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Nanofiltration: More Than 20 Years Contributing to Safety

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During one of my flights, I was listening to some music and heard a song from Lady Gaga with the title “Born This Way.” I liked the music, its energy, and started to think about the title.

A child does not ask where she or he is born and will grow up in the country where the first breath will fill the lungs. From that moment on, the child is on its journey to adulthood and (in its innocence) has no idea what the future will bring.

From the moment that the walking begins, and a bit later the first contacts with other children occur, life will change. The majority of the children will occasionally deal with health issues and fully recover, some will not.

Everybody who has children knows that there are many infections to which children are exposed during kindergarten and primary school—infections that they bring home affecting the parents as well. It is at that time that some children have more infections than others and seem not to respond the same way. Some children have a primary immune deficiency and will suffer from more infections, facing many days, weeks, and months of disease and will miss school. Unless...there is awareness and the right expert is consulted, resulting in the right diagnosis. Some children start their therapy with immunoglobulins and can lead a normal life; almost normal since therapy needs to be given on a regular basis.

Another group are the children with hemophilia. Some young boys are diagnosed at a very young age when the complications after circumcision are the first symptoms of a bleeding disorder. Other children have more bruises than others and (when they find the right expert) are diagnosed with hemophilia. Once that is done, the therapy with clotting factors begins and a normal life with normal life expectancy begins. Again, normal with the exception that regular treatments will be necessary.

The key words here are awareness, diagnosis, early treatment, and normal life. Sounds simple doesn’t it?

Unfortunately, that is not the case in all countries of the world. I was recently visiting an enormous hospital in China and learned that it is rare to see an adult person with hemophilia. This is not because they do not exist, it is because there are insufficient means to make therapy available. From the World Federation of Hemophilia, we know that 75 percent of all persons with hemophilia have no treatment at all, which means there will be many joint problems and the life expectancy is much lower.

The answer is not to say: “I Was Born This Way.” The answer is to find ways to bring life-saving therapies to all patients in the world. We have a lot of work to do.

Jan M. Bult, PPTA President & CEO
Plasma-derived medicinal products (PDMPs) have never been safer than they are today. There are several reasons that explain the current safety of these life-saving products. Many measures and initiatives have been taken during the last thirty years, starting at the plasma donor population epidemiology level (knowledge of presence or absence of certain diseases in a certain population), continuing with the process of qualification of the actual individual healthy plasma donor, and the introduction of the screening of donations and industrial manufacturing plasma pools for transfusion-transmissible pathogens by both serological and NAT (Nucleic acid amplification technology) testing. Based on these measures, plasma for fractionation can be considered safer than the plasma for direct transfusion.

At the same time, the improved understanding of the biological properties of PDMPs allowed the application of sophisticated technologies that induced a dramatic change in the purification strategy of most plasma derivatives, thus increasing the purity of plasma products. One of the major factors contributing to an increased safety margin is the introduction in the purification processes of specific steps/technologies with robust and demonstrated pathogen inactivation/removal capacity. The introduction of treatments such as Solvent-Detergent (S/D), Pasteurization, Dry-Heat, or Incubation in the presence of Caprylate greatly contributed to the current safety of PDMPs and marked a significant “before and an after” time-point in their implementation. For example, since the introduction of S/D treatment close to thirty years ago, there has not been a known case of transmission of an enveloped virus by S/D-treated PDMPs licensed in the developed world with over 35 million doses administered. Steps with effective virus inactivation/removal capacity are thus an absolute requirement in order to obtain any safe biological product. This is also stated in relevant guidance issued by the various Health and Regulatory Authorities.

Nanofiltration is one of the technologies that makes a significant difference in assuring the safety of PDMPs. We could describe Nanofiltration as a filtration process through a membrane that selectively permits the passage of certain compounds (filtering) while retaining others (excluding). The main mechanism of action is based on the size of the particles, and the reason why it is called ‘nano’ filtration is because we are working at the nanometer (nm) scale (less than 100 nm, or one billionth of a meter), which is the scale of molecules and macromolecules. The basic idea is ‘size-exclusion’, meaning that the plasma-derived product goes through the filter while the pathogen (as particles) would be retained, if present in plasma, in spite of all the above mentioned measures applied to ensure the safety of plasma.

Nanofiltration was first implemented in production of PDMPs in the early 1990s. The first nanofiltration membranes were developed in the late 1980s when improvements in their membrane structures allowed separation of protein particles with a high resolution. The main reason to introduce the Nanofiltration process was...
the need to improve the safety margin with respect to the smallest (in size) pathogens [e.g., small non-enveloped viruses like Parvovirus virus B19 (B19V) or Hepatitis A virus (HAV)], mainly for clotting factors. It is worth mentioning that neither HAV nor B19V are usually included in the screening of plasma for transfusion. This technology has also been shown to be effective against the bigger (in size) potential pathogens, providing, in fact, an efficient safety step also against emerging pathogens.8

Nanofiltration is now used not only for the production of PDMPs but also in the production of most of the products of biological origin such as recombinant plasma proteins or monoclonal antibodies. The aim is not only to prevent any contamination from the original biological materials (e.g., cells from cell cultures, which produce the recombinant protein) but also to safeguard the raw products used in the production that could contaminate and be amplified by the cell systems employed in the production (e.g., cell culture media, where the cells are grown).

Today, many nanofiltration filters from several manufacturers are available and the technology has always met the needs of the biological products manufacturing industry. While in the beginning the first products to benefit from this technology were the “smaller” molecules (e.g., plasmatic or recombinant Factor IX, Antithrombin, monoclonal antibodies), new developments from the filter makers, including a diversity of pore sizes and membrane structures, combined with the extensive research of the Research & Development (R&D) departments of the plasma industry, allowed this technology to be applied to very complex or fragile molecules such as polyvalent immunoglobulins and more specifically Fibrinogen9 (the von Willebrand Factor).8

As the mechanism of action is based on size exclusion, Nanofiltration does not change the often very sensitive proteins structure and functionality, thereby allowing excellent protein recoveries. Nanofiltration has been shown to be effective under a wide variety of process conditions (e.g., salts, pH, temperatures, protein concentration, etc.) and products.9 It is considered one of the most robust process steps providing pathogen removal capacity.

It is important to highlight here that the remarkable progress of Nanofiltration would not have been possible without the close collaboration between the R&D departments of the

We could describe Nanofiltration as a filtration process through a membrane that selectively permits the passage of certain compounds (filtering) while retaining others (excluding).
plasma industry and the filter manufacturers, especially in the beginning of their development (1990s-2000s). Filter producers came mainly from the chemical industry (and had experience in separation technologies through ultrafilters and microfiltration); the application to biological products, especially PDMPs, had particular needs and specificities and the relevant adjustments needed some time of close cooperation with the plasma industry.

When I first came in contact with this technology (nearly 20 years ago) at the R&D department of my company I really thought about the similarities of Nanofiltration with the final sterile filtration for bacteria and the concept of sterile products. At that time I thought we were far from achieving this with Nanofiltration, but today—together with all the safety measures described—it could be considered a near reality for some proteins and types of filters.

Today we cannot imagine new developments of medicinal products of biological origin without considering the implementation of Nanofiltration. Rather, one of the first questions that arise in one’s head is “In which step of the production process we will introduce Nanofiltration.”

References:
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PPTA Participates in Healthcare Business Development Mission to China

BY JAN M. BULT, PPTA PRESIDENT & CEO
JULIA FABENS, PPTA MANAGER, INTERNATIONAL AFFAIRS

MISSION TO CHINA SELECTION
For the first time, the United States Department of Health & Human Services (HHS) and the United States Department of Commerce (DOC) co-organized a Healthcare Business Development Mission to China, which took place on Oct. 16-21. PPTA was selected to be one of the 12 official delegate organizations, along with PPTA Global Member, Grifols.

MISSION GOAL
“The mission’s goal is to provide participating firms and trade associations valuable market insights, industry contacts, ideas for solidifying China business strategies, and advance notice of specific projects, with the overall objective of establishing a firm foundation for future business in China. This will take place through meetings with senior government representatives and face-to-face dialogue between government, industry and business leaders. Moreover, this mission reaffirms the U.S. commitment to sustained economic partnerships with China.”

