

From what we observe, there is an increased understanding by several key players that the EU needs to differently build its future related to blood and plasma if it wants to stay at the forefront of scientific and societal developments.

It is also important to note that on April 26, 2016 the EC published their report on the implementation of the Blood Directive in the EU countries and also the results of their survey on voluntary and unpaid donation practices vis-à-vis donors.² Although not all practices were identified, this third report is of great value as it describes more completely than in previous reports the different practices and compensation systems that are in place by countries in order to collect blood and blood components, including plasma.

Another development is the July 25, 2016 publication in the Official Journal of the European Union of COMMISSION DIRECTIVE (EU) 2016/1214, which amends Directive 2005/62/EC regarding quality system standards and specifications for blood establishments.³ It establishes that Member States shall ensure there are good practice guidelines available to and used by all blood establishments, which include detailed principles and guidelines for good manufacturing practices. In doing so, Member States shall take into account the Good Practice Guidelines jointly developed by the Commission and the European Directorate for the Quality of Medicines (EDQM) and Healthcare of the Council of Europe and published by the Council of Europe.⁴ The Member States have until Feb. 15, 2018 to implement these requirements.

In parallel, PPTA and the European Plasma Alliance (EPA) have been invited by EDQM to participate in their meeting of the TS093 Extended Plasma Supply Management Working Group on Sept. 20, 2016. The meeting will address topics such as self-sufficiency, safe and sustainable plasmapheresis, frequency of donation, and plasma master file. PPTA and EPA are strongly engaged in preparing the meeting and interacting with the TS093 group so that a constructive dialogue is developed for the questions that will be raised.

The current situation with regard to the Blood Directive is very dynamic. From what we observe, there is an increased understanding by several key players that the EU needs to differently build its future related to blood and plasma if it wants to stay at the forefront of scientific and societal developments. We will keep on reporting major advances related to this topic in The Source magazine. ●

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3 <http://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A32016L1214>

4 Good Practice Guidelines, included in the Guide to the preparation, use and quality assurance of blood components, Appendix to Recommendation No. R (95) 15 of the Committee of Ministers on the preparation, use and quality assurance of blood components adopted on October 12, 1995.



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The EU VUD Report and Its Impact

BY KARL PETROVSKY, PPTA SENIOR MANAGER, HEALTH POLICY

On April 26, 2016, the European Commission Directorate General Health-B4 Unit published the Commission Staff Working Document on the implementation of the principle of voluntary and unpaid donation (VUD) for human blood and blood components (the VUD Report). This VUD Report summarizes the results of a questionnaire survey on the implementation of the VUD principle, which was conducted in 2014 and sent to European Union (EU) Member States (MSs). The two preceding Reports were issued by the Commission in 2006 and 2011.

SCOPE OF THE VUD REPORT

The Report aims to map the implementation of VUD regarding blood and blood components in the EU. It mainly focuses on practices regarding donors and collectors, as well as provisions and policies related to VUD. It addresses the organization of collection and supply of blood, blood components, plasma derivatives, and how sufficient supply can be ensured through VUD.

MAIN FINDINGS AND CONCLUSIONS

With this Report, the Commission accomplished a substantial amount of in-depth ongoing work. The Report constitutes important progress compared to previous Reports as it is more complete.

The Report covers EU definitions of important terms that did not exist before, such as: compensation (with a rather positive tone), incentives (negative for DG Health, since it implies financial gain), national self-sufficiency (fulfilling needs from within a population), national sufficiency (fulfilling needs from within country and supranational cooperation), and shortage. The Report, however, expressly mentions that the definitions serve “for the purpose of this survey only.” This said, the definitions certainly reflect the thinking of the Commission and would need to be taken into consideration in future endeavors to possibly revise the EU Blood Directive.

VUD of blood and blood components is recognized in all EU MSs but differently enforced. Twenty-five EU MSs (and Norway) consider VUD as “mandatory” but mandatory is interpreted differently, sometimes as “encourage.” Seventeen EU MSs define penalties for infringement of their legislative provisions on VUD, however, none has ever been imposed.

Generally, the Report acknowledges the existence of a variety of compensation and incentive practices across the EU MSs:

- Around nine EU MSs have plans in place, referring to the strict term of “compensation,” linked to loss of earnings or inconveniences. The highest maximum values reported in the Report on compensation practices “lie between 25–30 euros (AT, CZ, DE, LV, RO).”¹
- Time off from work for public and private sector employees is offered in about half of the EU MSs; Greece and Bulgaria offer two days off, with Bulgaria offering this for employees in both the public and private sector, including for plasma donation.

However, not all of the existing practices are included, such as the tax deduction provisions for plasma donors (in the Czech Republic), which were mentioned in the previous Report and are still in place, but are not mentioned in the current Report.

Trans-border donation appears as a new item in this Report; this donation practice is confirmed by 11 EU MSs (amongst them “donor recipient” countries and “donor traveling” countries); 16 EU MSs have policies against trans-border donations. The Commission considers it as an individual practice.

Regarding the term “self-sufficiency,” the Commission set up two concepts: “national *self*-sufficiency” and “national *sufficiency*,” whereas the latter seems to be the broader concept and also includes the regional (EU) and international cooperation element to fulfill national needs. In doing so, the Commission seems to transform its concept of “community self-sufficiency” (in the recitals of Directive 2002/98/EC) into the “national *sufficiency*” concept, which includes regional and international cooperation. The newly used term of ‘sufficiency’ is an opportunity to look at sufficiency, or—over time—maybe better *availability*, as linked to regional (EU) and international cooperation outside the EU, so potentially including U.S. imports.

Donor pool aging and new epidemiological outbreaks, are cited as reasons for probable, occasional future shortages. Both elements are factors that have not been mentioned in the previous Reports nor in the Blood Directive.

The Commission is not taking a position on whether “ethics” play a role or not for VUD; the word “ethics” simply is not used.

Finally, the Commission is not drawing specific conclusions in this Report. However, from a general viewpoint, the Commission

sees the need to start an Evaluation process on the functioning of the Blood Directive 2002/98/EC.

