

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SPRING 2013

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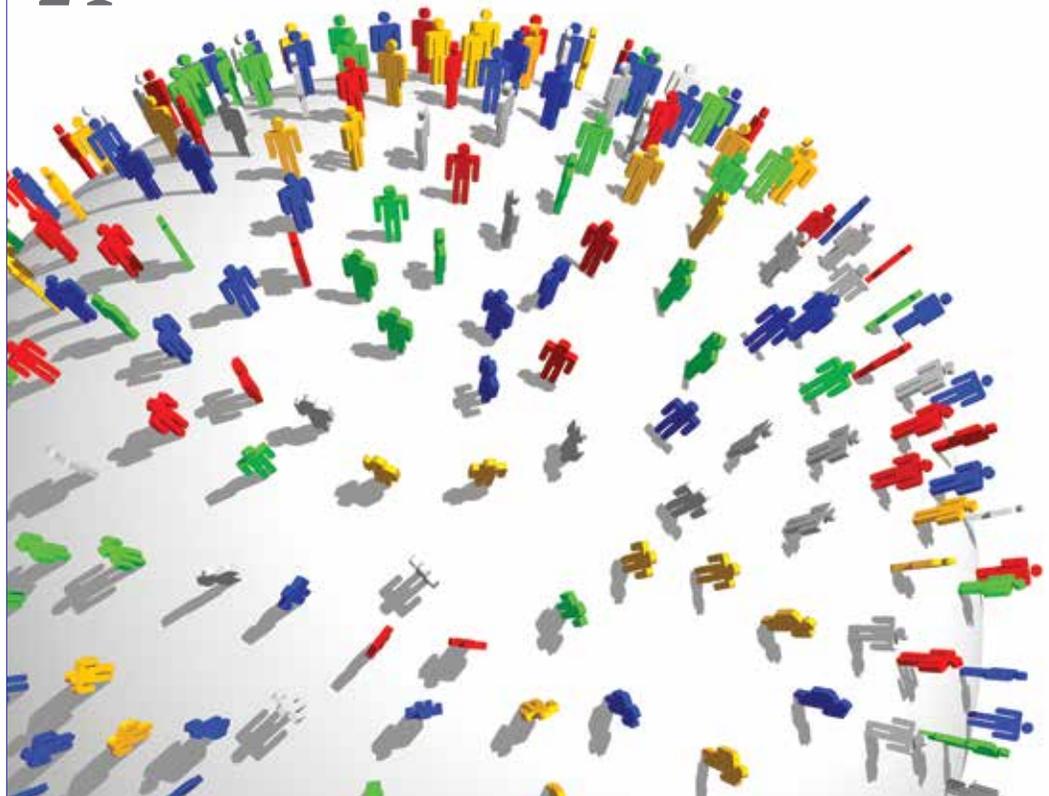
In the interest of encouraging broad and open discussion of issues relating to plasma protein therapies, collection and fractionation, the Source magazine may contain statements of opinion on such issues. These statements are those of the author and do not necessarily reflect the opinion of PPTA or its members. ©Plasma Protein Therapeutics Association 2013

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IN MY VIEW

BY JAN M. BULT, PRESIDENT AND CEO

EVERY FIVE YEARS THE JAPANESE MINISTRY OF HEALTH, Labor and Welfare (MHLW) has to review its National Blood Policy. This policy calls for self-sufficiency for blood and therapies that are made out of plasma. So far, the goal of self-sufficiency has only been accomplished for whole blood. The finished therapies have a varying degree of self-sufficiency but there are many comments that can be made about this.

Two years ago the MHLW convened a panel to discuss how to come to a future stable supply in Japan and achieve government goals of self-sufficiency. PPTA was invited to present our industry's views and it is encouraging to see that some of the discussion points have made it into the revision. One example is that for the first time it is mentioned that the use of immunoglobulins in Japan is low compared to other countries. While the government claims that the self-sufficiency rate for immunoglobulins is around 98%, PPTA is of the opinion that this is an artificial number since many patients with immune deficiencies are not diagnosed and need treatment. That treatment can only come from imported therapies and will then reduce the self-sufficiency rate.

In our conversation with MHLW we pointed out that there is a much bigger problem that needs to be tackled. Here are the reasons why:

- Japan has an aging population
- Japan has forecasted that in 15 years there will be a shortage of one million donors
- Many rare diseases are underdiagnosed
- The need for plasma protein therapies is increasing
- The volume of plasma for fractionation remains flat. In 2011 it was 920,000 liters per year
- The reimbursement system in Japan does not reward innovation

In recent interviews I have pointed this out and MHLW is paying attention to this. During my last visit I was asked to give examples where differences can be seen with other countries. I used that opportunity to again explain why I believe that there are many more patients with primary immune deficiency that require treatment with immunoglobulins. I am very encouraged by the positive tone of the conversation with MHLW.

I think it is time that more serious conversations need to take place with a view on how to secure the supply of life saving therapies to patients that need them. The domestic industry alone will not be able to do that. Our members currently provide therapies but I am convinced that more can be done. We are ready to engage in future dialogue. ☺