MEETINGS IN BEIJING
The trip started in Beijing where various meetings were conducted. The U.S. government (USG) was represented by the DOC, led by Deputy Secretary of Commerce, Bruce Andrews and by the HHS, led by Acting Deputy Secretary Mary Wakefield, Ph.D., RN. In addition, the USG was represented by U.S. Embassy staff (Commerce Division). In two meetings the U.S. Ambassador to China, Max Baucus, participated as well.

The meetings were high-level and provided good insight into the current thinking on many topics. It has to be understood that in this kind of one hour meeting, half of the time is filled with the usual greetings and introductions, 20-25 minutes with stating prepared talking points from the Chinese delegation and (at most) 5-10 minutes answering one or two questions. The true value for the participants was in the post meeting conversations. Being part of the delegations provided an opportunity to open doors that normally take much longer to open.

A few meetings stood out, such as with the National Health and Family Planning Commission. The lead person for the Chinese Government was Vice Minister Li Cui; the U.S. delegation was accompanied by U.S. Ambassador Baucus. After the usual introductions, the Vice Minister explained what happened with China Health Care Reform. She noted that the focus has shifted from the many infectious diseases to the problems with non-communicable diseases that account for 80 percent of the mortality rate in China. Twenty-eight new principles have been developed to provide high-quality healthcare in China. It is anticipated that in 2020, the life expectancy will be around 77 years. It was also explained that 95 percent of the population now has health
insurance with the comment that this is not as good as in the U.S. We learned that the insurance covers RMB 550 ($80) in the rural areas and about RMB 3,000 ($440) in the urban areas. Needless to say, this is not enough to cover healthcare. Though the meeting was held in a positive spirit, the Vice Minister made some surprising comments, such as:

- Why can’t you consider reducing your patent period?
- We would like to see an earlier transfer of intellectual property.
- Donations of products are very welcome.

The entire delegation, including the Ambassador, was taken by surprise. He did not let this go and asked whether he heard this right. The Ambassador made it clear that universal rules are needed when it comes to patent protection, copyright, and trademarks.

Another topic that was raised was the issue of rare diseases. One Chinese government official questioned the validity of the WHO rare disease definition for China and stated: “even with that definition, we end up with a lot of patients and that is a problem for China. There is need for a balance between affordability and the available financial means.” It was suggested that the creation of a screening panel to identify six rare diseases would help to resolve the problem.

These subjects were the focus of many discussions afterwards during a reception in the residence of the Ambassador. All delegation members had ample opportunity to interact with the U.S. delegation leaders and the Ambassador in a very relaxed atmosphere.

In a meeting with the Ministry of Human Resources and Social Security (MOHRSS) Vice Minister, Changsheng Kong, the issue of insurance was further elaborated upon and payment reform was explained. There is a total control of expenses for disease-based payments. To monitor the expenses, a Medical Services Management System was developed, whereby innovative products are reviewed by an expert review panel to determine whether they will be reimbursed. There is a policy for the prescription of generic products. According to MOHRSS, it is difficult to include innovative products because of the cost. Another interesting observation was that there are different insurance systems for urban and rural areas, leading to complaints from Chinese patients.

The last important meeting in Beijing was with the China Food and Drug Administration (CFDA), with the Vice Minister, Wu Zhen, as the main speaker. Again, the U.S. delegation included the U.S. Ambassador. The Vice Minister explained the 2014 reform with regard to the licensing priorities, noting the quality, efficiency, and speed of the approval process. There is currently an enormous backlog of applications but it is expected that this will be resolved by the end of 2018. One of the measures taken was to increase the number of reviewers from 120 to 400.

It was interesting to hear that the definition of a new drug has changed. The new Chinese definition is: “Only products that have never been marketed before are considered new drugs.” It was also explained that, though CFDA is not involved in the pricing of products, several rules will apply:

- Prices for drugs in mainland China cannot be higher than in the country of manufacturing or in neighboring countries. Companies need to “promise” that.
- Prices for drugs in mainland China cannot be higher than in Hong Kong.

It was clear that the CFDA understood the sensitivity of this subject and expressed the hope that these issues can be resolved between the Ministries of Health and not be a subject on the agendas of bilateral negotiations. Based on the comments that were heard from the delegation members, this approach is not supported by their U.S. counterparts.
MEETINGS IN CHONGQING

The second part of the trip took place in Chongqing, China, a city with 34 million inhabitants. It is important to realize that this city alone has more people than many countries in the world.

The DOC, the U.S. Consulate General in Chengdu, the U.S. Commercial Service China, and the Chongqing Medical Association organized the U.S.-China Advanced Medical Technology Symposium on various topics:
- Hematology and Oncology
- Woman’s and Children’s Health and Precision care
- Chronic Disease Management
- Eye Care: Myopia Management

The delegates all had an opportunity to present. Both Grifols and PPTA presented in the session on Hematology and Oncology, which was very well received. The title of the PPTA presentation was: “Plasma Protein Therapies: Promoting Safety, Efficacy and Patient Access Worldwide.” The presentation is available on the PPTA website (www.pptaglobal.org). Many of the people in the room did not know that plasma protein therapies cannot be imported, with the exception of albumin. The first question that was asked showed how much more education is necessary; the question was whether the industry could consider lowering its prices. It was explained that before price can be discussed, therapies must first be brought into China.

The last meeting in Chongqing was with the Chongqing Health Bureau, which provided an opportunity for the delegation to understand more about the execution of government policies within a province. The session was extremely enlightening and showed how different national and provincial policies are.

In the afternoon of the last day, a visit was organized to a large hospital in Chongqing. In 2015, this hospital—with 35 clinical divisions and 3,200 beds—had over 3 million outpatients, about 130,000 hospitalized patients, and performed over 95,000 surgeries. It was very informative to see the daily operation of such a large hospital. There are long lines just to get into the elevator. Long waiting times are very common and the time patients are seen by the doctor is very limited. Seeing 200 patients per day is not uncommon; the physicians are working very hard with the resources available.

While at the First Affiliated Hospital of Chongqing Medical University, the delegation visited the hematology clinic where most of the patients that were hospitalized were dealing with malignancies. We saw many busy doctors looking in microscopes and studying the cytology of biopsies to determine the hematological disorders. We also saw several isolation units for patients that have to undergo bone marrow transplants. When asked how many persons with hemophilia were seen in this clinic, the answer was that this number was very low because hemophilia was seen in the pediatric clinic. When it was pointed out that persons with hemophilia grow older, the next comment was shocking: “We do not see many adult persons with hemophilia because this is economically not possible.” Clearly, there is work to be done to accomplish PPTA’s mission, which is to “Save patients’ lives in all parts of the world.”
In the current EU context, there has not been a single week without a new development or initiative to keep healthcare expenditures under control at the EU level or at the national level. Not only has the economic pressure increased in the past few years, but there are also new drugs on the market and new rare disease treatments in the pipeline.

When you consider that the Hepatitis C treatment, Solvadi, surpassed $10 billion in its first year of launch in 2014, that 126 Orphan Medicinal Products (OMPs) have been approved in the EU since 2000, and that 1,621 OMPs are in the pipeline with an average cost of more than $166,000 per patient, it’s easy to imagine how the access to care boat is rocking at the moment. All of that in the context of an increase in life expectancy.

The debate on how nations can afford these new treatments has started, but so far no clear solutions have emerged and rare disease advocacy patient groups are already alerting policymakers about the growing population that will knock at their door if they don’t get reimbursement for their therapies. There is, in parallel, the development of the health technology assessment discipline and increased research in pharmacoeconomics and outcomes that should help key deciders make sound decisions and develop new economic models.

At the same time, policymakers are already making decisions that could be detrimental for maintaining or developing access to care for plasma protein therapies (PPTs). If we focus at the national level we see the following examples:

**CLAW BACK TAXES**

A mechanism by which companies distributing reimbursed drugs have to refund a part of their turnover based on the deficit of the healthcare system. It is generally quite unpredictable, variable, and highly questionable when there is already a procurement system through tenders or a demand management plan in place. Ongoing claw back discussions are happening in Greece, Portugal, Romania, Italy, and the UK, to name just a few. Fortunately, some countries (e.g., Belgium and Germany) understand the uniqueness of our sector and have granted exemptions to the pharmaceutical clawback tax. Also interesting is the WHO guideline on country pharmaceutical pricing policies that recommends considering “exempting essential medicines from taxation for reasons of equity and safeguarding access to adequate care.” PPTs are listed as “essential medicines” and should thus be exempted from these measures.
MAXIMUM REIMBURSED PRICE DECREASE:
Another classic process is a sudden decision by national authorities to decrease the reimbursement level of pharmaceuticals. Several calculation methods may apply but it usually affects all drugs allocated to specific categories (e.g., pharmaceuticals, generics, etc.). Generally speaking, the specific character of PPTs is not considered and measures may drastically affect the access to care of patients who need these essential medicines. Fortunately, in some cases the authorities conduct a public consultation before implementing the measures (e.g., UK) or may open dialogue with patients, caregivers, and industry (e.g., Portugal). However, the outcome of such dialogue is always quite uncertain.