REGARDING NEW DEFINITIONS

The Report acknowledges that Directive 2002/98 (in recital 23) takes account of the definition of the Council of Europe for what is considered voluntary and non-remunerated donation, but notes the lack of definitions of the terms compensation, incentive, sufficiency, or shortage. This is why the Commission provides the following new definitions:

- **Compensation:** reparation strictly limited to making good the expenses and inconveniences related to the donation;
- **Incentive:** inducement/stimulus for donation with a view to seeking financial gain or comparable advantage;
- **National self-sufficiency:** fulfilling the needs of human blood, blood components, and plasma derivatives for medical application of the resident population by accessing resources from within the country’s population;
- **National sufficiency:** fulfilling the needs of blood, blood components, and plasma derivatives for medical application of the resident population by accessing resources from within the country and through regional/international cooperation;
- **Shortage:** a relative deficiency in the supply with blood, blood components, and plasma derivatives for medical application, which requires creation of waiting lists or makes a certain therapy temporarily unavailable at national level.

VOLUNTARY AND UNPAID DONATIONS

Overall, the Commission admits that these practices vary from one EU MS to another and there may also be different practices within a single country. The following types of practices are extracted from a figure in the Report, which the Commission identifies in three categories:

CATEGORY ONE

- Refreshments
- Food voucher(s)
- Small tokens, such as pins, pens, towels, t-shirts, and mugs
- Free physical check-up (beyond what is required for the donations)
- Free or reimbursement of medical costs (e.g. additional medication, etc.)
- Reimbursement of costs linked to travel (to and from place of donation)

CATEGORY TWO

- Time off work - public sector
- Time off work - private sector

CATEGORY THREE

- Compensation linked to loss of earnings
- Compensation for the inconveniences related to donation
- Fixed sum of money, irrespective of actual costs, established at the national level
- Fixed sum of money, irrespective of actual costs, established by individual blood establishments

THE REPORT'S LANGUAGE ON MAXIMUM VALUE PER DONATION

Figure 1 of the Report mentions a maximum value in euros for donation¹ regarding all practices vis-a-vis donors except for category two “Time off work - public and private sector”; the only criterion mentioned for this category is days/donation.”² For category one: The “reported maximum values of refreshments and small tokens range between 1–10 euros, whereas for food vouchers (in six EU MSs) the value ranges between 1.4 euros (Latvia) and 15 euros (Romania)”. For category three: the “VUD Report mentions as “maximum values reported per donation the range between EUR 25–30 euros.”³ In Bulgaria and Czech Republic, the maximum values are defined as a percentage of the national minimum wage.”⁴ Overall, the report states that “the reported monetary reimbursement or compensation of more than one type should not be added.”⁵ Almost half of the EU MSs appear to have guiding principles regarding compensation to donors. However, overlaps exist between categories one and three since both categories imply financial elements, and obviously, category two also represents a financial value.

SELF-SUFFICIENCY

Almost all EU MSs (except Austria, Cyprus, and Finland) and Norway are reported to have policies in place to

achieve self-sufficiency and/or sufficiency of blood and blood derivatives. In order to achieve (self-) sufficiency, EU MS policies aim either at increasing the supply via VUD, or through export restrictions. It is worthwhile to note that, according to the Report, only four EU MSs and Norway launched projects to increase apheresis donation.

SUMMARY

The Report constitutes an important progress compared to previous Reports, as it is much more complete and covers EU definitions of important terms, which did not exist before. These definitions would need to be taken into account in forthcoming processes to improve the legal framework around plasma collection. Generally, the Report acknowledges the existence of a variety of compensation and incentive practices across the EU MSs. Compensation is seen by the Commission as rather positive, whilst incentives are rated as negative, since they imply, for the Commission, financial gain.

Finally, the Commission is not drawing specific conclusions in this Report. However, from a general viewpoint, the Commission sees the need to start an evaluation process on the whole functioning of EU Blood Directive 2002/98. PPTA is ready to participate in such evaluation consultation as one of the key stakeholders. ●

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2 Ibid. page 10.

3 Ibid. page 10.

4 Ibid. page 8.





Ensuring Patient Access to Orphan Medicines in Europe: Current Issues and Trends

BY IRINA ODNOLETKOVA, PPTA DIRECTOR HEALTH ECONOMICS & OUTCOMES

The technological advances built upon progressive understanding of the human genome and cell biology have led to a growing number of new therapies. In the past five years, about a third of applications for marketing authorization received by the European Medicines Agency (EMA), were for biotech medicines, including recombinant DNA, fusion proteins, and monoclonal antibodies.¹ Gene therapies are emerging and have proven effective in treatment of severe combined immunodeficiency and show promise in treatment of hemophilia B.

Despite the scientific progress, developing medicines to treat rare diseases represents a particular challenge, as their low prevalence—no more than 5 in 10,000 people in the European Union (EU)—contributes to an uncertainty with regard to the return on investment. In 2000, the Orphan Medicinal Product Regulation was introduced in the EU, with the purpose of stimulating therapeutic innovation in rare diseases, allowing marketing authorization applicants to receive clinical trial protocol assistance, fee waivers for the regulatory procedures, and a 10-year market exclusivity.

To date, 126 orphan medicines have received EMA approval. However, the unmet medical need is still high. For the more than 7,000 known orphan diseases affecting between 30 and 40 million people in the EU, only about one percent are currently addressed with an adequate treatment. With an annual cost of 150,000 euros (\$167,000) per patient on average and about 1,620 orphan medicines estimated to be in development,² decisions on the allocation of intrinsically limited national budgets across existing and emerging therapies are becoming increasingly difficult. The Healthcare Ministries (further referred to as ‘Payers’) have been ringing alarm bells on the high prices of medicines and there is a growing concern about the sustainability of healthcare systems in Europe.

A typical response by Payers to an increasing number of expensive medicinal products has been price cuts, compulsory rebates, and increased patient co-payments. Whether this approach can ensure patient access and stimulate biomedical research in the long term is questionable. It has been acknowledged that, if left unchanged, this approach may lead

A European marketing authorization does not guarantee reimbursement at the national level. Twenty-seven national decisions on the therapeutic added value and price of each drug need to be taken within the EU.



to a serious lose-lose situation: reduced opportunities for innovative therapies, drug shortages, loss of political capital for governmental decision-makers, and underserved patients.³

NEW APPROACHES TO EVIDENCE GENERATION

In order to understand whether health technologies are worth their price, the costs associated with their use have to be weighed against the respective health gains. Traditionally, the efficacy of a drug has been analyzed by means of frequentist statistics, i.e. a comparison of between-group difference of statistical means observed within a randomized controlled trial (RCT), powered based on an ‘a priori’ assumed effect. A consensus has been growing, however, that this approach may neglect the variability in patient response resulting from the biological complexity and variation of the human phenotype. Particularly in rare diseases, where adequate enrollment numbers are often unfeasible, and the mechanism of action of a drug not entirely understood, compulsory RCTs may slow the scientific progress. The need for a methodological shift to adaptive trial designs, which allow for iterative evidence generation and a timely recognition of the drug efficacy in certain subgroups has been recognized.⁴ Innovative regulatory concepts have been put forward by EMA, such as adaptive pathways and conditional marketing authorization, with mandatory post-licensing data collection accompanied by an appropriate risk management plan. The purpose of these currently piloted methods is to facilitate quicker patient access to new therapies addressing high medical need while reducing time and costs associated with large clinical trials. To ensure such post-authorization real-life evidence generation development and routine use of uniformly designed European patient registries seems essential.

