An Interview with PPTA’s President & CEO

Policymakers Recognize Importance of Non-Interchangeability

Proposals to Lower Drug Spending: What Could They Mean for Patient Access to PPTs?

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*THE SOURCE* is available as a PDF file at www.pptaglobal.org.

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

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As a college student at the University of Wisconsin, Madison, I supported myself by working at the student union, riding my bike in pre-dawn hours to open the commons desk in time to hand people their subscriptions to The New York Times. I also had weekend shifts as a building manager, which afforded me the opportunity to do cool things, like pay the bands after they finished playing in the Ratskellar or greet visiting speakers, including the Dalai Lama. Of course, it also included less pleasant tasks like unclogging drains in the basement kitchen or dealing with students who had been overserved. Looking back, the lessons I learned at that job were as valuable to my development as was my formal education.

My income, however, was not enough. There is a plasma center near the student union and I became a regular donor to help pay my bills. Eventually, I began reading the literature and talking to the phlebotomists about how plasma was used, and I remember being surprised to learn that plasma is used to make lifesaving medicines for life-threatening diseases. That revelation changed the way I looked at my donations. Donating became about more than just earning extra money to pay for ramen noodles — it was about contributing to saving lives. I was making a contribution.

Recently, I was privileged to visit a plasma center in Maryland. As we pulled up to the facility, I was struck by the eye-popping signage covering the windows, all promoting the benefits of plasma and featuring those who benefit from plasma protein therapies. On touring the center, I saw a familiar process, albeit more high-tech than in my donor days. The center manager explained the thorough process of first-time donors, including the myriad health and safety measures that evaluate the donor’s health and ensure that they understand each step. I was also taken through the journey of plasma, from collection, processing, and storage at the center. The quality assurance measures are thorough and followed precisely. Most moving, however, was having the chance to talk with a donor who’s a regular at the center. I asked why he donates plasma, and he said, without hesitation, “Because I like to know that I’m helping someone who needs it.” I thanked him, sincerely.

Donors make lifesaving therapies possible. On average, it takes 130 plasma donations to treat one adult patient with a primary immunodeficiency for one year. Likewise, 900 donations are required to treat someone with alpha-1 antitrypsin deficiency for a year, while treating someone with hemophilia for a year can take as many as 1,200 plasma donations.

Access to plasma-derived therapies has been lifesaving for countless people. For individuals living with common variable immunodeficiency (CVID), the 10-year survival rate has increased from 37 percent in the early 1970s to more than 90 percent today because of plasma protein therapies.

In this issue, you’ll find rich content on patient access, testimonials, stakeholder priorities, an advocacy roadmap, and a profile on the new leader of the International Patient Organisation for Primary Immunodeficiencies (IPOPI). You’ll also find coverage of decisions impacting patients and our industry, as well as a piece on how PPTA interacts with regulators.

I am proud of my history of plasma donation, and I am truly privileged, in my new role as President and CEO, to be serving the interests of those who develop and deliver vital medicines to people living with rare, chronic, and life-threatening diseases.
What most excites you as you take on this new role?
What most excites me is the ability to share the success stories behind our member companies’ lifesaving therapies. As PPTA, we are positioned to inform policymakers, health care providers, and other critical decision-makers toward a common goal of ensuring patients have access to essential medicines, particularly individuals living with rare diseases.

What do you feel are the greatest opportunities facing PPTA and the plasma protein therapeutics industry in the next five years? Ten years?
The greatest opportunity facing PPTA in the short term is working with partner stakeholders worldwide to advance the shared goal of increasing opportunities for plasma collection and the continued development of innovative therapies to treat rare diseases. Together, we have such a unique story to share! For individuals living with primary immunodeficiency, alpha-one antitrypsin deficiency, hereditary angioedema, neurological diseases, and various bleeding disorders, plasma donors are essential to meeting the clinical needs of patients. I’ve recently had the privilege to interact with patient organization representatives in the United States and Europe. I was deeply affected by the maturity of these groups and their ability to use their voice with policymakers to express the need for plasma and lifesaving medicines for their constituents. This good news story about the value and high impact of PPTs needs to be told to decision-makers around the world, and it must be heard.