Also, **regular price decrease** mechanisms are embedded in the laws of some countries—again, without any specific consideration for PPTs, as these medicines are lumped together with classical pharmaceuticals.

Another related mechanism is the **international reference pricing** that countries are using to compare national list prices and decrease their reimbursement level price. One of the side effects of this mechanism is that it creates a vicious spiral over the years in terms of maximum reimbursement price level. PPTs are not comparable to classical pharmaceuticals in terms of their life cycle and this system can put access to PPTs at risk.

TENDER
Tendering is a common practice in several countries and can be organized at the national, regional, or hospital level. Good tendering processes should allow for several selection criteria and not only focus on price. A classical downside of tendering is that it generally decreases the therapeutic choices and indirectly implies that PPTs are somehow considered interchangeable. In that context, it’s interesting to read the Council of Europe resolution including the following principle: “To take into account that human normal immunoglobulin therapeutic products differ from one another in terms of production processes, which might have an impact on specifications and clinical performance.”

MAXIMUM PRICE – MAXIMUM BUDGET
Another system is to allocate a limited price or budget for the procurement of certain drugs. This system is regularly done without looking at the other healthcare expenditures that will be caused by the lack of appropriate treatment or it’s simply not addressing the needs of the patients, which may cause their health to deteriorate; which is not uncommon in Eastern Europe. Our sector has high production costs and ongoing investments are important. If the reimbursement level is too low, the cost of production may not be covered, which puts access to care at risk. It’s interesting to note that the private sector is cost efficient for the production of PPTs and that several countries have closed or privatized their public facilities to produce PPTs (e.g., Scotland, Finland, Denmark, Switzerland, England, and Norway). In some countries, due to the lack of budget, some indications are simply not reimbursed or only for a very low number of patients. These countries are unfortunately struggling with priorities in budget allocation.

MIX
Of course, the mix of several measures is not excluded and several countries are blending the usage of these concepts to control expenditures. We regularly see that when the current ongoing systems are changed, there are issues with coordination and implementation, and sometimes even legal issues. This is due to the fact that national pricing and reimbursement systems for pharmaceuticals are complex and interconnected. Changing one element of the system can be very disruptive and may lead to unintended consequences.

APPROACH TO MAINTAIN AND DEVELOP ACCESS TO CARE FOR PPTS
The role of patient advocacy groups is crucial in the dialogue with authorities, since they conduct continuous awareness and education activities.

As a result of the change in the Statutory Scheme consultation, the UK has recently recognized the specific character of PPTs: “The UK Government has listened to concerns about maintaining adequate supplies of essential medicines and specific supply issues for products such as plasma protein therapies and nuclear isotopes.” The Government recognizes the need for provisions to allow for either temporary or permanent increases in maximum price in order to address short term or long term supply problems and ensure continued adequate supply of essential medicines.”

This is good news, but it has yet to be seen how these provisions will be implemented and if they will ensure sustained access to care. The drop in value of the British Pound due to “Brexit” has dramatically impacted the reimbursement level, adding to the complexity of the situation in UK.

As it can be seen, there are numerous challenges, but strong efforts should be made to make access to care a high priority on the agenda of key decision-makers. PPTA continues to engage with national authorities whenever necessary.

References:
http://dipbt.bundestag.de/dip21/btd/18/002/1800201.pdf
2. WHO guideline on country pharmaceutical pricing policies (2015) p.vii and p.10
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This year, PPTA was—for the first time—represented at the European Congress of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR). ISPOR was founded in 1995 and has been growing toward a global scientific and educational organization for health economics and outcomes research and its use in decision making. PPTA exhibited posters presenting the results of two literature studies on severe hemophilia A.

### REVIEW OF NATIONAL HEALTH TECHNOLOGY ASSESSMENT REPORTS ON TREATMENT OF SEVERE HEMOPHILIA A

The purpose of the first study was to review the national health technology assessment (HTA) reports in Europe comparing on-demand treatment with prophylaxis treatment of severe hemophilia A. To date, two HTA bodies have published their analysis on the topic: SBU (Sweden, 2011) and IQWIG (Germany, 2015). Both examined the effects of clotting factor concentrates therapy. A comparative analysis showed inconsistencies in methodological approaches. SBU included in the evidence review a broad scope of studies, including observational trials and discussion papers, and actively engaged external experts and patient organizations in the consultation process. IQWIG relied mainly on the judgment of the internal experts and focused on randomized controlled trials (RCTs) only, limiting the review to three studies: one in adolescents and adults (SPINART) and two in children (ESPRIT and JOS). This methodological difference led to controversial conclusions regarding the benefit of prophylaxis compared to on-demand treatment in severe hemophilia A. While the SBU report recognized that...
its conclusions are based on “best clinical practice” rather than high-level evidence typically generated by RCTs. At the same time, it acknowledged that performing RCTs in hemophilia is difficult if not impossible and reported consistent evidence that prophylaxis initiated at a young age can prevent future joint damage. The IQWIG report, in contrast, asserted that RCTs in hemophilia are possible and concluded positive effects of prophylaxis in adolescents and adults, and only signs of additional benefit in children.

To challenge IQWIG’s approach, I’d like to refer to the Cochrane Review2 on prophylaxis in severe hemophilia A published by Iorio et al. in 2011. In its evidence assessment, the authors went beyond RCTs and found strong evidence that prophylaxis preserves joint function in children with hemophilia. Confronting the national HTA bodies with inconsistencies in their practice is important, as they raise concerns about sustainable access to early prophylaxis for children with severe hemophilia A. Overall, improving consensus on the effectiveness assessment of therapies in rare diseases and appropriate patient involvement would enhance common understanding of the therapeutic value of treatments and potentially improve patient access in countries where access is limited.

COST-EFFECTIVENESS OF PROPHYLAXIS COMPARED TO ON-DEMAND TREATMENT IN SEVERE HEMOPHILIA A: SYSTEMATIC REVIEW

The second study critically appraised current evidence on the cost-effectiveness (CE) of prophylaxis compared to on-demand treatment in patients with severe hemophilia A. The topic is relevant, since many countries still do not offer prophylactic treatment with clotting factor concentrates as the standard of care.

The eight identified publications dating from 1996 to 2013 and assessing CE in the U.S., UK, Germany, Netherlands, Sweden, Italy, and Canada, reported strikingly conflicting results, from cost-saving to not cost-effective at all. A closer look at the methodologies showed that the studies applied a different time horizon: some of them modeled the results observed within the clinical trials in the future by using the lifetime perspective; others limited their analysis to a short-term perspective with the maximum of six years. Remarkably, the modeling studies show more consistent outcomes, - from cost-saving up to €80,566 ($85,345) per quality-adjusted life year, staying within or approaching the reimbursement thresholds common in Western economies.

To avoid confusion among the national healthcare policymakers, these inconsistencies need to be addressed. Following guidelines for health economic evaluations in chronic conditions and using a lifetime horizon is the only meaningful approach to the CE analysis. Indeed, the clinical and economic consequences of (avoiding) the bleedings in persons with hemophilia cannot be fully assessed until after many years. The results of the short-term economic evaluations must be therefore interpreted with caution.

The findings of both studies can strengthen patient access advocacy at the scientific and policy level.

References:
1. Health economics and outcomes research evaluates the effect of health care interventions on patient well-being including clinical, economic, and patient-centered outcomes.
2. Cochrane Review is considered to have highest level of evidence.
FIND-ID Seeks to Improve Early Diagnosis and Treatment of Congenital Immunodeficiencies

BY SANDOR TÓTH, PPTA SENIOR MANAGER, GERMANY

In order to recognize congenital immunodeficiencies in patients at an earlier stage, thereby improving their life expectancy and their quality of life, doctors from the German Jeffrey Modell Foundation centers, and representatives of the Deutsche Selbsthilfe angeborene Immundefekte (dsai e.V.)—a German association for patients with Primary Immunodeficiencies—gathered at the invitation of Fred and Vicki Modell for the 2008 European Society for Immunodeficiencies (ESID) Congress in the Dutch city of ‘s-Hertogenbosch. As a result of that meeting, Professor Tim Niehues (HELIOS Clinics, Krefeld, Germany), Professor Volker Wahn (Charité, Berlin) and Gabriele Gründl (dsai e. V.) developed a plan for a network initiative: FIND-ID was born.