EUROPEAN COOPERATION ON HEALTH TECHNOLOGY ASSESSMENT

A European marketing authorization does not guarantee reimbursement at the national level. Twenty-seven national decisions on the therapeutic added value and price of each drug need to be taken within the EU. A scientific assessment of the added therapeutic value of new medicines compared to the best available alternative and in relation to the associated costs is the purpose of Health Technology Assessments (HTAs).

So far, HTAs have only been partially successful in increasing the transparency of the reimbursement decisions. Due to many methodological differences of the national HTAs,⁵ a concordance on the therapeutic value of medicines in the EU seems to exist for no more than 50 percent of the new drugs.⁶ These differences in scientific judgment are difficult to explain to patients who are evolving toward an assertive, well-informed stakeholder within the healthcare sector and wonder why access to often life-saving treatments is not equal across the EU countries.⁷ Differences in the countries’ purchasing power represents an additional challenge. The rapid expansion of the EU, following the post-Soviet application of the East European countries, has been one of the EU’s most successful foreign policies, but has created economic inequalities within the EU. The cross-border-care directive (2011/24/EU) that assumes similarity of the healthcare provision, promotes work toward reducing the gap in healthcare quality within Europe.

In past years, these issues have been addressed through different initiatives at the scientific and policy levels. The European Commission has put considerable effort into the “European Network for Health Technology Assessment” (EUnetHTA) project, a voluntary network of the national HTA

bodies, aimed at joint assessment of health technologies, with the purpose of avoiding duplication and increasing transparency and outcome consensus of the scientific work, particularly with regard to the relative clinical benefit of new treatments. After developing common methodologies and piloting joint HTAs during the previous Joint Actions (2010–2015), the newly begun “Joint Action 3” of EUnetHTA aims to increase the number of joint assessments and establish a permanent mechanism for European cooperation on HTAs by 2020.

Since 2010, the EMA and EUnetHTA have cooperated on several topics related to the evaluation of medicines, e.g., design of adaptive pathways and an early alignment of the evidentiary requirements between the regulatory and HTA bodies through parallel scientific advice. Such cooperation has potential to facilitate efficient and adequate data collection to support the companies’ reimbursement requests and accelerate patient access to effective treatments. Further success of the European HTA cooperation will be measured by the extent to which different Member States will be willing to integrate the results of joint assessments into their reimbursement decisions. In the meantime, BeNeLux (a union of Belgium, the Netherlands, and Luxembourg), joined by Austria, is pioneering a proprietary cooperation on joint horizon scanning, HTAs, and principles of price negotiations on orphan drugs. Ten Eastern European countries with lower but comparable gross domestic product levels, have announced plans for similar cooperation. The role of the EC as a coordinating party has yet to be clarified and fits in the general debate on the purpose and scope of the joint activities within the EU, notably provoked by the recent decision by the United Kingdom to leave the EU (Brexit).

CITIZEN ENGAGEMENT IN THE REIMBURSEMENT DECISIONS

Citizens’ role in the reimbursement decisions is still very limited, due to a historically paternalistic functioning of the healthcare systems on one hand, and a specialized nature of such decisions on the other. Difficult ethical choices are often being made by the Payers without sufficient insights into the societal “willingness to pay”. In order to justify the reimbursement decisions within the publicly financed European healthcare systems, citizen engagement in policy-making seems crucial. Methods to structurally elicit the population’s views on reimbursement priorities are under exploration and may support decisions on the affordability of therapies to treat rare diseases.^{8,9}

As discussed above, the primary concern of the policy-makers at the moment seems to be the budgetary impact of new therapies. Among the emerging ‘managed entry schemes’ for expensive medicines, for instance, the price-volume contracts clearly outnumber the pay-for-performance agreements. Linking prices of medicines to their value would create a rational benchmark for budget allocation and increase transparency in the reimbursement decisions. Further conceptualization of ‘value’ will require more active patient involvement in the development and evaluation of health technologies and will enhance our understanding of the outcomes that are truly relevant. ●

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Emergent Biosolutions: Track Record of Growth and Innovation

Since its founding in 1998, Emergent BioSolutions (NYSE:EBS) has established itself as a specialty biopharmaceutical company with a simple mission – to protect and enhance life. The company, headquartered in Maryland and with manufacturing and product development sites in North America and an international presence, focuses on delivering medical countermeasures for civilian and military populations that address intentional, accidental, and naturally emerging public health threats and infectious diseases.

Throughout its 18-year history, Emergent has successfully grown organically and through strategic acquisitions. Within the last three years, when Emergent completed the acquisition of Cangene Corporation, a successful Canadian biotech, and RSDL, a chemical decontamination product widely used by the U.S. military, the company expanded its portfolio of preparedness solutions for governments from one product, BioThrax®, the only anthrax vaccine licensed by the U.S. Food and Drug Administration (FDA), to five products (vaccines, therapeutics, and devices) that address chemical and biological threats. With the Cangene acquisition, Emergent also expanded its manufacturing infrastructure with a fill-finish facility located in Baltimore and a hyperimmune-focused facility in Winnipeg, Manitoba, Canada.

Aside from its flagship product, BioThrax, Emergent has several FDA-approved human and equine plasma fractionated products for targeted therapy of specific indications. Products manufactured from human plasma

include purified gamma globulin containing polyclonal antibodies to specific antigen(s) obtained from fractionation of human plasma. These products are marketed as Anthrasil™, which is used for treating toxemia associated with inhalational anthrax in adult and pediatric patients in combination with appropriate antibacterial drugs; and Vaccinia Immune Globulin IV (VIGIV), an immune globulin indicated for the treatment of complications due to smallpox vaccination. The Centers for Disease Control and Prevention recently exercised a contract option for Emergent to conduct manufacturing runs and activities to maintain FDA-licensure of VIGIV.