I am excited to take “How Is Your Day?” — the awareness campaign PPTA launched last year — to the next level and work with our stakeholders to build effective educational tools in our shared effort to ensure patients have access to life-sustaining therapies. Today, patients are living longer, more fruitful lives. I want tomorrow to be even brighter for patients, and I am committed to PPTA’s steadfast pledge to this goal. Just as important, the “How Is Your Day?” campaign offers us an incredible opportunity to celebrate and thank source plasma donors for the gift they provide every time they donate. Quite literally, plasma donors save lives, and this campaign has taken great steps to recognize donors. I look forward to building that momentum.

Relatedly, what do you anticipate being some of our challenges?
A host of challenges stand in our path. Reimbursement policies are and will continue to be obstacles to ensuring access to essential medicines for our patients worldwide. PPTA plays a pivotal role in communicating the value of our therapies so that payers — both public and private — understand the benefit of plasma protein therapies for the patients who need them. We also face the challenge of counterparts around the world who don’t believe that compensated and uncompensated systems can coexist. We must find a way to move together into the future, with complementary collection systems, if we are to meet the needs of the patients we serve. We should also be concerned about sentiments of national self-sufficiency that are percolating in countries around the world. It is both unrealistic and unnecessarily limiting to put borders around the world’s supply of plasma. Disease, after all, recognizes no borders. Finally, we must remain steadfastly committed to the high voluntary standards of safety put in place by our industry and regulators over the past three decades. Our track record of safety during that period reflects the increased level of quality our members maintain.

What characteristics do you most admire in others?
I admire resilience in others. When I meet people who are faced with challenges — health related, financial, spiritual, or emotional — and they’re living an abundant life in defiance of those obstacles, I’m inspired. I have a child with a learning disability. She has faced teasing, misunderstanding, and even intolerance. In spite of that, she soldiers on every day with a gloriously positive attitude and refuses to let her learning disability limit her horizons.

Tell us something about yourself that might surprise us.
Two things — I was a plasma donor throughout my college years, and I’m very proud of that contribution. And I’m an NFL owner. I own two shares in the Green Bay Packers, my hometown football team and the only NFL team wholly owned by its fans!
Meet the IPOPI Chair: Martine Pergent

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

Martine Pergent assumed the role of Chair of the International Patient Organisation for Primary Immunodeficiencies (IPOPI) this year, following several years of involvement within the primary immunodeficiency disease (PID) patient community. Jose Drabwell will remain on IPOPI’s Executive Committee and will use her position to continue supporting the PID patient community. We reached out to Ms. Pergent to understand her outlook on IPOPI’s future and its role as a leading advocate for patients living with primary immunodeficiencies.

What is your vision for IPOPI?

IPOPI is dedicated to improving PID patients’ lives, worldwide. In order to do so, we have made it our mission to improve awareness, access to early diagnosis, and optimal treatments for PID patients around the globe. This mission statement is a fabulous ambition when it comes to patients with rare life-threatening conditions, most of whom will need lifelong treatment. It is important in a globalized world, in particular for rare diseases, to have a strong global patient representation that can support the regional and national efforts of its members and to be a strong representative of its community to provide proposals, recommendations, leadership, support or at times to act as a facilitator.

Our efforts and work are aimed at making patients stronger partners in their health care environment so they really benefit from patient-centered approaches, especially those that follow the trend toward personalization in both medicine and pharmacopoeia. In addition, PID patients who live with chronic disorders have become experts on their conditions and how to live with them: In this perspective, we want to ensure that patients are not only listened to, but heard.
What are the most important issues facing the PID community?

Diagnosis remains a huge issue. Most patients worldwide are undiagnosed, even in countries with reliable health systems. Then access to treatment is also crucial, especially because different options are available, from prophylaxis to palliative or to curative ones. Each of them has its own challenges. Ensuring access to immunoglobulin substitutive therapy (Ig therapy) is also among our top priorities. There is an increasing threat on the access to these lifesaving medicines for multiple reasons. One of them, not the least, is the increasing demand for Ig therapies. However, the world supply relies on a handful of countries that have proven their efficacy in collecting plasma, such including, of course, the United States, but also Germany, the Czech Republic, Austria, and Hungary. In many countries, the collection growth is insufficient, and I strongly think that stakeholders need to change their way of thinking and focus on the safety of patients from a supply perspective in addition to donor safety, for whom we are very grateful, with the ultimate goal that patients have access to their lifesaving treatment.

Of course, beyond this, countries should put in place health systems that allow patients to access the best available treatment option for them to lead normal lives with an optimal quality of life.