The creators of FIND-ID are focused on achieving early diagnosis and treatment for patients with congenital immunodeficiencies (primary immunodeficiency diseases – PID). Of the approximately 40,000-100,000 persons affected throughout Germany, only about 3,500-4,000 patients are currently diagnosed. Since its foundation in 2008, the work of FIND-ID has contributed to improving the rate of diagnosis and has increased awareness of the condition.

The number of patients documented in the ESID registry increased from 785 patients in 2009 to 1,978 patients in 2014. The campaign group has observed that a PID condition is still often diagnosed too late or the diagnosis is delayed by several years. Usually, the situation for adults is worse than that of children and an effective therapy can often only be started very late or not at all.

FIND-ID has two main objectives: to close the knowledge gap about PID among medical specialists potentially involved in the treatment of patients with PID like ENT doctors, dermatologists, pneumologists, gastroenterologists, oncologists, rheumatologists, and others; and to improve the communication between those medical specialists and the specialized immunodeficiency treatment centers who are the core members of the FIND-ID physicians network. By doing that, the cooperation with the immunodeficiency centers on complex laboratory diagnostics or on serious decisions about therapy will be strengthened and doctors will be educated about PID. Only appropriate diagnostic instruments should be used for a diagnosis and patients should be analyzed in relation to their heredity if there are cases of genetic defects.
FIND-ID CAMPAIGNS FOR IMPROVED QUALITY OF CARE

Ideally, patients and the physicians who treat them should be linked to a center specializing in primary immunodeficiencies. This ensures that the patients can be treated in accordance with the latest developments and insights of medical science. The number of immunodeficiencies known to date with their complex symptomatology is increasing but the number of patients is minimal. This calls for close cooperation between doctors and specialists.

FIND-ID STANDS FOR FAIR PARTNERSHIP

The network sees open communication as the basis for successful cooperation. Information loss between network partners must be avoided, and all those involved in treating the patients must be fully and continuously informed of everything they need to know for that treatment. These communications, along with prompt feedback, creates a robust network. This will also allow a joint therapy plan to be implemented along with primary treatment instances.

NEWBORN SCREENING FOR SEVERE CONGENITAL IMMUNODEFICIENCIES MUST BE IMPLEMENTED

“It is high time that severe congenital immunodeficiencies be added to the existing screening catalog for the newly born,” says Professor Dr. Tim Niehues, Director of the Center for Pediatric and Adolescent Medicine at the HELIOS Clinics in Krefeld, Germany. Moreover, the creator of the FIND-ID physicians’ network is delighted that, thanks to the possibilities offered by screening, immunology has “shifted as never before into the focus of preventive medicine.”

In the United States, severe combined immunodeficiencies (SCIDs) are already on the list of illnesses for which newborns are screen tested. This is not yet the case in Germany; the conditions covered by screening include congenital metabolic and hormone deficiencies, but, to date, no congenital immunodeficiencies. In particularly severe forms of congenital immunodeficiency—the so-called SCIDs—it is vital that the diagnosis be made very soon after the birth of the child so that an appropriate therapy can be initiated as soon as possible.

“One of the key objectives of FIND-ID is that newborn screening for SCID should be implemented reliably, economically, and, last but not least, in a patient-friendly and ethically justifiable manner,” Niehues declares. In this respect, FIND-ID works together with the Association for Pediatric Immunology (API), and with the Pediatric Immunology Working Group of the German Society for Immunology. “Such cooperation is of vital importance,” Niehues emphasizes. “Working via these specialist associations, FIND-ID can also involve policy-makers in its objectives.”

As a result of intensive discussions between the API and the National Association of Statutory Health Insurance Funds (GKV-Spitzenverband), in 2014 the latter requested the German Federal Joint Committee (G-BA) to assess newborn screening for congenital immunodeficiencies. Following standard procedures for assessing health technologies in Germany, the German Institute for Quality and Efficiency in Health Care started to evaluate SCID screening and published a preliminary report in the summer of 2016 with the following conclusion: “Newborn screening for severe combined immunodeficiencies (SCID screening) in combination with an infection-prophylactic therapy leading to curative therapy is indicative of a benefit.”

FIND-ID and API submitted brief statements on this preliminary report supporting the introduction of a nationwide SCID screening.

Based on the positive result of the benefit assessment, it seems likely that the final report will be positive, paving the way for the introduction of SCID screening in Germany.

“Especially in the adult sector, the awareness of this disease is not yet as well developed as we would wish it to be. It was for this reason that Professor Volker Wahn of the Charité in Berlin, Gabriele Gründl, Chair of the dsai patient organization for congenital immunodeficiencies, and I set up FIND-ID in 2008.”

For registered resident doctors in particular, FIND-ID provides fast and easy access to experts, to whom they could directly transfer patients with a suspected congenital immunodeficiency.

“WE NEED EVEN MORE RESIDENT PHYSICIANS IN FIND-ID.”

It is this very network concept that Niehues regards as being one of the key advantages of a membership in FIND-ID. “As a physician, you then have faster access to information, and we are represented with ID centers in almost all regions of Germany, so that resident doctors can quickly find an expert near to where they are.” He argues the case for further expansion of the network—with hub centers, smaller centers, and resident physicians—throughout Germany. “It would be splendid if we could have even more partners in private practices, so that we will begin to see more and more contact points for patients.”
The Council of Europe and its Relevance to the Plasma Protein Therapeutics Industry

BY KARL PETROVSKY, PPTA SENIOR MANAGER, HEALTH POLICY

The Council of Europe (CoE) is an international organization focused on promoting human rights, democracy, and the rule of law in Europe. Founded in 1949, it has 47 member states and covers approximately 820 million people in Europe, including countries like the Russian Federation and Ukraine.

The CoE is distinct from the 28-nation European Union (EU), founded in 1957, though the CoE is sometimes confused with the EU. This is partly because the EU has adopted the original European Flag which was created by the CoE in 1955 and all 28 countries that joined the EU are also members of the CoE.

The two big areas of interest for PPTA within the CoE concentrate on the European Directorate for the Quality of Medicines (EDQM) and the activities on Bioethics.

The Council of Europe upholds its founding principles of human rights, democracy and the rule of law through conventions such as the European Convention on Human Rights, and the Convention on Human Rights and Biomedicine. Such conventions are legally binding only on the member states that have signed and ratified them. The Council of Europe can also adopt non-legally-binding (so-called “soft law”) instruments in the form of Recommendations to the governments of its member states. Although such Recommendations are not legally binding, the Committee of Ministers of the Council of Europe may request governments of member states to inform it of the action taken by them with regard to such Recommendations, thereby making such Recommendations particularly authoritative.

Separately, the works of the Council of Europe can also be explicitly referenced in binding EU law, for example in the EU Blood Directive 2002/98/EC.

The CoE’s two statutory bodies are the Committee of Ministers—comprising the foreign ministers of each member state—and the Parliamentary Assembly, composed of
members of the national parliaments of each member state. The CoE headquarters are located in Strasbourg, France.

The CoE is structured into two Directorate Generals (DGs):

- **DG I Human Rights:** This DG hosts the activities around bioethics, with the Committee on Bioethics (DH-BIO)
- **DG II Democracy:** This DG hosts the activities of the EDQM

**EUROPEAN DIRECTORATE FOR THE QUALITY OF MEDICINES & HEALTHCARE**

The EDQM, created in 1996, is based on the Convention on the Elaboration of a European Pharmacopoeia of 1964. EDQM’s mission is to contribute to “the access to good quality medicines and healthcare.” Gradually, several activities were transferred to this entity including blood transfusion and organ transplantation in 2007 and pharmaceutical in 2008. The departments of the EDQM include:

- **The European Pharmacopoeia Department** is responsible for the Secretariat of the European Pharmacopoeia Commission and for preparing the General Chapters and Monographs of the European Pharmacopoeia with the groups of experts.
- **The Laboratory Department** contributes to the European Pharmacopoeia Department by providing analytical studies to develop its texts (General Chapters and Monographs,)

and by establishing the corresponding reference standards. Finally, it contributes to the development of Proficiency Testing Scheme studies for the General European Official Medicines Control Laboratories Network (GEON) and the World Health Organization.