PPTA PRESIDENT FEATURED IN JAPANESE PRESS

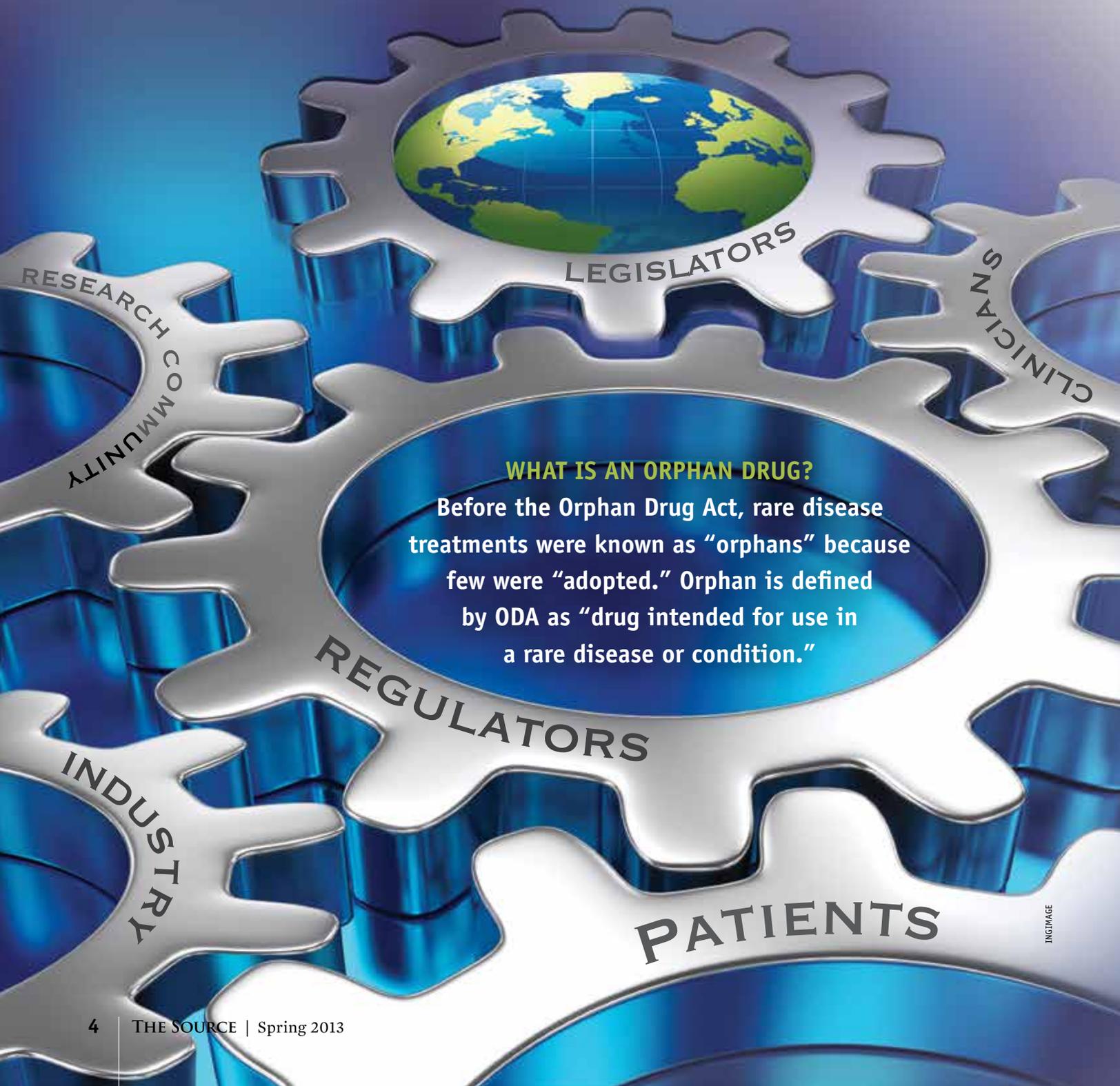
JAN M. BULT, PPTA PRESIDENT AND CEO recently headed a delegation to meet with the Japanese Ministry of Health, Labour and Welfare. PPTA submitted comments to the Ministry on proposed revisions to the *Basic Policy of Blood Products*.

During another visit in 2012, he was interviewed by the Japanese newspaper, *Nikkan Yakugyo*. Bult used the opportunity to reiterate the Association's concerns. Chiefly, the Japan Red Cross estimates that by the year 2027, there will be a blood donor shortage of over 1M people. Bult noted that the "amount of collected plasma has increased everywhere except in Japan." PPTA believes this demonstrates that patient needs are not being met.

According to Japanese policy, the domestic self-sufficiency rate of immunoglobulin products is more than 90%, but Bult indicated that "the amount may not appear insufficient based on formal statistical data, but there are many patients who are not receiving treatment even though they need immunoglobulins." Bult further suggested that "supply should be based on clinical needs, not by the amount provided by domestic manufacturers." Furthermore, he questioned the setting of plasma collection for 50 years, believing this to be highly speculative.



30 Years OF INNO



WHAT IS AN ORPHAN DRUG?

Before the Orphan Drug Act, rare disease treatments were known as “orphans” because few were “adopted.” Orphan is defined by ODA as “drug intended for use in a rare disease or condition.”