The PID field is fascinating, and thanks to successful research, we’re gaining an amazing body of knowledge more quickly than ever. From the patients’ perspective, the question is how will they benefit from the new research in their daily life?

Diagnosis and treatments are there, but the key word remains access: research, clinical knowledge, diagnostics, availability, affordability, and quality of life. There you touch upon a large range of stakeholders who need to cooperate for the sake of patients’ health. This is key to ensuring constant progress of knowledge and research, which translates into a better life for patients.

Developing solutions is critical. Can you highlight key IPOPI initiatives that are making a difference to improve patients’ quality of life?

IPOPI has achieved a lot in the past decade, thanks to its efficient and wonderful staff, sustaining continuous efforts to develop patient leaders who can act at national or regional level. Moreover, I have to say that I am very proud of my board members, each of whom has such a strong commitment to his or her own region. IPOPI works hard to launch new national member organizations, supporting them in their awareness, advocacy, and social outreach. As a case in point, just have a look at what has happened in Africa and Latin America. When it comes to Asia, our Bob LeBien Programme has allowed IPOPI to make outstanding progress with new patient representatives committed to their communities and working hand-in-hand with their physicians to obtain better access to diagnosis, treatment, and reimbursement.

IPOPI also works at the regional level, including Europe, to foster policies that take into account PID patients and interests. Through its European Policy Forums, IPOPI has raised the patient’s voice among members of Parliament and set up frames of what should be gold/good practices when it comes to PIDs as a model for rare diseases. Our work on newborn screening for PIDs, and broadly for rare diseases, is also telling.

IPOPI works as a global and regional partner among many different stakeholder platforms and fora, including physicians and health care professionals, industry, decision-makers, institutions and agencies, as well as other patient groups. To illustrate this, I will mention the International Primary Immunodeficiencies Congress, our clinical conference that has been so successful in spreading the knowledge and experience on clinical care that are so vital for patients and important for physicians, including the youngest who commit to work in this amazing but complex field.

Could you share with our readers what motivates and inspires you?

For me everything began by chance: Close friends of ours were having a child with PID, more than 20 years ago, in France. As a small group, we decided to set up a patient organization under the advice of Prof. Alain Fischer. Since then, I have done nothing else except learn, listen to people with unbelievable experiences, set out projects, read, write, and meet with fantastic people. Then came IPOPI, with its global scale and its fantastic evolution. The PID community is truly a special one, full of amazing people! Being a volunteer can be demanding when you really commit, but it is well worth doing. When I look over my shoulder at the 20 past years, I can see how life has dramatically improved for PID patients, how today lives of people who would have died yesterday are being saved. I feel proud and rewarded to have been a small part of this! And then, when looking toward the future, I can see all the improvements that remain to be implemented, patients in some countries still have such difficult lives. I also know that nothing is ever won forever and that we need to monitor carefully and continue advocating, supporting, and cooperating as an active, even proactive, stakeholder. Is this not enough to be thrilled? First, I feel privileged to be part of a great organization like IPOPI, and now to have the honor of serving as its chair. Second, I am fortunate to be part of such a noble and irreplaceable mission with many successes and so many challenges left to turn into new successes! •
In January, PPTA, along with other stakeholders involved in plasma collection and manufacturing, participated in the annual stakeholder conference of the Platform of Plasma Protein Users (PLUS) in Estoril, Portugal. PLUS is the umbrella organization representing a number of patient associations for rare or ultra-rare bleeding disorders, including the Alpha-1 Europe Federation, the European Haemophilia Consortium, the Guillain-Barré Syndrome/Chronic Inflammatory Demyelinating Polyneuropathy Foundation International, Multifocal Motor Neuropathy Foundation International, the International Patient Organization for C1 Inhibitor Deficiencies (HAEi), the International Patient Organisation for Primary Immunodeficiencies, the Immune Thrombocytopenia Support Association, and the World Federation of Hemophilia. Topics discussed included new and emerging threats to plasma, plasma supply, and patients. Presentations addressed plasma collection, as well as availability of plasma-derived medicinal products (PDMPs) in Europe, including the potential impact of Brexit on access to medicines for patients in the United Kingdom and in the European Union. One of the key concerns identified in the meeting was the need to collect more plasma in Europe and to educate stakeholders on the importance and value of plasma donation. The meeting concluded with participants agreeing on a range of consensus points and strategies to encourage blood and plasma donations in Europe, including:

- Recognize PDMPs as lifesaving medicines and the dependency of patients on a stable and safe supply, whilst maintaining the health and safety of patients and donors;
- Acknowledge global self-sufficiency as a goal overarching any national or regional efforts to collect more plasma;
- Involve patients and donors in the policy- and decision-making process on multiple levels;
- Acknowledge the crucial role of increasing plasmapheresis for increasing PDMP availability in Europe; and
- Further emphasize the differences between blood and plasma, as well as between labile blood products and PDMPs, to adequately address targeted collection and distribution programs.

These consensus points were presented at the meeting of the European Directorate for the Quality of Medicines & HealthCare on Plasma Supply Management in Strasbourg at the end of January.
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On Aug. 7, 2018, the United Kingdom’s (UK) Department of Health and Social Care (DHSC) published a proposed amendment to the Statutory Scheme to increase the amount of payment paid by manufacturers to the government on the net sales income received from the supply of medicines. The proposal would remove the current exemption of albumin and immunoglobulin sales. The purpose of the proposal is to set a payment percentage that is calculated to limit the expected growth of net branded health service medicines sales.

PPTA met with all manufacturers of plasma protein therapies (PPTA members and non-members) providing these treatments in the UK to draft a letter as a joint answer to the consultation regarding the Statutory Scheme alerting the authorities about the unintended consequences these measures could easily create, putting fragile patient communities at risk. PPTA stated the proposed annual payment percentage increase would particularly impact our sector and would endanger access to care for patients whose lives depend on plasma protein therapies. PPTA referred to the uniqueness of plasma-derived medicinal products (PDMPs) with regard to human plasma as a starting material, the long and complex manufacturing process of up to 12 months, the non-interchangeability of PDMPs due to the variability of manufacturing of particular products, and to the unique cost structure bringing pressure on the economics of fractionation.

PPTA’s main concerns and rationale for the request to exempt all plasma protein therapies from the proposed payment percentages were discussed in a follow-up meeting with representatives of the DHSC and the National Health Service. On Dec. 3, 2018, the DHSC published the consultation response to comments and announced the final decision of the Statutory Scheme, which was effective as of Jan. 1, 2019. The final regulation implements a new payment scheme that is
somewhat less than what was initially proposed. The payment percentage will increase from the current 7.8 percent to 9.9 percent in 2019 and subsequently to **14.7 percent** in 2020 and **20.5 percent** in 2021. It is estimated that these are the payment schemes necessary to limit the nominal annual growth rate to 1.1 percent from the expected 2018 baseline of relevant sales. Despite PPTA's best efforts, plasma protein therapies were not exempt from the final rule.

According to the consultation response, the DHSC decided not to exempt plasma protein therapies because it believes an exemption is inadequate to mitigate the risks raised by PPTA. Instead, they have decided on a mechanism that will provide flexibility to address any issues that may arise concerning plasma protein therapies. However, in the consultation response, the DHSC recognized the uniqueness of plasma protein therapies, by stating: “We acknowledge that blood plasma products are not easily substitutable or necessarily interchangeable. To support patient access to effective medicines, supply of a range of comparator blood products does need to be secured.”

This confirms that they agree with the medical providers who state that plasma protein therapies are not interchangeable. In fact, the UK's Department of Health Clinical Guidelines for Immunoglobulin Use states that immunoglobulin products cannot be used interchangeably. This position is similar to the one found in the International Patient Organisation for Primary Immunodeficiencies' (IPOPI) Principles of Care, which state, “No single IG product or administration method is suitable for all PID [primary immunodeficiency disease] patients; it is crucial to ensure optimal treatment is provided on an individualised basis. Patient health and personal preferences must be considered in treatment decision-making.”

For patient advocates, the language found in the consultation response is useful when advocating that patients need access to a range of plasma protein therapies. In addition, the consultation response may be helpful if the new scheme results in patients being asked to change to a therapy that is not the most medically appropriate for that individual. In these situations, citing the consultation response acknowledging plasma protein therapies are not “easily substitutable or necessarily interchangeable” in conjunction with the guidelines from the UK Department of Health and IPOPI would send a strong message.