The equine product line is comprised of purified immune globulin fragments derived from polyclonal antibodies to specific antigen(s) obtained from fractionation of equine plasma. The core product developed on this platform is BAT®, the only FDA-licensed heptavalent antitoxin indicated for the treatment of symptomatic botulism following documented or suspected exposure to botulinum neurotoxin serotypes A, B, C, D, E, F, or G in adult and pediatric patients.

“Our mission at Emergent is to protect and enhance life. That single-minded focus is at the heart of everything we do. It is why we pursue innovative ways to develop and create safe, effective products that save lives every day, across the globe,” said Mark Lobe, vice president and general manager of Emergent’s Winnipeg Operations. “As the U.S. license

holder and manufacturer of Anthrasil™ and VIGIV, both human plasma-derived hyperimmune products, we are pleased to be a member of the Plasma Protein Therapeutics Association and to work with others in this industry who are equally committed to protecting lives.”

Manufacturing is a core competence of Emergent, a clear example of which is that the company was designated by the U.S. Department of Health and Human Services (HHS) as one of three Centers for Innovation in Advanced Development and Manufacturing (CIADM) in the nation. A public-private partnership with the Biomedical Advanced Research and Development Authority, the CIADM helps to accelerate the development and manufacture of medical countermeasures, such as vaccines and therapeutics used to protect the public in emergencies, and which can transition quickly and cost effectively between products as threats emerge.

Emergent's CIADM, located in Baltimore, Md., has been tapped recently by HHS to develop and manufacture a vaccine candidate for the Zika virus. The candidate will be used in a Phase 1 clinical trial slated to potentially begin early next year. Additionally, the facility is currently manufacturing monoclonal antibody therapeutics for Ebola under a task order by the U.S. government. It previously completed the manufacture of an Ebola vaccine that was used in a Phase 1 clinical trial last year.

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As a long-standing partner of the U.S. government, having proven the company's reliability in developing, manufacturing and delivering medical countermeasures to the Strategic National Stockpile, Emergent has its eyes set on continuing not only its support of the U.S. government's and allied governments' biosecurity and preparedness needs, but also its growth trajectory and successful history of strategic acquisitions.

With a portfolio that spans vaccines, antibody therapeutics and medical devices for biological and chemical threats as well as emerging infectious diseases, Emergent is working towards its vision of protecting and enhancing 50 million lives by 2025. ●



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Alpha-1 Foundation's New President and CEO— Henry Moehring

BY BOB CAMPBELL, COMMUNICATIONS DIRECTOR, ALPHA-1 FOUNDATION

When Henry Moehring became the Alpha-1 Foundation's president and CEO in late April, he had a long history of volunteer involvement with the Alpha-1 community.

He had been vice chairman of the Foundation Board since 2014, when the Alpha-1 Association merged into the Foundation. He had been an Association Board member since 2010, and chaired the Board before the merger. He also served on the Integration Task Force that brought the two organizations together.

Moehring was diagnosed with Alpha-1 antitrypsin deficiency in 1997, because of elevated liver enzymes in two consecutive routine physicals. Still an exceptionally healthy Alpha, he sees a liver and lung specialist and uses inhalers daily to help manage his disease.

"It has always been a tenet of mine that whatever I do professionally has to have an element of giving back," he says. "I got that attitude from both my parents. We were always active in church, and I still am. It's part of who we are. I've worked with for-profit and not-for-profit organizations, but whatever I do, there's always some element of giving back to society."

Moehring, 57, has a master of business administration degree from Johns Hopkins University and has been a healthcare administration professional for 30 years. He was executive director of Asbury Methodist Village, a nonprofit retirement community in Gaithersburg, MD, with more than 1,400 residents and 840 employees, before resigning in April to lead the Alpha-1 Foundation.

Well before that, he had discussed the change with his wife, Mary Louise, and their son Matthew.

"John Walsh (Alpha-1 Foundation co-founder and CEO for two decades) and I had been talking, just between the two of us, for a few years about his desire to step aside as president and CEO of the Foundation," Moehring says. "One of John's great abilities is, he's a visionary, and that was where he wanted to spend his energy. My own skill set is, I'm entrepreneurial, but I also have the nuts and bolts of management experience as well."



“Any organization has opportunities. There are areas that we can look at together and say, is there another way we can approach this? Where are the areas that we would like to change? Change is always hard; even small change is impactful. How do you manage that change so it is truly an improvement, and not just change for change’s sake?”

They came up with a specific plan in 2015, and took it to the executive committee of the Foundation Board at its October 2015 meeting.

Walsh, the Foundation’s co-founder, president and CEO, proposed that Moehring become the President and CEO, while Walsh stepped aside to the titles of Founder and Chief Visionary Officer. The Board unanimously accepted the proposal at the same meeting.

“John and I had a plan,” Moehring says. “There was going to be a gradual transition, I was going to be connected with John, we were going to go everywhere together, and I was

going to springboard off those relationships.” Then Walsh was severely injured in a fall on an icy street in Washington, D.C. on Jan. 20, and continues to undergo rehabilitation. “When John was injured, that plan went out the window,” Moehring says.

The Foundation reacted quickly, appointing Immediate Past Board Chairman Ab Rees as acting president and CEO, and promoting its two longtime vice presidents. Chief Operating Officer Marcia Ritchie and Chief Financial Officer Robert Barrett both became executive vice presidents. “The transition committee went to work to accelerate Henry’s move to become our new President and CEO,” said Board Chair Gordon Cadwgan.

“Fortunately,” says Moehring, “the Foundation is in a very solid position. It has amazing volunteer leadership in many areas, including the board of directors. We have a clear mission and a dedicated staff that get the job done well. All of those things lend themselves to success. Many times you come into an organization and you have to fix something before you can move forward. There’s nothing here that needs to be fixed.”

His thoughts on the future of the Foundation: “I’m an inclusive leader. My initial strategy was to learn the organization and to develop relationships, so I have met one-on-one with all our staffers. I’m fortunate that things are going well at the Foundation, so I had time to do that.

“Any organization has opportunities. There are areas that we can look at together and say, is there another way we can approach this? Where are the areas that we would like to change? Change is always hard; even small change is impactful. How do you manage that change so it is truly an improvement, and not just change for change’s sake?”