INGIMAGE

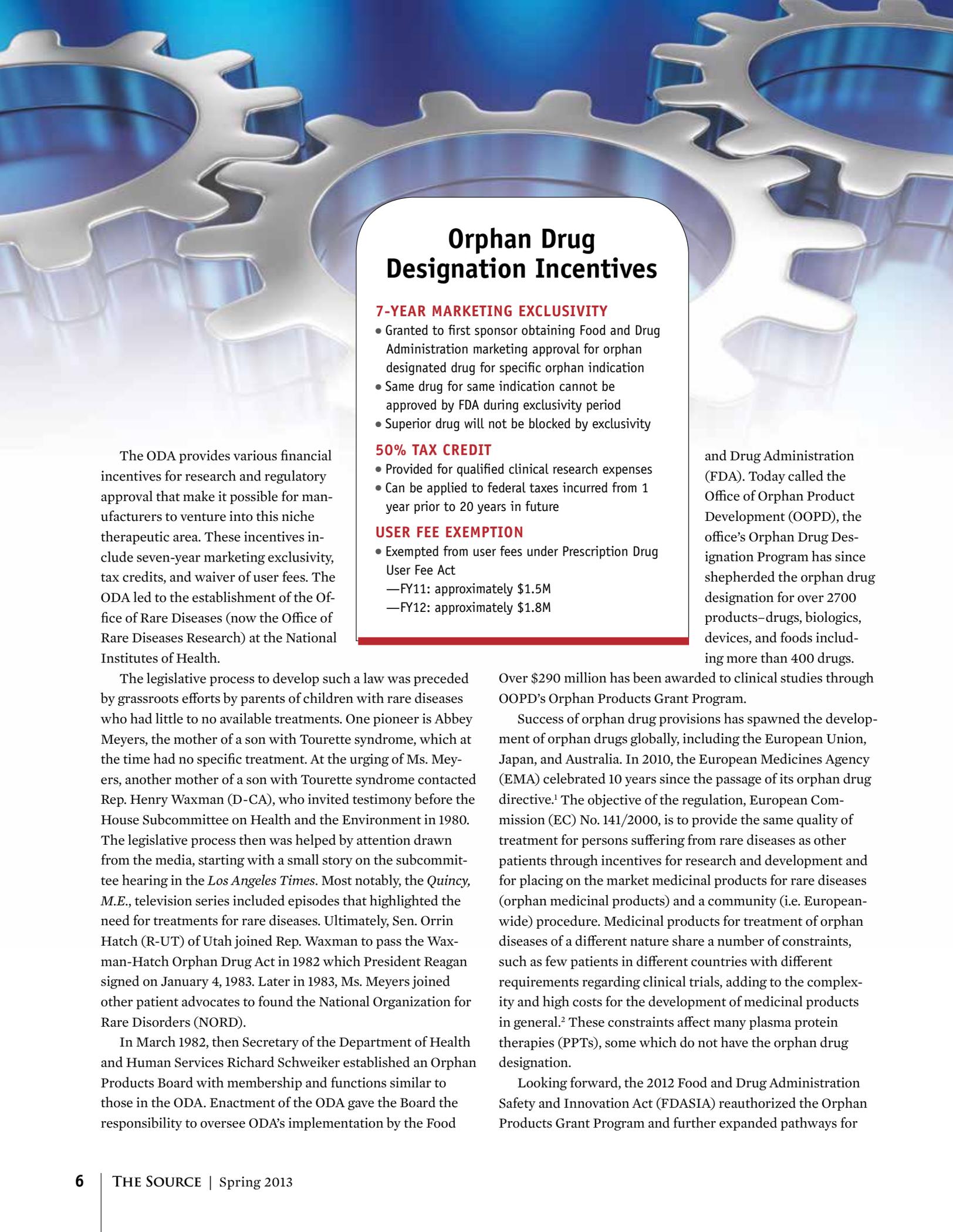
VATION AND PROGRESS

The Orphan Drug Act

BY MARY CLARE KIMBER AND MARY GUSTAFSON

THIS JANUARY MARKED THE THIRTIETH ANNIVERSARY of the passage of the U.S. Orphan Drug Act (ODA). People with rare diseases (one that affects less than 200,000 people in the U.S.) have benefited from this milestone legislation. Its passage and implementation has encouraged the development and marketing of numerous therapeutic products to treat rare diseases that would most likely not have been developed without this legislation.

"It has been an unbelievable 30 years since the Orphan Drug Act legislation was enacted. Each year we see a continued increase in research opportunities and emphasis on rare diseases and orphan product research," said Stephan C. Groft, Phd.M.D, Director of Office of Rare Diseases Research (ORDR) at the National Institutes of Health—ORDR is one of FDA's 30 Rare Disease Heroes.



Orphan Drug Designation Incentives

7-YEAR MARKETING EXCLUSIVITY

- Granted to first sponsor obtaining Food and Drug Administration marketing approval for orphan designated drug for specific orphan indication
- Same drug for same indication cannot be approved by FDA during exclusivity period
- Superior drug will not be blocked by exclusivity

50% TAX CREDIT

- Provided for qualified clinical research expenses
- Can be applied to federal taxes incurred from 1 year prior to 20 years in future

USER FEE EXEMPTION

- Exempted from user fees under Prescription Drug User Fee Act
 - FY11: approximately \$1.5M
 - FY12: approximately \$1.8M

The ODA provides various financial incentives for research and regulatory approval that make it possible for manufacturers to venture into this niche therapeutic area. These incentives include seven-year marketing exclusivity, tax credits, and waiver of user fees. The ODA led to the establishment of the Office of Rare Diseases (now the Office of Rare Diseases Research) at the National Institutes of Health.

The legislative process to develop such a law was preceded by grassroots efforts by parents of children with rare diseases who had little to no available treatments. One pioneer is Abbey Meyers, the mother of a son with Tourette syndrome, which at the time had no specific treatment. At the urging of Ms. Meyers, another mother of a son with Tourette syndrome contacted Rep. Henry Waxman (D-CA), who invited testimony before the House Subcommittee on Health and the Environment in 1980. The legislative process then was helped by attention drawn from the media, starting with a small story on the subcommittee hearing in the *Los Angeles Times*. Most notably, the *Quincy, M.E.*, television series included episodes that highlighted the need for treatments for rare diseases. Ultimately, Sen. Orrin Hatch (R-UT) of Utah joined Rep. Waxman to pass the Waxman-Hatch Orphan Drug Act in 1982 which President Reagan signed on January 4, 1983. Later in 1983, Ms. Meyers joined other patient advocates to found the National Organization for Rare Disorders (NORD).

In March 1982, then Secretary of the Department of Health and Human Services Richard Schweiker established an Orphan Products Board with membership and functions similar to those in the ODA. Enactment of the ODA gave the Board the responsibility to oversee ODA's implementation by the Food

and Drug Administration (FDA). Today called the Office of Orphan Product Development (OOPD), the office's Orphan Drug Designation Program has since shepherded the orphan drug designation for over 2700 products—drugs, biologics, devices, and foods including more than 400 drugs.

Over \$290 million has been awarded to clinical studies through OOPD's Orphan Products Grant Program.

Success of orphan drug provisions has spawned the development of orphan drugs globally, including the European Union, Japan, and Australia. In 2010, the European Medicines Agency (EMA) celebrated 10 years since the passage of its orphan drug directive.¹ The objective of the regulation, European Commission (EC) No. 141/2000, is to provide the same quality of treatment for persons suffering from rare diseases as other patients through incentives for research and development and for placing on the market medicinal products for rare diseases (orphan medicinal products) and a community (i.e. European-wide) procedure. Medicinal products for treatment of orphan diseases of a different nature share a number of constraints, such as few patients in different countries with different requirements regarding clinical trials, adding to the complexity and high costs for the development of medicinal products in general.² These constraints affect many plasma protein therapies (PPTs), some which do not have the orphan drug designation.