- **The Biological Standardisation, Network of Official Medicines Control Laboratories (OMCL),** is responsible for
  - The Biological Standardisation programme,
  - The GEON, including the European Official Control Authority Batch Release (OCABR) for Medicinal Products Derived from Human Blood and Plasma.

The **OCABR Network** develops and maintains codified procedures and guidelines in order to:

- **Facilitate** mutual recognition within CoE member states
- **Provide** a platform for confidential exchange of information and data
- **Foster** the use of harmonized approaches
- **Promote** work-sharing and maximization of resources
- **Create** a unified voice for feedback and exchange with manufacturers, regulatory bodies, the EU Commission, and European Pharmacopoeia expert groups
Amongst the Divisions of the EDQM are:

- The **Certification of Substances Division (DCEP)**, which is responsible for implementing the procedure for Certification of Suitability (CEP) of the monographs of the European Pharmacopoeia. The DCEP is also responsible for the organization of onsite inspections of manufacturing sites and their follow-up, including the implementation of any subsequent action regarding the related CEPs and communication with the concerned authorities.

- The **Reference Standards and Samples Division**, which is responsible for the production, storage, and dispatch of the European Pharmacopoeia Reference Standards. It also procures samples, which are analyzed in the process of elaborating the European Pharmacopoeia Monographs.

- The **Quality, Safety & Environment Division (QSED)**, which coordinates the development and maintenance of the EDQM’s quality, safety, and environmental management systems and aims to continuously improve EDQM products and services. The QSED is also responsible for the release of reference standards and samples produced at the EDQM.

In 1964, the **European Pharmacopoeia** was created on the basis of the Convention on the Elaboration of a European Pharmacopoeia. In 1975, the European Pharmacopoeia gained a mandatory status for all EU/European Economic Area (EEA) member states. In 1994 the OMCL was created as well as the certification procedure for substances for pharmaceutical use.

The **Steering Committee on Blood Transfusion** is an intergovernmental committee created in 2007, which directly reports to the Minister Committee. Its tasks include:

- The exchange with European Pharmacopoeia
- The collaboration with EU Commission (DG SANTE);
- The elaboration of important EDQM Guiding principles related to blood;
- The elaboration of the “Guide on the preparation, use and quality assurance of blood components.” The “Guide” is updated on a yearly basis, the 19th edition (2016) is currently being reviewed. The Guide has the status of a legally non-binding Recommendation.

On Sept. 20, the EDQM organized on a meeting of its extended “Plasma Supply Management Group” to which PPTA was invited. Included on the agenda was plasma donor safety, volume replacement, and stakeholder comments on the “Guide’s” 19th edition update. It was an excellent occasion for PPTA to address its views regarding these items.

Finally, the EDQM also has competencies on issuing recommendations regarding the plasma protein therapeutics side. This is why the EDQM has been involved for several years in the Wildbad Kreuth/Germany workshops on treatment with plasma protein therapies, which it co-organized with the Paul-Ehrlich-Institute, and the University of Munich; these workshops were attended by other authorities, patients, and industry. In 2015, these initiatives resulted in the adoption of two important Resolutions by the CoE/EDQM:

- The Resolution CM/Res (2015)3 on general principles regarding the therapies for haemophilia, and
- The Resolution CM/Res (2015)2 on general principles regarding the therapies with human normal immunoglobulin for immunodeficiency and other disorders.

**THE ACTIVITIES OF THE COUNCIL OF EUROPE ON BIOETHICS**

The basis of the CoE’s work in this area is the “Oviedo Convention for the protection of human rights and dignity with regard to the application of biology and medicine.” All 28 EU Member States have signed and are legally bound to the Oviedo Convention.

A key stipulation in this Convention contains unclear wording with regard to the compensation of donors of blood and plasma. A CoE working group is currently working on the clarification. In this context, it is PPTA's view that compensation of plasma donors must include a financial lump sum for the time it takes to donate and the inconveniences related to the donation. This has been well functioning in countries like Germany and Austria for decades and is crucial for the collection of plasma to be manufactured into life-saving plasma protein therapeutics for patients who heavily depend on them.

**SUMMARY**

The aforementioned activities illustrate the importance of the CoE to PPTA members and the plasma protein therapeutics industry. The CoE’s importance is best illustrated by its very close interactions with the EU Commission's Directorate General Health.
PPTA Interview: Lisa Butler, Executive Director of the GBS|CIDP Foundation International

BY JULIE BIRKOFER, PPTA SENIOR VICE PRESIDENT, NORTH AMERICA & GLOBAL HEALTH POLICY

The GBS|CIDP Foundation International is the preeminent global non-profit organization supporting individuals and their families affected by Guillain-Barré syndrome (GBS), chronic inflammatory demyelinating polyneuropathy (CIDP), and related syndromes such as multifocal motor neuropathy (MMN) through a commitment to support, education, research, and advocacy.

Can you tell me about the GBS|CIDP Foundation and the work you do?

We were founded 35 years ago by Estelle Benson. Her husband, Bob Benson, was diagnosed with GBS and when she went to look for information and support services for this rare condition, nothing existed. She vowed that she would do something about this. The foundation was started in her living room outside of Philadelphia, Pennsylvania. Estelle Benson approached the University of Pennsylvania and Dr. Arthur Asbury responded with his resident, Dr. David Cornblath, and it blossomed from there. Now we serve 35,000 members and have opened our doors to other autoimmune neurologic disorders such as CIDP, MMN, and others.

From the very beginning, the foundation wasn’t founded to look for a cure—it was founded to provide support. We’ve been true to that mission from the beginning. We strive to provide access to early and accurate diagnosis, appropriate and affordable treatment, and knowledgeable support...
The four mission pillars that guide us are: support, education and awareness, research, and advocacy. Every decision we make is based on these four pillars.

services. The four mission pillars that guide us are: support, education and awareness, research, and advocacy. Every decision we make is based on these four pillars. We are very fortunate to have support from the pharmaceutical industry. We also have an incredible global medical advisory board with some of the world's top neuromuscular neurologists who guide our science and research. We have an amazing board of directors and are fortunate that we are never lacking for volunteers. People are always stepping forward and wanting to give back. We have over 200 volunteers worldwide who provide support. They are available for patient visits, they organize local chapter support group meetings, they hold walk events, and they engage in advocacy from the grass roots up to Capitol Hill.

What is Guillain-Barré syndrome?
Guillain-Barré syndrome (GBS) is an inflammatory disorder of the peripheral nerves. GBS is characterized by the rapid onset of numbness, weakness, and often paralysis of the legs, arms, breathing muscles, and face. It is a rare condition affecting 1-2 people per 100,000. Typically, in GBS, the treatment protocol is plasmapheresis or intravenous immunoglobulin (IVIG).

What is Chronic Inflammatory Demyelinating Polyneuropathy?
Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) is a rare disorder of the peripheral nerves characterized by gradually increasing sensory loss and weakness associated with loss of reflexes. CIDP is a chronic form of GBS. Patients need infusion therapy every four to six weeks. Most commonly CIDP patients are treated with IVIG, which is the only approved treatment for CIDP.

How did you get involved in the GBS|CIDP Foundation International?
In 1990, my father-in-law was diagnosed with GBS. My family had never heard of it and couldn't pronounce it. He survived and made a very good recovery. He was treated at the University of Pennsylvania and the physicians there put my mother-in-law in touch with Estelle Benson.

Ten years later, our son started displaying some issues with his gait. We thought there was some similarity to my father-in-law's symptoms but had no idea that children could get GBS. He was indeed diagnosed with a serious case of GBS and was treated with IVIG and eventually made a pretty full recovery. When my son was diagnosed, my mother-in-law reached out again to Estelle and the Foundation for information and support. The Foundation sent us literature and when my son relapsed they put us in touch with a parent volunteer who helped to put our son's neurologist in touch with a pediatric neurologist who specialized in GBS in order to help determine the best course of treatment.