References:

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On March 29, 2017, the United Kingdom (UK) notified the European Council of its intention to withdraw from the European Union (EU). On March 30, 2019, the UK will cease to be a member of the EU — a process also known as “Brexit.” One of the many consequences of Brexit is the physical relocation of the EU’s medicines regulatory authority, the European Medicines Agency (EMA), from London, UK, to Amsterdam, the Netherlands.

This is a major move for one of the most important agencies of the EU: EMA is responsible for the scientific evaluation of many medicines in the EU and the European Economic Area (EEA), serving a market of about 500 million people. Since its inception in 1995, the EMA has been based in London.

The planned relocation carries significant consequences for EMA’s day-to-day activities. Apart from the practical difficulties of relocating the agency, a key concern is the relocation of 890 full-time staff and minimizing staff loss, which could be as high as 30 percent. According to EMA’s Brexit Preparedness Business Continuity Plan (BCP), a minimum of 65 percent staff retention is needed to ensure “core activities” related to pharmacovigilance, quality, safety, and supply of medicines are adequately maintained.

On Jan. 23, 2019, the EMA implemented phase 4 of its BCP, including the physical relocation from London to Amsterdam and restricting activities to those deemed highest priority until at least the end of June 2019. On Jan. 29, 2019, EMA officially closed its doors at its London headquarters, with the shutdown of all activities in London planned for March 1, 2019. EMA staff is expected to move gradually to Amsterdam and resume work as of March 15, 2019. The move to the agency’s permanent headquarters is planned for November 2019.

EMA stressed that the restriction or suspension of activities to mitigate staff loss and the practical consequences of moving will be temporary. However it remains to be seen if there are any longer-term effects on the agency’s operations.

References:
1. EU (Notification of Withdrawal) Bill. https://services.parliament.uk/bills/2016-17/europeneunionnotificationsofwithdrawal/documents.html
4. EU drugs agency to see 30% staff losses over Brexit move. 02 August 2018, updated 27 August 2018. https://www.euractiv.com/section/health-consumers/news/eu-drugs-agency-to-see-30-staff-losses-over-brexit-move/
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This roundtable supports PPTA’s ongoing efforts regarding the possible revision of the current EU Blood Directive and needs to be seen in the following context: After the launch of the EU Commission evaluation of the EU blood, tissues, and cells legislation, the revision of the EU Blood Directive 2002/98/EC has become a topical issue on the European level. The publication date of the final commission evaluation report is expected to be published before Summer 2019.

PPTA has participated in several EU stakeholder consultations and bilateral meetings to educate policymakers about the plasma sector and the growing clinical need for plasma-derived medicinal products (PDMPs). The roundtable in the European Parliament took place on Feb. 21, 2019, in collaboration with Members of the EU Parliament. Representatives from patient organizations, health care professionals, national and European policymakers, and industry representatives participated. PPTA remains a trusted advisor in any efforts to change the European Blood Directive and has offered a well-vetted and thoughtful proposal to improve the European Blood Directive in the event that it is opened for reconsideration.
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WORKSHOP
Since the 1960s, the U.S. Food and Drug Administration (FDA) has required the potency of each IgG product lot to be tested for antibody specificities against the measles virus, poliovirus, and diphtheria to ensure functionality and lot-to-lot consistency. More than a half century has passed since this regulation was put in place, and a few issues have become apparent: 1) declining levels of measles-neutralizing antibodies in IgG lots related to donor vaccination; 2) the impact of approaching total eradication of poliovirus infections; 3) the relevance of the current potency tests to the clinical outcomes in PI patients on IgG-RT and whether modern and more appropriate potency tests should be considered in the future. In November 2017, a workshop titled “Immune Globulin Potency in the 21st Century” was
organized through the collaborative efforts of the FDA, PPTA, the National Institute of Allergy and Infectious Diseases, and the Immune Deficiency Foundation (IDF). This workshop was an important event embraced by IgG manufacturers, regulators, the patient community, physicians, and researchers from governmental institutions and academia. Presentations at this event were recorded with support from the IDF, and transcripts are available on the FDA website.1

A SUPPLEMENT TO TRANSFUSION
After the workshop, the FDA and PPTA collectively worked with speakers to publish scientific work relevant to the workshop in a peer-reviewed journal (published December 2018). Our thanks go to Richard Kaufman, M.D., (the editor of Transfusion), who accepted our proposal and to the publisher and staff who worked tirelessly on making sure the supplement was published, “A Supplement to Transfusion,” Volume 58, Issue S3 and made accessible to readers by Open Access (financial support provided by the FDA and PPTA).2 In the supplement, Dorothy Scott, M.D., from the FDA Center for Biologics Evaluation and Research (CBER) offered a comprehensive assessment of all aspects of the workshop. IDF President John G Boyle also provided many valuable comments.