Looking forward, the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA) reauthorized the Orphan Products Grant Program and further expanded pathways for

Over the past century, the United States—largely through innovative pioneering by private industry and medical researchers in universities—has led the world in developing new drugs that have saved millions of lives. That is a gift to mankind we can be very proud of.

treatments for rare diseases. FDA’s Performance Goals and Procedures for the reauthorization of the Prescription Drug User Fee Act (part of FDASIA) include Advancing Development of Drugs for Rare Diseases. Of note, FDA plans to establish a Rare Disease liaison within the office of the director of the Center for Biologics Evaluation and Research (CBER)—the FDA Center that regulates plasma protein therapies—by the end of FY 2013. CBER’s Rare Disease liaison will join the Rare Disease Program in FDA’s Center for Drug Evaluation and Research to develop and disseminate guidance and policy related to advancing and facilitating development of drugs and biologics for rare diseases. FDA has set additional performance goals through the end of FY 2016. The Agency plans to conduct a public meeting to discuss complex issues in clinical trials for studying drugs for rare diseases (mid-FY 2014), develop and implement staff training related to development, review, and approval of drugs for rare diseases (end of FY 2015), and develop an evaluation tool to evaluate the success of the activities of the Rare Diseases 24 Program (end FY 2016).

PPTA is engaged with FDA on issues of rare diseases and orphan drugs through participation at Agency hearings and workshops and submission of comments on Agency documents and regulations to the docket. In particular, PPTA has encouraged FDA to revise rulemaking to allow orphan drug designation for second-to-market plasma protein therapies, without showing “clinical superiority,” while maintaining the requirement for marketing exclusivity. By such a revision, PPTA has argued, FDA will protect the integrity of the orphan drug program, encourage broad exploration for rare diseases, and allow for the development of brand diversity such that patients will have access to the therapy best suited to their individual needs.

PPTA joins FDA in its year-long celebration of the thirtieth anniversary of the ODA and looks to a bright future where orphan drug availability and choice continue to grow. The Association echoes President Reagan’s words over thirty years ago: Over the past century, the United States—largely through innovative pioneering by private industry and medical

Product Classes of Orphan Designated Plasma Protein Therapies

| Product Class | Rare Disease |
|---------------------------------|--|
| Alpha1-proteinase inhibitor | Alpha-1 antitrypsin deficiency |
| Immunoglobulin | Chronic inflammatory demyelinating polyneuropathy |
| Rho(D) immune globulin | Idiopathic thrombocytopenic purpura |
| Cytomegalovirus immune globulin | Cytomegalovirus disease in organ transplant patients |
| Hepatitis B immune globulin | Hepatitis B in organ transplant patients |
| Factor VIII | Hemophilia A |
| Factor IX | Hemophilia B |
| Factor VIIa (recombinant) | Hemophilia A and Hemophilia B with inhibitors |
| Factor XIII (plasma-derived) | Factor XIII deficiency |
| Fibrinogen | Congenital fibrinogen deficiency |
| C1 esterase inhibitor | Hereditary angioedema |
| Protein C concentrate | Protein C deficiency |
| Von Willebrand factor | Von Willebrand’s disease |

APPENDIX C –TABLE 2, HYMAN, PHELPS & MCNAMARA, P.C., WHITE PAPER: THE NEED FOR CHANGES TO THE FOOD AND DRUG ADMINISTRATION’S ORPHAN DRUG REGULATIONS: A CASE FOR DELINKING ORPHAN DESIGNATION AND CLINICAL SUPERIORITY

researchers in universities—has led the world in developing new drugs that have saved millions of lives. That is a gift to mankind we can be very proud of.³

Endnotes

1. Ten years of orphan medicines legislation in Europe—European Medicines Agency reviews success and looks ahead, May 20, 2010, http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2010/05/news_detail_001027.jsp
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3. Ronald Reagan: “Statement on Signing the Orphan Drug Act,” January 4, 1983. Online by Gerhard Peters and John T. Woolley, The American Presidency Project. <http://www.presidency.ucsb.edu/ws/?pid=40583>.

MARY CLARE KIMBER, *Manager, Regulatory Policy and* MARY GUSTAFSON, *Vice President, Global Regulatory Policy*



FINANCIAL PRESSURES ON PATIENT ACCESS: U.S.

BY KYM KILBOURNE

THE CURRENT UNITED STATES FISCAL CLIMATE continues with uncertainty with the passage of the American Taxpayer Relief Act of 2012, late last year, which delayed sequestration until March 1. This control measure specifies across-the-board cuts to government spending estimated now at \$83.5 billion for the year (\$42.7 billion for non-defense spending).

Some in Congress have proposed targeted reductions and revenue raisers. But, if the sequester were to go into effect, it is estimated that non-defense spending would be cut by 5.3% based on fiscal 2013 appropriations. The terms of the sequester cap cuts to Medicare at 2%, leaving the remaining non-defense cuts to be distributed across the other agencies resulting in layoffs and changes to programs and contracts across the federal government. Couple these possible cuts with the expiration of the current continuing resolution, which funds the government at current levels at the end of March and the short-term raising of the debt—to be passed by the House and the Senate by April 15 the salaries of those lawmakers in either branch of Congress that fails to do so would be withheld.

And, while the more than 27% pay cut for doctors serving Medicare patients has been pushed off until the end of the year, discussions centered on reforming Medicare, remain part of the Republican dialogue. Lawmakers continue to discuss proposals to reduce spending in entitlement programs like Medicare, which was \$551B in 2012 and is expected to swell to \$1T by 2022. Tools to reduce spending

under discussion include raising the eligibility age to 67 and providing the Centers for Medicare and Medicaid Services (CMS) the ability to implement a least costly alternative policy for drugs that could significantly limit access to the most medically appropriate treatment as prescribed by a patient's physician. Others propose to combine the deductible for Medicare Parts A and B or to impose rebates on the population of Medicare enrollees that is dual eligible (also eligible under the Medicaid program). PPTA continues to meet with members of the House of Representatives and the Senate to discuss the value of plasma protein therapies (PPTs) in treating rare disease patients and to consider how patient access would be negatively affected in this environment.