As soon as my son recovered, both he and I started volunteering for the Foundation and eventually took over as the parent liaison for children who were diagnosed. I started working full time with the Foundation three years ago, helping with the volunteer program. I next started working in marketing and development. When the Executive Director stepped down about a year ago, I was fortunate to step into the role. I am honored and humbled that I spend my days helping other people—there is nothing more fulfilling. The biggest impact we provide is connection and community in a world where people feel isolated and alone.
What has been your greatest challenge?
I think our biggest challenge is awareness, on many levels, with physicians, emergency room staff, and the general public. People have issues with not being able to obtain early diagnoses and therefore do not receive early treatment, which can affect their outcomes.

I think the hardest day is when you talk with patients and you don’t have an immediate answer for their concern or situation. That, very often, deals with financial or access to care issues. Continually reevaluating the programming we’re providing and making sure we are answering the patient’s needs—that’s a challenge but that is our mission.

What are the greatest strengths of the Foundation?
Providing that sense of community and connection for patients and making them feel a part of something. Often, people come to one of our events and it is the first time they’ve met other people who have had a similar journey and it can be a very emotional and powerful moment. Knowing that we are providing these connections is incredibly fulfilling. We provide a place where everyone can belong.

Also, our doctors are so collaborative. The Chairman of our Global Medical Advisory Board, Dr. Kenneth Gorson at St. Elizabeth’s in Boston, Massachusetts and our Vice Chairman, Dr. Bart Jacobs, at Erasmus University in the Netherlands represent this group of world’s finest in the field of neuromuscular neurology. It is incredible how they’ve devoted themselves to these conditions.

What are the Foundations priorities?
We have never swayed from our mission. We are solidly focused on our mission. We need to be constantly responding to new challenges that our patients face. For example, dealing with the impact of the Zika virus that can cause GBS. Puerto Rico has been significantly impacted with Zika-related GBS and the Foundation has partnered with the Centers for Disease Control and Prevention (CDC) to host a large chapter meeting where past GBS patients will attend and be a resource for newly diagnosed GBS patients. The CDC will participate and talk about how they will support these patients. Our medical advisory board published an article in the New England Journal of Medicine about the Zika virus and its association with GBS. We’ve talked with our industry partners about donating IVIG to Central and South America where there is a real need for therapies.

Our newest mission is advocacy and we’ve done a lot of work both on Capitol Hill and at the grass roots level to reach out to members of Congress. On the occasion of our 35th anniversary, we honored Congressman John Garamendi (D-CA), who has a family connection to GBS. Congressman Garamendi has been an incredible leader for us.

GBS has also been named as an eligible condition with the Peer Reviewed Medical Research Program, a U.S. Department of Defense program. The Foundation was able to establish the connection between the deployment of service members and the increased exposure to triggers of GBS. This opens up a lot of research avenues and is a very exciting development.

What motivates you day to day?
We receive 75-100 inquiries weekly. The knowledge that we can help that many people is incredibly motivating. Being able to provide the resources and community for people who have questions and who are looking for other people who have had a similar path is very fulfilling.

We have had some of our “walk and Rolls” where people have literally taken their first steps. People will come to a walk in their wheelchair and during the walk with get out of their wheelchair and take their first steps. It is so moving and exciting to see their journey and their success.

What does the future hold for the Foundation?
What I see for the future is that we move forward without forgetting where we’ve been. We should never forget our beginnings in Estelle Benson’s living room and remain committed to our mission to provide support, education, awareness, research, and advocacy.

We don’t presume to have all of the answers but we strive to be the connector to the best resource for our patient community. As we have grown and flourished over the past thirty-five years, we look forward to ensuring our legacy by strategically planning for the future needs of our patients.
Collect with confidence and optimize operational efficiencies

As the demand for life-saving plasma therapies continues to grow, we understand the challenges you face – from cost pressures to increasing regulations to managing your supply chain. Our comprehensive portfolio of integrated solutions is designed to support multiple facets of your operations, helping you achieve efficiencies and manage costs, while maintaining compliance and safety.

Committed to helping our customers in their missions to provide life-saving plasma therapies to patients around the world, Haemonetics always strives to deliver:

- Improved **donor safety and satisfaction**
- A reliable supply chain to help ensure **business continuity**
- Industry-leading **customer support and training**
- Innovative solutions to ensure **safe and efficient plasma collection**

For a list of worldwide office locations and contact information, visit www.haemonetics.com/officelocations

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Next Generation DMS™ — our new plasma management software solution

Next Generation DMS is the first in a series of truly integrated solutions designed to help plasma centers optimize collections. With its intuitive, web-based user interface and customizable features, this new software provides the flexibility required to meet the unique needs across your plasma collection network.

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Source plasma experts from North America and Europe convened for the annual PPTA Business Forum in Orlando, Fla. More than one hundred officials from PPTA member companies attended the event. This year’s Business Forum marked a departure from previous years in that it featured an extended agenda with deeper discussion topics and more concentrated content. The day began with the PPTA Source Board of Directors Chairman, Shinji Wada, who discussed the changing environment surrounding the plasma collection industry, noting that PPTA is approaching its twenty-five year anniversary.

As an introduction to the day’s agenda, attendees viewed clips from “Bad Blood,” a documentary that chronicles the tragic time during the 1980s among the hemophilia community. The general program began with keynote speaker, John Carlisle, who contrasted plasma collection practices in the present with those of twenty-five years ago and earlier. He noted that many in the audience were just beginning their careers at that time and now they hold positions as leaders within their organizations.

The audience then heard from two communications experts who gave their perspective on donor retention and recruitment in different regions. First, Vlasta Hakes (Grifols, Inc.) provided her thoughts on challenges in the United States, ending her talk with her belief that we should always make sure that our donors feel appreciated for their generous gift of time. Next, Aleksandr Fabian (CSL Plasma GmbH) contributed his observations on retention and recruitment issues in Europe, particularly for Germany. Overall, the approaches were similar in Europe and the U.S., although differences were inevitable due to legal and regulatory concerns and cultural variances.

Following this, in accordance with the PPTA Source division’s by-laws, the officers of the PPTA Source Board of Directors reported on the health of the PPTA Source division. Roger Brinser (BioLife Plasma Services/Shire) outlined the progress of PPTA Regulatory Policy Steering Committee’s work with U.S. Food and Drug Administration, the European Medicines Agency, and other regulatory bodies. Michael Deem (CSL Plasma) discussed the accomplishments of the International Quality Plasma Program (IQPP) Standards Committee. Beth Eacret (Grifols Plasma Operations) reviewed the work of the Source Industry Profile Committee. The Treasurer of the PPTA Source Board of Directors, David Morad (Southern Blood Services) provided an overview of the division’s finances. Finally, Stephan Walsemann, MD, Ph.D. (KEDPlasma GmbH) provided an update on the work of the European Plasma Alliance.

After a networking lunch, company leaders discussed best practices for viral marker control and correction using case studies. Charles Auger (Grifols Inc.) and Chuck Borders (BioLife Plasma Services/Shire) reviewed how their companies identify and implement strategies for keeping plasma centers in compliance with the IQPP Viral Marker Standard. Additionally, center manager, Aaron Rogerson (Biotest Pharmaceuticals), shared his experiences.

Next, the Forum addressed a topic that is increasingly of interest to members: cybersecurity and data integrity and how they relate to plasma collection. Ed Ferrara (CSL Behring) shared some of his expertise as the Chief Information Security Officer for his organization. He outlined some of the cybersecurity risks in our industry and discussed how they can be addressed. John Delacourt (PPTA) then reviewed the legal and regulatory risks surrounding data integrity, and examined possible mitigations. For a more detailed look at this discussion, see page 27.

The 2016 Business Forum concluded with a presentation of the annual Robert W. Reilly Leadership Award to Mr. John Carlisle. You can read more about this year’s award on page 28.
PPTA's Business Forum Delves into Cybersecurity Issues

PPTA’s 2016 Business Forum hosted a panel titled, “Cybersecurity and Data Integrity in Plasma Collection,” which provided an overview of cybersecurity risks, including big data, its implications for the plasma protein therapies industry, and the risks it poses. Additionally, the panel discussed the regulatory and legal risks, including potential mitigations, with regard to data integrity for the plasma protein therapeutics industry.

The panel presenters were Ed Ferrara—Chief Information Security Officer for CSL Behring and John Delacourt—PPTA’s Vice President for Legal Affairs and Global Operations. The panel was moderated by Sonia Balboni, PPTA’s Senior Manager for Source and Standards.