In an interview shortly before the National Alpha-1 Education Conference in June, Moehring talked about his own priorities.

“...John was a master at building relationships and he’s had more than 20 years to do this; I’ve been on the job since April 29. So these are my two personal priorities: Connecting much more closely with the community and with our partners. I need to focus on building my own connections.”

He loved the energy he felt from people at the Alpha-1 national conference in Miami, Fla.

“I was talking to an Alpha pretty much every 10 minutes that I was awake—just two people talking, no agenda, but I could hear their perspective, hear their concerns. I walked away with a lot of valuable information. Overall, I felt the conference was an amazing success. People left, as they always do, feeling charged up. The conference affirmed the energy of the community. I think John would be proud.” ●

Inside PPTA



Top: MP Maria Michalk (CDU) visiting the plasma collection center of Haema AG in Dresden, Germany.

Right: MP Dr. Georg Kippels (CDU) with a donor and Carola Lipps (Center Director) in the plasma collection center of Plasma Service Europe in Cologne, Germany.



● Members of the German Parliament Visit a Plasma Collection Center

Over the course of this year, PPTA invited several Members of the German Parliament to a plasma collection center located in their respective constituencies.

MP Maria Michalk (CDU), Health Policy Spokeswoman of the CDU/CSU Parliamentary Group and Member of the Health Committee, visited Haema in Dresden; MP Fritz Güntzler (CDU), Member of the Finance Committee and rapporteur for value-added tax (VAT), visited CSL Plasma in Göttingen; MP Andreas Schwarz (SPD), Member of the Finance Committee and rapporteur for VAT, visited KEDPLASMA in Bayreuth; and MP Georg Kippels (CDU), Member of the Health Committee, visited a plasma collection center of Plasma Service Europe in Cologne.

The overarching purpose of these meetings was to familiarize important policy stakeholders with the specifics of plasma collection, the medicinal therapies that are made from plasma,

and the differences between blood and blood components for transfusion and plasma for manufacturing—as well as to emphasize the relevance of every single plasma donation collected in Europe. Patients with rare, chronic diseases, such as primary immunodeficiency and hemophilia, rely on plasma protein therapies to save and improve their lives.

Depending on the specialty field of the invited stakeholders, additional topics were addressed such as the non-harmonized value-added tax (VAT) application to plasma deliveries for industrial processing, how to raise awareness for plasma donation, and activities to thank donors for their valuable contributions. ●

ALEXA WETZEL, PPTA Senior Manager, Source Europe
SÁNDOR VON TOTH, PPTA Senior Manager, Germany

● Highlights from the 2016 Plasma Protein Forum in Washington, D.C.

Dr. Shari Ling, Deputy Chief Medical Officer, Centers for Medicare and Medicaid Services (CMS), delivered the keynote address opening the 2016 Plasma Protein Forum. Dr. Ling's remarks highlighted CMS's movement toward value-based purchasing and quality-based payment programs. Dr. Ling noted that CMS will utilize alternative payment models and will link fee-for-service payments to quality or value as it moves toward these goals. She also outlined the role that the CMS Innovation Center will play in developing, testing, and implementing new payment and service delivery models.

The Plasma Protein Forum in Washington, D.C. had more than 300 attendees from industry, government, and patients reflecting a robust agenda that covered issues that were timely and relevant to all stakeholders. Topics included pathogen safety, patient access to care, and traceability of plasma protein therapies.

The Chairman of the PPTA Global Board of Directors, Mr. David Bell, delivered the Chairman's Message. Mr. Bell emphasized the importance of and dedication to safety and quality standards for plasma collection and plasma protein therapies, noting that, "We require an absolute zero failure rate." His call to action was to continue the industry's focus on quality, supply, access, and best in class safety.

Professor Dr. Herold J. Metselaar delivered a talk on the role of the liver in plasma protein deficiencies. Professor Metselaar used the story of one patient with hemophilia B with hepatitis C virus (HCV) infection and showed how plasma protein therapies, HCV treatment, and a liver transplant resulted in a total cure. He then shared insights on the future of liver transplants.

The panel, "Traceability under the Drug Supply Chain Security Act," moderated by PPTA Senior Manager, Regulatory Policy, Mary Clare Kimber, examined how plasma protein therapies are traced through the entire supply chain. The panel covered everything from law and regulatory issues to the design of enterprise resource planning systems, to wholesale distribution, to the specifics of packaging requirements.

Dr. Thomas Kreil presented the industry experience and evolution in pathogen safety issues. Though product safety is



Left: David Bell, Executive Vice President and General Counsel, Grifols Inc. and Chairman of the PPTA Global Board of Directors.

Right: Professor Dr. Herold J. Metselaar, Erasmus University Hospital, Netherlands.

as high as it has ever been, the pathogen safety experts remain focused on new developments to ensure that patients will get safe therapies.

Mr. Jan M. Bult, PPTA President & CEO, presented Mr. Joe Rosen with a special industry recognition award for his four decades of dedication to the industry. In his remarks, Mr. Rosen asked that we remember that safety is central to this business, respect your regulators, appreciate your donors, keep pursuing access to all markets, update the International Quality Plasma Program and Quality Standards of Excellence, Assurance, and Leadership, be vigilant for emerging viruses, continue to invest in R&D, and, finally, respect one another.

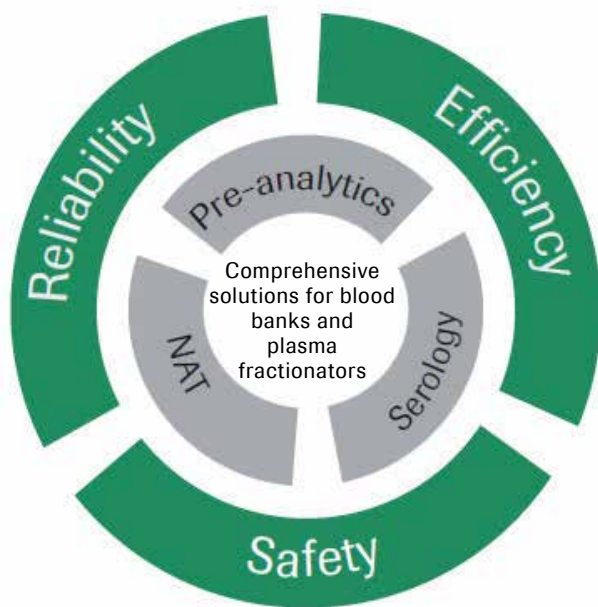
The panel, "Patient Access: The A-PLUS Perspective," moderated by PPTA Senior Director, State Affairs, Bill Speir, examined key issues from the patient point of view. The panelists from a number of different organizations talked about newborn screening for severe combined immunodeficiency and efforts to get all 50 states to screen newborns as well as several issues that could have a negative impact on patient access to plasma protein therapies, including preferred drug lists, federal regulation on patient premium assistance, and the proposed Medicare Part B Prescription Drug Model rule change. Also discussed were Durable Medical Equipment competitive bidding, specialty drug tiers, questions around biosimilars, and a change to the Medicaid pharmacy reimbursement methodology.

continued on page 29 »



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» continued from page 27

Panelists noted that a common issue is that the patient voice is not being considered in these matters.