While the nation's fiscal concerns are front and center, the implementation of the Affordable Care Act (ACA) health insurance exchanges continues with individuals slated to begin enrolling in October and for coverage to begin January 2, 2014, which remains an ambitious deadline. The goal of the state insurance exchanges are to provide affordable and comprehensive health insurance plans that consumers can compare and shop for in an online marketplace that



All of these issues combine to create an environment in which pressures to reduce spending to curb the federal deficit need to be balanced with sound health policy that protects access to lifesaving therapies for the most vulnerable patient populations.

offers competitive health insurance plan rates based on federal guidelines for benchmark requirements. Consumers without insurance provided through government programs like Medicare and Medicaid, a state program which insures the poorest citizens for which the federal government provides matching funds, and private insurance provided by employers, or Federal or State employee insurance programs can either participate by purchasing a plan through the exchange or pay an annual penalty if they forgo purchasing coverage.

There are many lingering questions regarding how states and the federal government will implement exchanges, what effect different state approaches to expanding Medicaid eligibility

may have on providing basic health insurance coverage, and how individuals with rare, chronic diseases requiring lifelong infusions of PPTs will be assured of access to their medically appropriate therapy and to specialist care required to maintain their health.

All of these issues combine to create an environment in which pressures to reduce spending to curb the federal deficit need to be balanced with sound health policy that protects access to lifesaving therapies for the most vulnerable patient populations. ☪

KYM KILBOURNE, *Director Federal Affairs*

THE POWER OF A COMMUNITY: PASSING THE MEDICARE IVIG ACCESS ACT

BY MARCIA BOYLE

REPRESENTATIVE KEVIN BRADY (R-TX) explained the plight of patients with primary immunodeficiency diseases (PID) on the floor of the House of Representatives on December 19, 2012. He spoke as lead sponsor of the Medicare IVIG Access Act (HR 1845) and steadfast advocate of the primary immunodeficiency community. After passing by an overwhelming majority in the House and unanimously in the Senate, with provisions of the Strengthening Medicare and Repaying Taxpayers (SMART) Act included, President Obama signed the Medicare IVIG Access Act into law on January 10.

The long road to this historic milestone for the PID community was paved with challenges, but we succeeded because of the tremendous leadership of our Congressional champions, the relentless efforts of the Immune Deficiency Foundation (IDF) and our community of supporters. The resulting law creates a demonstration project supporting access to all products in all sites of care for Medicare beneficiaries with PID by improving access to intravenous immunoglobulin (IVIG) in the home. It is important to understand the evolution of the law and the power of a community who made it a reality.

The Medicare Modernization Act (MMA) of 2003 created a new benefit for Medicare patients with PID to receive IVIG infusions in the home, in addition to the other covered settings, such as hospital outpatient departments, infusion clinics and physician offices. This was a critical achievement for IDF and our community, giving Medicare patients with PID the choice to avoid the hospital when their immunity is at the lowest. However, the law as written only provided coverage and reimbursement for the immunoglobulin product, not the items and nursing services needed to infuse it. A separate provision in the



PPTA congratulates IDF on this legislative achievement.

MMA changed the reimbursement methodology for IVIG and other Part B drugs. The unintended consequence of this change was to reduce IVIG reimbursement to the point where it was less than the cost of infusing IVIG in many settings, essentially rendering the IVIG home infusion provision an “empty benefit.” Concerned providers and patients flooded IDF with calls and e-mails. Very few physicians wanted to take our patients; hospitals were not always an option, and virtually no infusion companies wanted to take Medicare patients on IVIG, creating an unsettling time for our community.

IDF took many steps to educate Members of Congress and the Centers for Medicare and Medicaid Services (CMS), including countless meetings and testimonies. IDF mobilized grassroots volunteers to sign up for the first IDF Action Alert to directly contact their legislators to help in the effort. To increase our visibility on Capitol Hill, IDF retained the services of Hart Health Strategies.

To provide data behind the calls and anecdotes received, in 2006 IDF conducted patient, physician and pharmacy surveys to document their Medicare and IVIG experiences. The patient component of these surveys found that patients on Medicare, when compared to their private pay insurance counterparts, were much more likely to report having IVIG therapy postponed, treatment intervals increased and their IVIG dosage decreased. IDF survey data was cited in the 2007 report published by the Department of Health and Human Services (HHS) Office of the Assistant Secretary for Planning and Evaluation (ASPE), documenting the access problems for Medicare patients. Months

later a report published by the HHS Office of Inspector General, confirmed the information provided by IDF surveys.

During this time, Representative Brady became personally involved. His leadership began after meeting with his constituent, Carol Ann Demaret, member of the IDF Board of Trustees and mother of David Vetter, diagnosed with Severe Combined Immune Deficiency and known around the world as the “boy in the bubble.” If not for Representative Brady and Carol Ann, the Medicare IVIG Access Act would not have been introduced and become law.

The resulting law creates a demonstration project supporting access to all products in all sites of care for Medicare beneficiaries with PID by improving access to intravenous immunoglobulin (IVIG) in the home.

In 2007, Representative Brady introduced the first of several bills in subsequent years that would allow Medicare to reimburse for the items and services needed to receive IVIG infusions in the home. Senator John Kerry (D-MA), during the healthcare reform debate in 2010, introduced an amendment creating a demonstration project as an intermediate step to solving the home infusion problem. The idea of a demonstration project gained some political traction and that same year Representatives Doris Matsui (D-CA) and Brady introduced a Medicare IVIG demonstration bill based on Senator Kerry’s amendment. The project would allow Medicare beneficiaries with primary immunodeficiency diseases to receive IVIG infusions in the home and gather data to work toward permanent reimbursement for IVIG in the home.

With a new Congress in 2011, Representatives Brady and Matsui again introduced the demonstration bill that is now law—the Medicare IVIG Access Act (HR 1845).