The supplement covers several topics, one of which is related to the potency testing of IgG products as a measure of their functionality against the measles virus. This issue has relevance for IgG manufacturers as well as individuals with primary humoral immunodeficiency who rely on lifesaving and life-long IgG-RT. Industry investigators showed that titers of measles-neutralizing antibodies in plasma donors steadily declined since the introduction of vaccination against measles infection. They also established that lowering the lot release specification for measles neutralizing antibody from 0.48x to 0.30x CBER Standard lot 176 (16.5 percent) for IgG products would still be sufficient to protect patients against measles.

Another topic addresses new challenges the industry is facing in view of the global eradication of wild-type poliovirus through the World Health Organization efforts. The main issue is related to addressing the highest level of safety measures in the laboratories handling the poliovirus. New developments in genetic engineering research offer a possible solution to the industry to address this concern while continuing to perform potency testing of IgG products for poliovirus-neutralizing antibodies. A few articles explore possibilities for new and more advanced potency assays, instead of those currently in place, that could be considered in the future as more appropriate for clinical outcomes in individuals with primary humoral immunodeficiency.

The editors’ goal would not have been achieved without the contribution of reviewers who were represented by industry investigators, regulators, and researchers from academia. PPTA believes this supplement will serve as a reference guide for future IgG products — and as a starting platform for discussions of appropriate potency tests relevant to clinical outcomes in individuals with primary antibody deficiencies.

LETTER
An important outcome of the workshop came in the form of an FDA decision to issue a recommendation “Letter to Immune Globulin (Human) Licensed Manufacturers: Option to Lower Lot Release Specification for Required Measles Antibody Potency Testing,” dated Nov. 5, 2018.3 This letter informs manufacturers that under 21CFR 640.104(b)(2), CBER sets the minimum specification for measles neutralizing antibody levels in Immune Globulin products as 0.36 x CBER Standard lot 176 (16.5 percent). It also advises adding labeling to the prescribing information that contains corresponding recommendations for dosing of patients with primary humoral immunodeficiency who have been exposed, or are likely to be exposed, to measles.

References:

SPRING 2019 | THE SOURCE
In January, PPTA staff participated in a three-day Symposium on Plasma Supply Management in Strasbourg, France, organized by the European Directorate for Quality of Medicines (EDQM).

The purpose of this scientific meeting was to collect evidence-based data supporting the revision of the 20th edition of the “Guide to the Preparation, Use, and Quality Assurance of Blood Components with Regard to Plasmapheresis.”

PPTA, which had also been part of the scientific organizational committee, was given the opportunity to present data and insight regarding the collection of plasma in the EU. PPTA’s role in assisting the EDQM address these important issues has been important in terms of representing the industry and helping to develop initiatives for global plasma sufficiency and access to finished products for patients. Specifically, with regard to the Association’s role on the agenda and at the meeting, PPTA staff and industry experts alike presented on the experiences of the private sector and donor motivation, the use of recovered plasma in Europe, technical recommendations for the evaluation of donor suitability, strategies on the protection of iron stores in plasma donors, data on donor adverse reactions, and the industry’s ongoing efforts in plasmavigilance.

It was the first time that stakeholders from patient groups and donor associations, as well as public and private sector collection establishments, came together to discuss the current issues in an open dialogue. This dialogue provided a more complete picture of the current situation. The industry was able to establish itself as a credible part of the discussion and an expert in the field of plasma collection with its long-term experience focusing on patient safety and donor health. Only a week after the “PLUS Consensus Principles on Strategies to encourage Blood and Plasma Donation in Europe” in Estoril, Portugal, the patients emphasized again that any policies aimed at increasing blood and plasma collection should ensure that it is both patient- and donor-centered with the goal of meeting clinical needs. They presented the newest Consensus Statement developed in Estoril and signed by many organizations.

It has been recognized that human plasma is a crucial component for the manufacturing of lifesaving therapies on which patients with rare and chronic diseases depend heavily. It was also recognized that the need for plasma has increased in recent years and there is a need to foster and support collection systems in Europe.