CYBERSECURITY

Ed Ferrara provided the audience with a quick overview of cybersecurity risks, with special attention to big data, its definition, the opportunities it can provide, the signals it broadcasts, and some of the technology used to collect and manipulate data. To give the audience an idea of how large “big data” actually is, Ferrara noted that Facebook alone has 1.3 billion users and collects 600 terabytes of data everyday, which is used to model behavior.

For the plasma protein therapeutics industry, Ferrara noted, big data can be used to improve decision-making about everything from the location of donor centers to donor behavior to marketing campaigns. Using big data can allow the industry to create truly customer-centric profiles to better anticipate their needs.

For all the benefits, Ferrara said, big data also carries risks. The collection and storage of large amounts of data is a target for hackers looking to sell information. The average cost of dealing with a data breach for a company is four million dollars. Ferrara next posed a series of questions that companies should be asking to ensure that their data is secure, including who has access to the data, where is it physically stored, and how is it protected from internal and external attacks.

There are, however, ways to mitigate this risk, stated Ferrara. A company should have robust policies, standards, and procedures in place that address what Ferrara terms the key pillars of big data security: authentication, audit, architecture, and authorization.

Because of the big potential benefits and the big potential risks, plasma companies need to look at big data from both the opportunity and risk perspective—developing appropriate security controls to maximize the benefit and minimize the risk.

Ferrara acknowledged that cybersecurity risks for plasma collectors do not apply only to big data. He noted that companies with smaller collection operations face cybersecurity risks as well. Although these can be just as daunting for a smaller company, they can also be mitigated with appropriate measures.

DATA INTEGRITY

Data integrity, John Delacourt explained, is a different but related issue to cybersecurity. Because the plasma protein therapeutics industry deals with therapies and medical devices, the Food and Drug Administration (FDA) provides guidance and regulations about paper and electronic data integrity. Data integrity, according to the FDA, refers to “the completeness, consistency, and accuracy of data,” said Delacourt. He noted that this is an important issue because data integrity is key to preserving data needed to conduct business, to produce safe and effective products, and to limit litigation risk and meet the needs of regulators and auditors.

Delacourt believes that data integrity begins to overlap with more general cybersecurity when implementation comes into play. This is when issues such as data being attributable, accurate, and auditable must be taken into consideration, as should securing devices and systems against external threats, he said.

Delacourt noted that the FDA guidance does not apply to all company data—only to data relating to the safety, efficacy, and quality of drugs. In the plasma protein therapeutics industry, this means that compliance efforts directed at the FDA guidance should be focused on donor health and plasma quality metrics, Delacourt said. These constitute the largest volume and most complex sets of data. How collection centers generate, process, review, and report data should all be assessed with the principles of data integrity in mind.

Delacourt concluded by examining a recent court case in which the Federal Trade Commission sanctioned a company for not properly protecting its customers’ personal information.

The panel finished with a question and answer session engaging the audience. Questions included whether the amount of data a company stores increases or decreases risk, how to use technology to mitigate risk, and the use of anonymization of data to protect privacy.●
John Carlisle Recognized with the Robert W. Reilly Leadership Award

John Carlisle was presented with the Robert W. Reilly Leadership Award at PPTA’s 2016 Business Forum in Orlando, Fla., recognizing his leadership in the plasma protein therapies industry. For more than 40 years, Mr. Carlisle has been a leader in the industry with a focus on Source plasma and the management of plasma collection centers.

“When I look at the list of recipients of this award, to be included in that list is... amazing. I’ve had the opportunity to work in this industry for over 40 years. I’ve seen a lot of change and I feel like I’ve been able to contribute to that change. Thank you,” said Mr. Carlisle.

He began his career with Hyland Laboratories (previously Baxter Bioscience, now a division of Shire) and recently retired from ViroPharma Incorporated as the Vice President, Plasma Operations. There, he had overall responsibility for ViroPharma’s U.S. plasma operations, with emphasis on the management of the plasma supply to support the manufacture of Cinryze (C1 esterase inhibitor).

Mr. Carlisle is a past member of the American Blood Resources Association Board of Directors and served as treasurer. He was one of the early supporters of the International Quality Plasma Program and other PPTA regulatory and quality initiatives. Because of Mr. Carlisle’s extensive industry experience, he has often been a speaker and panelist at PPTA Plasma Protein Forums and Business Forums.

The Robert W. Reilly Leadership Award recognizes an individual for their valuable contributions, achievements, and leadership on behalf of the source plasma collection industry.

For more than 40 years, Mr. Carlisle has been a leader in the industry with a focus on Source plasma and the management of plasma collection centers.
PPTA’s Global Regulatory Policy team advocates for regulatory and quality policies that reflect the special nature of plasma protein therapies and promotes a harmonized approach globally. Focusing on plasma-specific topics, the team addresses a broad range of regulatory issues—from donor eligibility and donation testing to final therapy requirements for clinical trials, chemistry, manufacturing and controls, and release specifications. The team promotes the image of the industry in terms of safety, quality, and scientific innovation and creates awareness of related industry initiatives.

The team represents PPTA before regional regulatory and health authorities and its day-to-day work includes identifying and prioritizing key regulatory policy issues; advocating for rational policies that impact industry; monitoring, assessing, and guiding development of new and potential regulatory policies; and developing regulatory initiatives and alternatives for advancing stakeholders’ interests. In the United States, interactions are primarily with the Center for Biologics Evaluation and Research (CBER), the Food and Drug Administration (FDA), and the Department of Health and Human Services; in the European Union (EU), interactions are with the European Medicines Agency (EMA), National Competent Authorities (NCAs) of EU Member States (MS), and the Council of Europe (CoE) European Directorate for the Quality of Medicines (EDQM). Globally, the team interacts with the World Health Organization (WHO). The team works within a matrix with other PPTA divisions regarding complex and multifaceted issues. When beneficial to members, the team liaises or collaborates with other industry trade groups, such as the American Association of Blood Banks, Pharmaceutical Research and Manufacturers of America, and the European Federation of Pharmaceutical Industries and Associations.

PPTA regulatory activities are driven by monitoring the U.S. regulatory environment (CBER), the EU regulatory environment (EMA, MS NCAs, EDQM, the European Commission (EC)), the WHO and other stakeholders, and by direction from Association Boards of Directors, including the Global, North American, Source, and European Boards. PPTA regulatory activities are coordinated by the Regulatory Policy & Compliance Steering Committee (RPSC) in North America and the Regulatory Affairs Steering Committee in Europe, as well as the Global Pathogen Safety Steering Committee. Additional input comes from committees, sub-committees, and task forces of industry subject-matter experts that address specific issues. Those groups include: the Medical Policy Committee, RPSC Regulatory Workshop Sub-Committee, the RPSC CLIA Waiver Sub-Committee, the Plasma Master File Group (EU), and the Quality & Operations Task Force (EU).

In addition to interactions with regulators on an as-needed basis, PPTA regulatory staff meet with relevant healthcare authorities at least once a year to identify priorities and topics important for the continued success of industry and health of patients, to share views and concerns, and work toward mutually beneficial solutions. In the U.S., PPTA and FDA hold a Liaison Meeting with Association members every fall; in the EU, PPTA staff meet with representatives from EMA’s Blood Products Working Party and Biologics Working Party.
October 9-15 marked the fourth annual International Plasma Awareness Week (IPAW), which was celebrated globally by PPTA member companies and patient groups. Each year, PPTA and the Source Industry Profile Committee (SIPC) strategize on ways to continue to raise global awareness about plasma donation and plasma protein therapies, as well as recognize the contributions of plasma donors. The SIPC is comprised of communications experts from Source member companies. Membership on the Committee is balanced with company representatives from Europe and the United States. The cross-continental makeup of the Committee has allowed for an international effort to strengthen the Source industry profile and continually enhance IPAW.

PPTA member companies planned a variety of activities at plasma collection facilities in the U.S. and Europe, including patient visits to local centers, donor appreciation events and themed days, as well as support for patient group fundraising. In addition, patient organizations, representing individuals who rely on plasma protein therapies that treat rare diseases, have pledged their support.

International Plasma Awareness Week Proclaimed Across the United States

PPTA is proud to have the support of Congresswoman Doris Matsui (D-CA) for IPAW 2016. She is a longtime champion of rare disease and access to care issues, with an important focus on patients with primary immunodeficiency diseases (PID). Rep. Matsui co-authored and advocated for the passage of the Medicare IVIG Access Act, which allows PID patients to receive life-saving therapies in their homes.