It takes an extraordinary amount of support and data to pass any legislation. Over the five year period, patients sent more than 10,000 letters to Congress and made hundreds of visits to Capitol Hill. To answer Congress’ questions about cost, a financial analysis by Dobson DaVanzo was commissioned and its findings were disseminated. The June 2012 report from the Medicare Payment Advisory Committee (MedPAC) also helped our cause. At the behest of Congress, MedPAC looked at home infusion, including the access problem for Medicare beneficiaries with (PID). MedPAC reported to Congress that “a targeted



COURTESY IDF



Carol Ann Demaret,
Congressman Brady
and Marcia Boyle.

“Regular access to IVIG therapy means a better quality of life, less disability, and potentially the difference between life and death.”

—Representative Kevin Brady (R-TX)

expansion of home infusion coverage focusing on a subset of drugs would have more likelihood of savings ...Drugs with a narrow indication and precise diagnostic criteria (e.g., IVIG for PID) would be less subject to a woodwork effect than drugs with broad uses or less precise diagnostic criteria...” We were encouraged by this report and IDF volunteers shared it with their legislators.

Our community was optimistic of the bill’s success but remained vigilant and continued to advocate for it. By mid-December 2012, the bill began moving and never stopped.

Some say the way the Medicare IVIG Access Act passed is amazing. Our community appreciates the significance of it passing as its own bill maintaining its original name. The fact that “IVIG” remains, an acronym that is such an important part of our community, is extremely meaningful. But the most amazing and meaningful aspect is the power of a community. When Representative Brady named Carol Ann, IDF and me in his remarks on the floor of the House on December 19, I realized the magnitude of the work of so many, for so long:

“I especially want to thank my constituent friend, Carol Ann Demaret, the mom of David, for her decades of hard work on behalf of these patients. And I appreciate so much Marcia Boyle, the founder of the Immune Deficiency Foundation, and all those patients who for years have come up here asking for this help and change. Today, this Congress, Republicans and Democrats alike, join together in providing that help and that access.”

Ultimately the Medicare IVIG Access Act was about just that—**access** to care. Medicare beneficiaries living with PID have the right to access the site of care that is best for them, including the right to receive their life-saving IVIG in the home setting. The process and end result exhibit the strength and commitment of our community, including thousands of patients and families. My humble gratitude goes to Carol Ann Demaret; Representatives Brady and Matsui; Senators Kerry and Lamar Alexander (R-TN); Hart Health Strategies and Sue Ramthun; PPTA and its member companies; the members of American Plasma Users Coalition and National Organization of Rare Disorders; American Academy of Asthma, Allergy & Immunology; Clinical Immunology Society; the wonderful staff at IDF; and all those who supported the bill.

With continuing work and advocacy, it is my hope that the demonstration project will prove what we have expected all along and a permanent resolution will become law. ☺

MARCIA BOYLE, *President & Founder, Immune Deficiency Foundation*

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DIAGNOSING PLASMA PROTEIN DISORDERS: THE IMPORTANCE OF

BY PROFESSOR ALBERT FARRUGIA



Hence the prevalence, or how many individuals in the total population suffer from one of these diseases at any given time, is difficult to determine. And yet we find that assessing prevalence is a crucial component of ensuring access to PPTs worldwide.

A CRUCIAL SECTION

of the plasma protein recipient population is the rare disease community. Plasma protein therapies (PPTs) are classifiable in many ways, and one of the divides occurs between those recipients who require treatment once, or, at most, on a few occasions, and those for whom treatment will be a life-long experience. We can therefore view therapies as being for either acute or chronic care. Most patients on chronic plasma protein treatment suffer from inherited deficiencies of plasma proteins, such as coagulation Factor VIII and immunoglobulin G, leading to hemophilia A and primary immune deficiency (PID) respectively. These are rare diseases and it is likely that most primary care physicians will not see more than one case, if at all, in all their years of practice.

PPTs and orphan drugs

Rare plasma protein disorders are treated with therapies which are classifiable as orphan drugs or orphan medicines. Such drugs are defined as those that are used to treat low-prevalence patient populations. The acceptance of a drug as conforming to the definition of an orphan drug rests with regulatory authorities, which accord this status to a drug based on its prevalence and its promise to treat a condition. This status is important. Once a drug is accepted as an orphan, the company marketing it is eligible for certain benefits, which act as inducements for the development of such orphan drugs. Otherwise, companies wishing to develop innovative drugs for treating rare conditions would be impeded by the high costs and regulatory hurdles which are incurred in bringing a new treatment to the market. Therefore, determining the prevalence of a condition, through accurate and verifiable diagnosis,

GETTING IT RIGHT

is crucial to supporting a drug's status as an orphan and directly enhances access to innovative therapies for patients with rare disorders.

How much is enough?

The second reason is linked to the usage of the therapy once it is approved for marketing. Given the long time it takes for the process of plasma protein manufacture to progress from plasma collection to patient treatment, it is essential to have a good idea of product clinical demand. Clearly, this is not possible without an accurate idea of the prevalence of the disease. Once we get an accurate estimate for the prevalence of a disease, we can estimate the latent therapeutic demand for a plasma protein therapy. The latent therapeutic demand indicates the amount of product which doctors would prescribe for patients if they were not constrained by factors such as reimbursement, plasma supply etc. For example, using the best available clinical evidence for factors such as prevalence, dosage and others for the treatment of PID with immunoglobulin (IG), the PPTA's research indicates that the latent therapeutic demand is of the order of 100 g/1000 population.

Why is this important?

This means that in most countries, where the total IG consumption is less than this figure, PID is under-treated. And the biggest reason for this is that the disease(s) are substantially under-diagnosed. All other things being equal, we would expect the prevalence of a genetic disorder such as PID to be relatively similar between similar sized large populations, and for PID this is expected to be approximately 5 per 100,000 population. However, we observe that in some countries, including wealthy countries with a strong health care system, several factors combine to disincentivize diagnosis of rare disorders. The classic example is Germany (see Sharndorf, *The Source*, Summer 2011).

This has potential serious effects for patient care in all countries. We are saddened to see certain authorities pointing to the low IG consumption in countries with a high health standard as an excuse to curtail access to IG in their respective jurisdictions. It is unacceptable that patients are shifted to the lowest level of treatment because of factors which are specific to certain countries, irrespective how rich, and which need themselves to be corrected.