The outcomes of the symposium will be translated into general recommendations for the editorial committee of the guide. PPTA and the European Plasma Alliance will continue to be official stakeholders in the extended expert group and offered their availability for any additional input in the activities of the TS093 core working group. It was noted that the collaboration between the EDQM and the European Commission was intensified, which may have an impact on the potential revision of the European Blood Directive. Regarding the timeline, the draft of the 20th edition is expected to be published for public consultation in July 2019. The final text will then be approved for at the end of 2019.
Creating world-class customer experience through state-of-the-art testing services

Services
- Donor Screening
- Immunohematology Reference Laboratory
- Method Suitability and Sterility Testing
- Cellular Therapy Reference Laboratory
- Biological Raw Material Infectious Disease Testing
- Research and Development

About QualTex
- Continuity of service through multiple locations
- 24/7/365 laboratory and customer service operations
- IRL certified by the American Rare Donor Program
- FDA registered and CLIA certified
- ISO 9001:2015 Certified

Locations
SAN ANTONIO
ATLANTA

QualTex Laboratories are approved and/or accredited by multiple companies and health agencies worldwide.
Government spending on Centers for Medicare & Medicaid Services (CMS) programs (Medicaid, Children’s Health Insurance Program, and Medicare) accounts for 37 percent of the total national health expenditure and 41 percent of the total prescription drug expenditure in the United States. Some projections estimate these percentages will increase over the next decade as a result of enrollment growth due to the aging population, changes in projected income growth, and changes in program eligibility. In addition to this trend, all payers are grappling with how to budget for new high-cost, curative therapies. Often the solution for public payers is to propose laws or regulations that will curb prescription drug spending by reducing choice and utilization.

Much of the rhetoric about government spending on prescription drugs at the federal and state level surrounds a perceived lack of competition and/or negotiation. Accordingly, many proposed solutions exemplify increased competition and/or negotiation. For example, a recent federal proposal would introduce third-party vendors to negotiate prices with pharmaceutical manufacturers and sell them to providers. However, the proposal does not require vendors to make all therapies available, nor does it mandate that physicians must contract with enough vendors to make all therapies available. A failed state proposal would have established a closed formulary, meaning individuals would not have access to all therapies. Rather, manufacturers would have to compete for approval by the state.

These and other proposals attempt to assuage access concerns by saying therapies may be available after prior authorization or step therapy/fail first. These utilization
management techniques may be suitable for generics and interchangeable biologics, but they are not appropriate for non-interchangeable sole source biologics, such as plasma protein therapies. Access to all therapies is better achieved by having multiple competing manufacturers and products, a position affirmed by CMS Administrator Seema Verma who has stated: “This is yet another call to action for CMS to increase market competition and consumer choice within our programs to help control costs and ensure that our programs are available for future generations.”

Several patient advocacy organizations have conducted research projects to determine the effects of insurance issues on access to plasma protein therapies for individuals in their communities. Findings from the Hemophilia Federation of America’s Project CALLS show that approximately half of the patients who experience a coverage issue due to utilization management delay care. A recent survey from the Immune Deficiency Foundation finds that the primary reason individuals skip treatment is due to problems with health insurance. Delays in care for individuals who use plasma protein therapies can exacerbate medical issues and lead to more expensive hospitalizations and negative health outcomes.

These restrictions also ignore expert clinical recommendations that require access to all available brands to ensure quality care and positive health outcomes. Each plasma protein therapy is approved by the U.S. Food & Drug Administration for distinct clinical indications, and each individual responds to a plasma protein therapy differently. Approximately one-third of patients receiving plasma protein therapies will experience intolerance to a particular product, as plasma protein therapies are not broadly interchangeable. Physicians with expertise in plasma protein deficiencies must have the ability to select the most medically appropriate therapy for patients in their care. In its responses to such proposals, PPTA has urged policymakers to respect the expert medical guidelines put forth by stakeholder groups:

- “It is unacceptable to limit availability of augmentation therapy in any way and especially to a single product.” — Alpha-1 Foundation Medical and Scientific Advisory Committee Clinical Practice Guidelines
- “IVIG is not a generic drug and IVIG products are not interchangeable. A specific IVIG product needs to be matched to patient characteristics to insure patient safety.” — American Academy of Allergy Asthma & Immunology Principle #8

PPTA’s mission is to promote the availability of and access to safe and effective plasma protein therapeutics for all patients in the world. PPTA, its members, and stakeholders all advocate for access to providers, sites of care, and all brands of plasma protein therapies. As new proposals emerge, the Association will continue to emphasize the unique nature and value of plasma protein therapies.