Congresswoman Matsui’s statement in recognition of IPAW is important in creating awareness of the life-saving gift of plasma protein therapies. A copy of the statement can be found on the PPTA website (www.pptaglobal.org/meetings-events/international-plasma-awareness-week/partners).

With the help of member companies and resident supporters, PPTA staff contacted the offices of state governors to advocate for and raise awareness about plasma donation and plasma protein therapies. PPTA received 47 proclamations from 46 states and the District of Columbia which honor the contributions of plasma collection centers and health, committed donors, in addition to recognizing patients who rely on these life-saving therapies.

Thank You To The Following
IPAW-Supporting Patient Organizations:
PPTA WOULD LIKE TO THANK THE FOLLOWING GOVERNORS AND MAYOR FOR DECLARING PLASMA AWARENESS WEEK IN THEIR STATES:

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Cristiano Ronaldo Helps Bring Awareness to Blood and Plasma Donation

PPTA has partnered with Abbott on the BE THE 1™ Campaign, featuring international football star, Cristiano Ronaldo.

WHAT IS THE BE THE 1™ CAMPAIGN?
The BE THE 1™ campaign is a global multimedia campaign to encourage and grow blood and plasma donations by simplifying and demystifying the experience and subsequently spark a global social movement using global ambassadors. Cristiano Ronaldo is the global ambassador. The BE THE 1™ campaign aims to inspire young people around the world to become life-long blood and plasma donors to potentially save lives.

HOW DID CRISTIANO RONALDO GET INVOLVED IN DONATING?
Cristiano Ronaldo is the world’s most recognized football player, he has strong proven appeal with young donors and he is the most popular social media athlete, with more than 117 million Facebook fans. Yet, in addition to being incredibly popular with world youth, Cristiano Ronaldo is a genuine, dedicated, and passionate blood donor. We would not have selected Cristiano if he did not have a genuine passion for blood donation. Cristiano Ronaldo is a champion for blood donation. He personally donates regularly and understands the importance of blood donation in keeping people healthy and allowing them to live their fullest lives. Cristiano first gave blood when he was 24 years old after seeing his teammate struggle to get bone marrow donations for his son. Since then, he has continued to donate regularly. By partnering with Cristiano Ronaldo, we are creating a new way to reach young donors and to inspire them to donate blood and plasma.

WHAT IS THE GOAL OF THE BE THE 1™ CAMPAIGN?
Around the world, developed and developing countries are mutually experiencing a decline of new, young blood and plasma donors, driven by a growing apathy among the youngest generation of eligible donors. While blood and plasma services around the world invest independently in donation drives, there is no connective, sustainable communication strategy that unites these programs. BE THE 1™ is a new global blood and plasma donor movement created to inspire and motivate young people to become lifetime donors.

How Do Donors Get Involved?

1. **GO TO:** www.BETHE1DONOR.com.
2. **JOIN THE MOVEMENT:** Post your commitment to donate, and tag your friends, directly to their Facebook page (https://www.facebook.com/bethe1donor)
3. **DONATE PLASMA NOW:** Go to: www.donatingplasma.org and find participating local donation centers around the world.
4. **VIEW AND SHARE:** Share the campaign call to actions with your friends and family on social media.

If your company is interested in participating in the BE THE 1™ campaign, please contact PPTA’s Director of Global Communications, Bill Murray. You can reach him at +1-443-458-4669 or wmurray@pptaglobal.org.
Join us in Prague for the 2017 International Plasma Protein Congress

The International Plasma Protein Congress (IPPC) provides an important platform to explore different aspects related to the provision of safe and effective plasma proteins therapies for the patients, who depend on these life-saving treatments. This event provides a unique opportunity for patients, physicians, policymakers, regulators, and industry to come together and discuss vital topics. The IPPC plays a major role in enhancing efforts to improve access to plasma protein therapies for patients.

The agenda will include eight sessions. The following key areas will be covered and discussed interactively during the Congress:

- **Primary Immunodeficiency (PID) Care**: A dedicated session will address the optimal organization of PID care and also projects to enhance diagnosis and treatment.
- **International developments**: Throughout the Congress, a selection of national and international changes will be discussed.
- **Anti-D treatment**: There will be an entire session addressing the current situation of the hemolytic disease affecting newborns and how to provide access to treatment for all patients.
- **Access to care**: Access issues at both the national and regional level will be discussed and reviewed during the Congress with top speakers and patient group representatives.
- For more information on the 2017 IPPC, visit the PPTA website: [www.pptaglobal.org](http://www.pptaglobal.org).

In 2017, PPTA will be celebrating 25 years of saving and improving lives. In honor of this, we will be hosting a black-tie gala in Washington, D.C. on the evening of June 13, 2017 at the Mellon Auditorium in Washington, D.C. You will be able to register for this event in conjunction with the 2017 Plasma Protein Forum on a first-come, first-served basis since there are limited seats available. Watch the PPTA website for more information in the coming months.
One Company. Two Solutions.

Haematologic Technologies Inc.

Products and services for hemostasis research
- Purified blood coagulation factors
- Antibodies
- Factor deficient plasmas
- 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.

Haemtech Biopharma Services

Testing and services for protein biotherapeutics
- Thrombin generation assays
- Factor Xla detection in IVIG
- Immunogenicity testing
- Stability & release testing
- Host cell protein mitigation

HBS is a cGMP-certified, QC testing laboratory that specializes in providing services for protein biotherapeutics manufacturers from drug inception through market release.

haemtech.com

haemtechbiopharma.com
# Upcoming Events

## February
- **8 – 9**  
  3rd International Rare Diseases Research Consortium Conference  
  *Paris, France*
- **15 – 18**  
  61st Annual Meeting of the Society of Thrombosis and Hemostasis Research  
  *Basel, Switzerland*
- **22 – 23**  
  Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies (Hosted by EURORDIS)  
  *Brussels, Belgium*
- **28**  
  Rare Disease Day

## March
- **5 – 8**  
  Keystone Symposia – Rare and Undiagnosed Diseases: Discovery and Models of Precision Therapy  
  *Boston, United States*
- **14 – 15**  
  International Plasma Protein Congress (IPPC)  
  *Prague, Czech Republic*
- **31 – Apr 4**  
  5th African Society for Immunodeficiencies (ASID) Biannual Congress

## April
- **20 – 21**  
  Spanish Hematology Meeting  
  *Buenos Aires, Argentina*

## May
- **16 – 17**  
  International Plasma Fractionation Association (IPFA)/Paul-Ehrlich-Institut (PEI) 24th International Workshop on Surveillance and Screening of Blood-borne Pathogens  
  *Zagreb, Croatia*

## June
- **13 – 14**  
  Plasma Protein Forum  
  *Washington, D.C., United States*
- **22 – 25**  
  European Hematology Association (EHA) 2017 Congress  
  *Barcelona, Spain*

## July
- **8 – 13**  
  XXVIth Congress of the International Society on Thrombosis and Haemostasis (ISTH)  
  *Berlin, Germany*

## September
- **11 – 12**  
  IPFA/Blood Centres of America (BCA) 3rd Global Symposium on The Future for Blood and Plasma Donations  
  *Atlanta, United States*
- **11 – 14**  
  European Society for Immunodeficiencies (ESID): Autoimmunity & Inflammation in PID; Beyond the Paradox  
  *Edinburgh, United Kingdom*

## October
- **8 – 14**  
  International Plasma Awareness Week (IPAW)

## November
- **8 – 10**  
  International Primary Immunodeficiencies Congress (IPIC): Focus on Clinical Care and Diagnosis  
  *Dubai, United Arab Emirates*
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<tr>
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Join Cristiano Ronaldo and Abbott to incorporate the BE THE 1™ program into your donor recruitment campaign. Please contact your local Abbott representative for details. BE THE 1™.

Sign up to donate at BeThe1Donor.com
Aurora is the automated system that streamlines plasma collection, producing virtually cell-free plasma and providing an improved experience for both operators and donors.

- Intuitive touch screen display
- Aurora data management provides easy, accurate data collection, remote procedure setup and paperless documentation
- Designed to help improve plasma center efficiency

Find out more today at [www.fresenius-kabi.us/aurora](http://www.fresenius-kabi.us/aurora)