What is PPTA doing to correct this? We are working to ensure that diagnosis of plasma protein disorders occurs in the best way possible—through medical assessment. We support the biggest data base of immunodeficiency in the world—the European Society for Immune Deficiencies (ESID) Registry of patients. In the Registry, patients whom have had their PID diagnosis confirmed by a specialist are registered with their disease profile and other useful information, including therapeutic information on treatment efficacy and adverse events. This allows estimates, among other things, of latent clinical needs as well as informing specialists on the best treatments. And in countries like Germany where under diagnosis is obvious for reasons which are becoming clearer we support patients and clinicians in increasing awareness of rare disorders and overcoming artificial barriers to access. (See Sharndorf, *The Source*, Summer 2011)

Above all, we focus on the patients. We are strongly encouraged by the new era of medical care—that of individualized medicine and patient centeredness in anything affecting patient care. We appeal to patients to make full use of all the tools available in this age of instant information, to learn and influence the decisions which affect their lives. As we stated on the Fall 2010 cover of *The Source*, "All Roads Lead to the Patient." 

ALBERT FARRUGIA PH.D., Vice President Global Access

Therefore, determining the prevalence of a condition, through accurate and verifiable diagnosis, is crucial to supporting a drug's status as an orphan and directly enhances access to innovative therapies for patients with rare disorders.

U.S. STAKEHOLDERS DEFINE 2013 PLANS AND PRIORITIES

BY JULIE BIRKOFER AND LISA LOVULLO

REPRESENTATIVES FROM U.S. BASED CONSUMER ORGANIZATIONS, industry, including North America Board members and PPTA staff met in January to share advocacy priorities and alignment on issues and opportunities for collaboration.

There was widespread consensus on the importance of numerous issues. Stakeholders are enthusiastic about the Food and Drug Administration's (FDA) Patient-Focused Drug Development initiatives, which seek to gain patient input in two ways. First, under Food and Drug Administration Safety and Innovation Act (FDASIA), the Agency will "solicit the views of patients during the medical product development process and consider their perspectives during regulatory discussions."¹ Similarly, PDUFA V provides for "more systematic and expansive approach to obtaining the patient perspective on disease severity or the unmet medical need in a therapeutic are to benefit the drug review process."² Furthermore, there is considerable interest in Patient-Centered Outcome Research Institute (PCORI)'s rare disease advisory panels and comparative effectiveness research.

In addition, both PPTA and consumer groups intend to devote time reaching out to more than 90 new members of Congress to establish a baseline position for patient access to plasma protein therapies. In addition, to the Association's Capitol Hill Fly-In on May 8th, several groups have planned advocacy days on the Hill and PPTA and industry plan to participate. There was an equally strong emphasis on educating and training grassroots advocates.

There was also consensus on the need for data to support patient advocacy, specifically, to use evidence-based guidelines (www.guidelines.gov). PPTA's work in developing health technology assessment models (HTA's) provide a good example of how effective data can be in working with decision-makers.

Health care reform continues to top agendas

with the implementation of the Affordable Care Act (ACA) underway, Medicaid expansion and its impact on State health exchanges, Essential Health Benefits (EHB) plan management and Medicaid Managed Care are front and center hot buttons. PPTA, consumer organizations and the State Patient Access Coalition (SPAC) are monitoring these developments closely.

More targeted efforts relate to condition-specific issues. The primary immunodeficiency community will continue to advocate for newborn SCID screening which has been approved in 16 states, the Navajo nation and Puerto Rico. The hemophilia community is placing a strong emphasis on consumer education and grassroots advocacy.

These joint meetings offer an important opportunity for those in industry and the rare disease community to join forces on mutual goals. Together we can effectively inform policymakers about plasma protein therapies, the importance of patient access to those therapies and the unique needs of rare disease populations.

Finally, collaboration offers its rewards. We were delighted to celebrate the Immune Deficiency Foundation's (IDF) victory with the passage of the Medicare IVIG Access Act which President Obama signed on January 10. (see p.10) ☺

JULIE BIRKOFER, *Senior Vice President, North America*,
LISA LOVULLO, *Senior Manager, Communications*.

1. U.S. Food and Drug Administration (FDA), Fact Sheet: Increased Patient Participation in Medical Product Regulation, <http://www.fda.gov/RegulatoryInformation/Legislation/FederalFoodDrugandCosmeticAct/FDCAAct/SignificantAmendmentstotheFDCAAct/FDASIA/ucm311045.htm>

2. Ibid.

These joint meetings offer an important opportunity for those in industry and the rare disease community to join forces on mutual goals.



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ASSOCIATION TO LAUNCH ANNUAL INTERNATIONAL AWARENESS EVENT

BY LISA LOVULLO

PPTA IS PLEASED TO ANNOUNCE the first International Plasma Awareness Week (IPAW) to be celebrated October 13-20, 2013. The Association and its member companies are planning activities and events designed to:

- Raise awareness about source plasma collection
- Celebrate and recognize the contributions of plasma donors
- Expand knowledge about plasma protein therapies and rare diseases

In spearheading this global effort, PPTA has responsibility for coordinating efforts, designing promotional materials, communication to various stakeholder groups and media relations. The Association is developing a toolkit for member companies to use including posters and banners in various sizes and multiple languages (English, French, German, Spanish, Italian and Czech), radio ads, press releases,

The event will be promoted internally at the International Plasma Protein



Congress (IPPC), the Plasma Protein Forum (PPF), and via Association publications, websites and communications. External outreach consists of an international media campaign, public service announcements, outreach to policymakers. In addition, efforts will be made to secure proclamations and resolutions from lawmakers in various jurisdictions to officially recognize the week. As those are obtained, the public relations offices of those governments will add additional weight to promotional efforts and outreach.

Member companies are actively planning events including donor appreciation celebrations at plasma collection centers, open houses and tours for patients, community leaders and the media, and internal company communications and

activities. Both the Source Industry Profile Committee (SIPC) and the Global Communications Steering Committee (GCSC) are responsible for planning and executing company events and promotions.

The initial response to IPAW has been enthusiastic. In particular, stakeholder groups have expressed interest in being involved and helping to celebrate the event. All are welcome to participate and take advantage of toolkit resources to plan events or promote the week.