The Chairman of the PPTA Source Board of Directors, Mr. Shinji Wada, provided an update on the state of the source plasma industry. Mr. Wada spoke to the unique environment in which the source plasma industry operates. He stated that PPTA's Source priorities are, "donor health and safety, enhancement of the IQPP standards, increasing industry transparency and public awareness of the industry, increasing awareness of donor contributions, expanding and solidifying European activities, and continuing to assure the availability of high quality plasma."

The "Evolving Technologies in Plasma Collection" panel addressed opportunities presented by the development and application of new technologies including the National Donor Deferral Registry, the Cross Donation Check System, donor health management systems, and the use of social media.

Mr. John Delacourt, PPTA Vice President, Legal Affairs & Global Operations, moderated the "International Dynamics" panel. This panel examined the complicated environment faced by the plasma protein industry in both China and Canada, as well as provided an update on Transatlantic Trade and Investment Partnership (TTIP) negotiations. The discussion on China noted challenges, such as Article 49, that prohibits the importation of plasma or plasma products, a lack of accurate knowledge about Chinese rare diseases and patients, uneven health care infrastructure, and political barriers to advocacy. Also presented was an overview of the TTIP with a focus on the potential opportunities for the plasma protein industry including mutual recognition of inspections, regulatory convergence, and global sufficiency for source plasma. Finally, challenges faced by the source plasma collection industry in Canada were examined, specifically the issue of compensated plasma donation. The robust and effective safety initiatives in place were noted, as was the impeccable safety record of the industry for the last few decades. The industry is working to counter inaccurate information and bring the debate back to facts to ensure access to safe and effective therapies for Canadians. ●

The robust and effective safety initiatives in place were noted, as was the impeccable safety record of the industry for the last few decades.



Chairman of the PPTA Global Board of Directors, David Bell and PPTA President & CEO, Jan M. Bult presented the 2016 Dr. Otto Schwarz award to Dr. Thomas R. Kreil.

Dr. Thomas R. Kreil Honored with 2016 Dr. Otto Schwarz Award

Since 2012, the Dr. Otto Schwarz Award has recognized leadership in the plasma protein therapeutics industry and related scientific fields. Chairman of the PPTA Global Board of Directors, Mr. David Bell and PPTA President & CEO, Jan M. Bult presented the 2016 Dr. Otto Schwarz Award to Dr. Thomas R. Kreil.

Dr. Kreil, who is the Senior Director Global Pathogen Safety for Baxalta, Inc. (now part of Shire), was honored for his work in pathogen safety and ensuring that plasma protein therapies are safe for the patients who need them.

The Dr. Otto Schwarz Award was created in honor of Dr. Schwarz, one of the founders of the International Plasma Products Industry Association (IPPIA), the association representing the manufacturers of plasma protein therapies, and the forerunner of PPTA. As one of the first chairs of IPPIA, he recognized the importance of developing an industry view and was the first to recognize the importance of qualified donors and how to best apply nucleic acid test. PPTA is pleased to recognize Dr. Kreil's legacy through the Dr. Otto Schwarz Award. ●

DR. OTTO SCHWARZ AWARD RECIPIENTS

2016	Dr. Thomas R. Kreil	2013	Larry Guiheen
2015	Dr. Herbert Dichtelmüller	2012	Dr. Steve Pettaway
		1998	Jack Ryan
2014	Professor Reinhard Burger	1996	Knut Hansen

● PPTA Sits Down with Joe Rosen to Talk Past, Present, and Future of the Industry

BY WILLIAM MURRAY, PPTA DIRECTOR,
GLOBAL COMMUNICATIONS

Joe Rosen has four decades of experience in the plasma protein industry. He has built and run companies that collect plasma, helped to create the Quality Plasma Program (QPP)—now the International Quality Plasma Program (IQPP)—and served as chairman of the PPTA Source Board of Directors to name just a few accomplishments. We caught up with him recently to talk about the state of the plasma protein industry and its future.

Q How did you start in the industry?

In late 1968, with some partners, I opened a plasma center in New Brunswick, New Jersey. We collected plasma specifically for diagnostic use, mainly as raw material for blood typing serum. It was a small center with two donor beds. We expanded, moved locations, and started collecting bulk plasma for fractionation, in addition to plasma for diagnostic use. We sold the company with one center in 1971 to the Rite Aid Corporation. With new financing, my partners and I began to open up centers, primarily on college campuses. Over the years we expanded to non-college campus locations. Now, I have a company—Plasma Consultants, LLC. We match up buyers and sellers of plasma and plasma derivatives primarily, and negotiate the contracts leaving them to contract with each other. It is something like a matchmaker and we are successful because of our good reputation.

Q What do you view as your proudest accomplishments?

With the Association [Plasma Protein Therapeutics Association], I am proudest of starting, along with others, the Quality Plasma Program (QPP). In the beginning it was just in the U.S. We had to convince people that the industry should self-regulate and put into place standards beyond U.S. Food and Drug Administration requirements to improve the quality of our operations on a voluntary basis. This was a shock to many in the industry at first but eventually we were able to convince first the PPTA Source Board of Directors and then members of the value of this initiative. This helped to show that this was a reputable industry with good leadership that wanted to operate at the highest level of quality and safety.

On a personal level, my greatest accomplishment has been building a company from one plasma center with one



employee to 80 centers with more than 1600 employees. And, while doing this, maintaining a quality operation with a good reputation.

Q What are your views on the debate around compensated plasma donation?