References:
1. CMS National Health Expenditures 2017; CMS National Health Expenditure Projections 2017 – 2026
2. CMS-5528-ANPRM
4. The Hemophilia Federation of America Project CALLS (Creating Alternatives to Limiting and Lacking Services)
5. The Immune Deficiency Foundation National Health Insurance Surveys, 2014 - 2016

UTILIZATION MANAGEMENT

is a technique that insurers/payers use to control the products or services beneficiaries access. In terms of medicines: prior authorization is when beneficiaries must get approval from the insurer/payer before they can access their prescribed therapy. Step therapy or fail first policies require beneficiaries to try and fail on another product before being able to access their prescribed therapy. These requirements are often a form of non-medical switching or cost-motivated treatment changes and are generally opposed by PPTA, its member companies, and stakeholders.
"My name is Jennifer. I'm 31 and started donating plasma after I had children. While I was pregnant it was discovered that I was Rh negative, a condition in which the mother produces antibodies against the baby's red blood cells and requires a plasma protein treatment throughout pregnancy. Without the plasma protein treatment, I would never have been able to have children.

"I got involved in donating plasma because someone gave plasma for me. Without it, I would not have my kids.

"Donating plasma is my way of giving back to all the mothers who have the same condition I had, who could not have had their children unless someone donated. Being able to help others gives me a sense of purpose and meaning. I've been donating once a week for many years."

"I gave because someone gave for me."
## Upcoming Events

### April

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<tr>
<th>Date</th>
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<tr>
<td>1 – 30</td>
<td>National Primary Immunodeficiency Awareness Month</td>
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<td>4 – 7</td>
<td>Hemophilia Foundation of America (HFA) Annual Symposium</td>
<td>San Diego, California, United States</td>
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<td>10 – 12</td>
<td>World Orphan Drug Congress USA 2019</td>
<td>Oxon Hill, Maryland, United States</td>
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<td>17</td>
<td>World Hemophilia Day</td>
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<td>22 – 29</td>
<td>World Primary Immunodeficiencies Week</td>
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### June

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<tr>
<td>18 – 19</td>
<td>PPTA Plasma Protein Forum</td>
<td>Reston, Virginia, United States</td>
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<tr>
<td>20 – 22</td>
<td>Immune Deficiency Foundation (IDF) National Conference</td>
<td>National Harbor, Maryland, United States</td>
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<td>21 – 23</td>
<td>Alpha-1 National Education Conference</td>
<td>Orlando, Florida, United States</td>
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### July

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<tr>
<td>6 – 10</td>
<td>XXVII Congress of the International Society on Thrombosis and Haemostasis (ISTH)</td>
<td>Melbourne, Australia</td>
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### October

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<tr>
<td>3 – 5</td>
<td>National Hemophilia Foundation (NHF) Annual Conference</td>
<td>Anaheim, California, United States</td>
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<td>7 – 11</td>
<td>International Plasma Awareness Week (IPAW)</td>
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<td>9 – 12</td>
<td>Latin American Society for Immunodeficiencies (LASID) Meeting 2019</td>
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<td>19 – 22</td>
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<tr>
<td>6 – 8</td>
<td>International Primary Immunodeficiencies Congress (IPIC)</td>
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<td>13 – 14</td>
<td>World Federation of Hemophilia (WFH) Global Forum</td>
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<td>PLATFORM OF PLASMA PROTEIN USERS</td>
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Lifesaving therapies are available for those living with rare, chronic disorders because of the generous contributions of plasma donors around the world. Learn more at: www.HowIsYourDay.org

Become a part of the initiative — Follow, like, and share! @HIYDglobal
In 2019 we are expanding our test menu to include viral marker testing (VMT) and related ancillary assays to offer our clients a comprehensive testing solution for their plasma screening needs. Since 1991, NGI has a history of providing industry-leading testing services and cost-efficient solutions for global plasma safety, all the meanwhile setting the standards for customer service and exceptional turnaround times.

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Learn more at: fresenius-kabi.com/us

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