We've documented over the years that compensating donors for donating plasma is not connected to issues of donor health, safety, or quality. In addition, when considering the ethics of compensation, I believe it is unethical not to compensate donors for the time they take to donate. Of course we greatly appreciate those donors who volunteer without compensation to donate blood. All that said, there needs to be an environment with a strong commitment to safety and quality by the industry, such as the International Quality Plasma Program (IQPP) and the Quality Standards of Excellence, Assurance, and Leadership (QSEAL), and a strong regulatory environment such as exists in the United States and the European Union.

Q What do you see as the biggest ongoing challenges?

One is to be able to collect enough plasma to meet the need for plasma protein therapies. We need to be able to expand collection while maintaining quality and safety. We also need to maintain and review IQPP to ensure it stays up to



date as technology and understanding of plasma and plasma therapies advances.

We must be vigilant about emerging pathogens to ensure the safety and quality of plasma protein therapies as well as the health of our donors.

Free access to markets around the world should be something that is constantly worked on to ensure that those who need plasma therapies around the world have access to them.

Finally, it is important to maintain good relationships with regulators. Industry should continue to develop good relationships with regulators through cooperation and transparency. This helps to show that industry is not an adversary—we all want what is best. The Association has been very effective in this role.

Q How has the public profile of the industry changed over the years?

In the early years, the industry rarely took a public position – it was a quiet industry. In the past decade or so, the industry has moved into the public eye through publishing studies and papers in peer-reviewed journals, through International Plasma Awareness Week, and other activities. The image has improved through these efforts and active engagement with stakeholders and will continue to improve. But this remains true only as long as quality and safety remain at the forefront through programs like IQPP and QSEAL.

Q How do you see PPTA's relationships with patient groups?

We've established very good relationships between the Association and patient groups. Not only do we want their

support but want to be able to demonstrate the industry's commitment to quality and access to plasma therapies. These relationships have been built over many years and speaks to the industry's commitment and credibility.

Q What is your primary area of concern with respect to the industry?

I'm always worried about emerging or new pathogens that might impact the quality of therapies and ensuring there is a strategy to address any potential new threats. I also remain concerned about maintaining access to therapies for patients.

Q What do you see as the primary areas for growth and innovation?

There is a lot of room for innovation around donors. We can improve our understanding of what motivates donors and improve our efforts to thank donors.

We have a big responsibility to maintain the health of our donors and should continue to look for ways to ensure this happens. We must also always strive to improve our viral marker rates.

As for innovation, continuing research into other products that can come from plasma.

Finally, in the long run, improving access to therapies around the world for all who need them is both an area for growth and for innovation. There are whole geographies that don't have access to therapies. This needs to change.

Q Do you have any final thoughts?

Never forget it all starts with a donor donating plasma—that's where the business is. There is no therapy until you collect the first liter of plasma. ●

Legislation in Nebraska: A Win for the Plasma Industry

BY BILL SPEIR, PPTA SENIOR DIRECTOR, STATE AFFAIRS

PPTA staff worked with the members of the PPTA State Affairs Steering Committee and the PPTA Source Board of Directors to pass legislation in Nebraska that would allow 18-year-olds to donate plasma in the state. Nebraska has a statute that sets the age of majority at 19 but allows 18-year-olds to contract. Member companies requested that PPTA pass legislation that allows 18-year-olds to donate plasma in the state.

Senator Mark Kolterman filed Legislative Bill 813, which states: "Any individual of sound mind and eighteen years of age or more may consent to donate plasma without the permission

of a parent or guardian. The consent is not subject to later disaffirmance because of minority."

Senator Kolterman's bill was heard in the Health and Human Services Committee. Senator Kolterman, Octapharma staff and PPTA staff testified in support of the bill. There was no opposition testimony. The bill passed the Committee and Nebraska Legislature. The Governor signed the bill on April 6, 2016.

Because of Senator Kolterman's legislation, plasma donation centers in Nebraska may now accept donations from 18-year-olds. It is hoped that this will lead to more qualified donors for plasma protein therapies. ●

● 2016 Capitol Hill Fly-In Brings Access Issues to Forefront

PPTA held another successful annual “Day on the Hill” on May 12 as PPTA member companies, allied patient groups, and PPTA staff visited United States Senate and House of Representatives offices. PPTA staff, serving as leaders of eight teams, lead discussions with Senators, House members, and their staff on a variety of issues important to PPTA membership and stakeholders. Access issues took the forefront as the teams joined with over 316 organizations to explain how the proposed Medicare Part B rule would limit patient access to life-saving drugs. The teams also spoke about patient-sponsored legislation such as the “Patients’ Access to Treatments Act of 2015” and asked for congressional support to pass this effort to eliminate discriminatory insurance practices.

This year, PPTA held 48 meetings in seven hours with—for the first time—as many Senate offices as House offices. Additionally, meetings were held for the first time with the separate staff

of the Energy & Commerce and Ways & Means Committees, which have jurisdiction of health-related issues. Each team distributed and discussed material on separate areas of concern to our membership and built foundations for continued dialogue during this next congressional term. Questions were often raised by congressional staff that the teams brought back to PPTA for follow-up meetings and discussions. ●

MARK YOUR CALENDARS!

The fourth annual International Plasma Awareness Week will be held Oct. 9–15. Find out how to get involved by visiting: www.donatingplasma.org

In Memoriam: Dr. Richard M. Lewis

With great sadness, PPTA reports the death of Dr. Richard M. Lewis on July 11, 2016. For the past five years, Dr. Lewis served as an auditor for PPTA’s Quality Standards of Excellence, Assurance and Leadership (QSEAL) program. He was also a valued resource and friend of PPTA. Dr. Lewis had a wealth of knowledge and experience in the plasma protein therapies and regulatory fields, as well as an infectious smile and engaging sense of humor.

During his career, Dr. Lewis was a civilian chemist with the United States Army Research Institute of Infectious Diseases and served at the U.S. Food and Drug Administration (FDA/CBER) for many years, culminating as the Deputy Director of the Office of Blood Research and Review. He most recently was the Chief Executive Officer of Access BIO, which he operated with his wife of 40 years, Dr. Joy Cavagnaro. He is survived by his wife, three daughters and five grandchildren. ●



MEET THE

PPTA Staff

William Murray

DIRECTOR GLOBAL COMMUNICATIONS

Q How long have you been with PPTA?

I started with PPTA in October 2015.

Q What do you focus on in your role as the Director for Global Communications?

Most simply put, my role is to, with my team, build and raise awareness of the plasma protein therapeutics industry and PPTA where there is a need. I work closely with the Global Communications Steering Committee, the Source Industry Profile Committee, and my colleagues at PPTA to determine communications needs and then design and execute communication efforts and campaigns, ensuring that all we do helps to advance the industry and the goals of the Association. The communications portfolio includes International Plasma Awareness Week, The Source magazine, PPTA's websites, and mobile apps to name a few.

Q Tell us about your background.

Prior to joining PPTA, I spent the last 15 years as a consultant to various parts of the Department of Defense working on strategic communications. I had the honor of working with a great number of dedicated professionals in and out of uniform. As a consultant I never stayed very long with any one client – my job was to quickly assess their communications needs, set up an effective, sustainable program that met their needs, and then move on. While it was always hard to leave behind colleagues, the upshot was that I worked with a number of different organizations in the Department of Defense in areas as diverse as space, missile defense, occupational safety, and religious support. The other benefit to having such diverse clients was that I was able to gain experience in just about every aspect of communications including research, planning, media analysis, social media, website design and management, and message development.



Q What is your proudest professional achievement?

I think my work supporting the Army's Warrior Transition Command, which served as the support and an advocate for wounded, injured, and ill soldiers has been some of my most rewarding work to date. Specifically, I was part of a small communications team supporting Army athletes for the first Warrior Games, the Department of Defense's equivalent of the Paralympics. We provided them the support they needed to tell their incredible stories to the world.

Q What is most rewarding about working in this industry?

One of the must-haves for any job I have is that my work benefit people in a tangible way. In my short time with PPTA, I've already seen the impact the Association and its communications efforts have, especially for those who need plasma protein therapies. It is incredibly rewarding to know that I am part of a team that makes a difference every day.

Q Tell us something that not many people know about you.

I played the clarinet all through middle school, high school, and much of college. I am a proud "band geek" and played in marching bands, wind ensembles, and solo. ●

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Upcoming Events

CONFERENCES & SYMPOSIUMS

September

- 21 – 24** 17th Biennial Meeting of the European Society of Immunodeficiencies (ESID) together with the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and the International Nursing Group for Immunodeficiencies (INGID)
Barcelona, SPAIN
- 23 – 24** Guillain-Barré Syndrome (GBS)/Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) Foundation International Symposium 2016
San Antonio, Texas, U.S.

October

- 3 – 5** 6th International Conference on Hematology
Orlando, Fla., U.S.
- 7 – 9** European Haemophilia Consortium (EHC) Annual Conference
Stavanger, NORWAY
- 9 – 15** International Plasma Awareness Week (IPAW)
- 17 – 18** NORD's Rare Diseases and Orphan Products Breakthrough Summit
Arlington, Va., U.S.
- 19 – 22** RareX 2016 & 11th ICORD Annual Meeting (International Conference on Rare Diseases & Orphan Drugs)
Cape Town, SOUTH AFRICA
- 20** PPTA Business Forum (Members only)
Orlando, Fla., U.S.

- 22 – 25** American Association of Blood Banks (AABB) Annual Meeting
Orlando, Fla., U.S.
- 25** 2nd Cambridge Rare Disease Summit
Cambridge, UK

November

- 5 – 6** Annual Symposium on Primary Immunodeficiency: AUTOIMMUNITY
Newport Beach, Calif., U.S.
- 15 – 17** World Orphan Drug Congress
Brussels, BELGIUM

December

- 3 – 6** 58th ASH Annual Meeting & Exposition
San Diego, Calif., U.S.

2017

February

- 24 – 25** 7th International Meeting on Pulmonary Rare Disease and Orphan Drugs
Milan, ITALY

March

- 14 – 15** 2017 International Plasma Protein Congress
Prague, CZECH REPUBLIC

GLOSSARY OF TERMS

CAP – CENTRALISED APPLICATION PROCEDURE

CCI – COMMERCIALY CONFIDENTIAL INFORMATION

CIADM – CENTERS FOR INNOVATION IN ADVANCED DEVELOPMENT AND MANUFACTURING

CDCS – CROSS DONATION CHECK SYSTEM

CMS – CENTERS FOR MEDICARE AND MEDICAID SERVICES

CT – CLINICAL TRIAL

CTA – CLINICAL TRIAL APPLICATION

EC – EUROPEAN COMMISSION

EDQM – EUROPEAN DIRECTORATE FOR THE QUALITY OF MEDICINES

EMA – EUROPEAN MEDICINES AGENCY

EPA – EUROPEAN PLASMA ALLIANCE

EU – EUROPEAN UNION

EU COM – EUROPEAN UNION COMMISSION ('OM' LOWERCASE)

EUNETHTA – EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT ('NET' LOWERCASE)

FDA – U.S. FOOD AND DRUG ADMINISTRATION

HCV – HEPATITIS C VIRUS

HHS – U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES

HTA – HEALTH TECHNOLOGY ASSESSMENT

IB – INVESTIGATOR'S BROCHURE

Ig – IMMUNOGLOBULIN

IMP – INVESTIGATIONAL MEDICINAL PRODUCT

EudraCT – EU CLINICAL TRIAL DATABASE

IPAW – INTERNATIONAL PLASMA AWARENESS WEEK

IPPIA – INTERNATIONAL PLASMA PRODUCTS INDUSTRY ASSOCIATION

IQPP – INTERNATIONAL QUALITY PLASMA PROGRAM

IT – INFORMATION TECHNOLOGY

IVIG – INTRAVENOUS IMMUNOGLOBULIN

MA – MARKETING AUTHORIZATION

MS – MEMBER STATE

NDDR – NATIONAL DONOR DEFERRAL REGISTRY

PPT – PLASMA PROTEIN THERAPY

QPP – QUALITY PLASMA PROGRAM

QSEAL – QUALITY STANDARDS OF EXCELLENCE, ASSURANCE AND LEADERSHIP

RCT – RANDOMIZED CONTROLLED TRIAL

SEAPID – SOUTHEAST ASIAN PID NETWORK

SmPC – SUMMARY OF PRODUCT CHARACTERISTICS

TBSF – TAIWAN BLOOD SERVICES FOUNDATION

TTIP – TRANSATLANTIC TRADE AND INVESTMENT PARTNERSHIP

VAT – VALUE-ADDED TAX

VIGIV – VACCINIA IMMUNE GLOBULIN IV

VUD – VOLUNTARY AND UNPAID DONATION



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