We are in the early stages of developing what promises to be a successful annual effort. Together we are bound by a common goal—to *save and improve lives*. IPAW serves as a catalyst for sharing that mission and our many contributions to helping patients throughout the world. 🌐

LISA LOVULLO, *Senior Manager, Communications*

ERIK BERNTORP HONORED WITH HILFENHAUS AWARD

EACH YEAR THE ASSOCIATION RECOGNIZES an individual who has made outstanding contributions to the provision of plasma protein therapies its prestigious Hilfenhaus Award.

Erik E. Berntorp, M.D., Ph.D, Professor of Coagulation Medicine, Maime Centre for Thrombosis and Haemostasis, Lund University Sweden, Skane University Hospital Maime was named this year's recipient in a ceremony at the IPPC in Dublin. In presenting the award, Jan M. Bult, President and CEO said, "I'm pleased that the Association

is able to recognize the excellent work and contributions made by Dr. Berntorp to patients throughout the world. It is uplifting to know that individuals of his calibre are making a difference in the lives of people every day."

Dr. Berntorp is one of the world's leading researchers and clinicians working in hemophilia and coagulation disorders. For thirty years, he has worked to develop modern and efficient methods of treating haemophilia with a sincere commitment to improving patients' quality of life. Upon

receiving the award, "I feel very honored to receive this award because it's an appreciation for work I have done during my professional career. I hope what I have done has benefited patients and will do so in the future."

His research interests include prophylactic treatment of hemophilia and of von Willebrand disease, genetic aspects on inhibitor development and treatment of inhibitors in hemophilia including by-pass therapy and immune tolerance induction. His scientific contributions comprises have resulted in over 200 publications in leading scientific journals, serves as a reviewer of numerous journals including Blood,

DUBLIN PROVIDES BACKDROP FOR INTERNATIONAL PLASMA PROTEIN CONGRESS

BY LAURA SAVINI
AND LISA LOVULLO



Jan M. Bult and
Global Board
Chairman,
Paolo Marcucci

NEARLY 300 INDUSTRY LEADERS, policymakers and patients from around the globe convened in Dublin for the 10th Annual International Plasma Protein Congress (IPPC). The two-day conference provided a broad range of presentations from experts in their fields.

Dr. Barry White kicked off IPPC with the keynote address on the Irish Comprehensive Care Model for Hemophilia. This successful system is underpinned by legislation and is based on a strategy that aligns objectives among patient organizations, finance and health care. It has led to improvements in quality, costs and access and reduced overall expenditures by 15% while increasing treatment nationally.

Following the keynote, James Fischer, Chair of the PPTA Europe Board of Directors provided a state of the industry report focusing on Europe. The goals of PPTA Europe are to preserve and expand patient access and investment and innovation in therapies. An

aging population and economic crisis are impacting the industry and PPTA's European strategy. High on the PPTA's agenda are the European Commission's Sectoral Study which may lead to a possible revision of the Blood Directive.

Erik Berntorp, was awarded the Association's Hilfenhaus Award and delivered a presentation on his work in prophylactic treatment for hemophilia. The rationale was first proposed in the early 1960's and has proven to be very effective.

In another session, regulators from the European Medicines Agency and the Food and Drug Administration discussed the requirements for pre-market authorization of therapies and post-market monitoring. As plasma protein therapies (PPTs) treat rare diseases and involve relatively small numbers of subjects, these studies can prove difficult. Increasingly regulatory agencies are seeking patient input at all levels of the regulatory process.

Paolo Marcucci, the newly appointed Chair of PPTA's Global Board of Directors took the floor to articulate an expanded mission and vision that focuses on a global, multinational perspective. Marcucci said, "I believe we have an ethical commitment to developing orphan drugs. This is not a matter of quality of life, but a matter of life."

Professor Albert Farrugia, PPTA Vice President Global Access provided a mechanism for realizing that expanded vision. Through the development of Health Technology Assessment (HTAs) models, Farrugia and his colleagues have

been able to successfully demonstrate the cost effectiveness of several plasma protein therapies. In this global economic climate, these instruments are proving invaluable for decision-makers and to preserving patient access to therapies.

An international session continued the global theme. Delin Kong outlined the difficulties for hemophilia patients in China to access therapies. An individual's social standing known as hukou may affect access and reimbursement to therapies. He represents Hemophilia Home of China, a patient organization that started in 2000 and is working to secure supply, access to a variety of therapies and remove health policy obstacles to reimbursement.

Dr. Ahmed Bousfiha, turned the focus to Northern Africa and primary immune deficiency (PID). A geographic area that includes Morocco, Libya, Tunisia, Algeria and Egypt, the region has a higher prevalence of PID than either Latin America or Europe. Strong inbreeding and limited access to health care and education contribute to the high incidence of the disease.

Alain Weill, President of the World Federation of Hemophilia presented the work carried out by the Federation over the past 50 years in terms of access to treatment and information to patients. He highlighted the importance of national patient organizations learning from each other and becoming aware of how to raise hemophilia's profile on the political agenda in order to improve access to treatment for all.

Journal of Thrombosis and Haemostasis and Hemophilia and has been editor for several major textbooks. Berntorp has also served on the councils of the World Federation of Hemophilia and the European Hematology Association. In addition, he has been a vocal advocate for patients within social and political debates. In addition to receiving the award, Dr. Berntorp delivered a presentation on the "Prophylaxis and the Future Treatment of Hemophilia."

The Hilfenhaus has been awarded since 1998. It is named in honor of Dr. Joachim Hilfenhaus, a well-respected virologist who chaired the Committee of company experts dealing with pathogen safety. 

INSIDE PPTA

The second day opened with an overview of plasma protein therapies by Patrick Robert of the Marketing Research Bureau. Another session focused on the collection of whole blood and plasma in Italy by Professor Jommi. He described the complexities with the various regions in Italy. He was followed by Rutger Wouters who provided more insight of the findings of the Con-Quaestor study into the public and private activities of Sanquin in The Netherlands. PPTA President and CEO, Jan M. Bult and Bob Perry, Executive Director of the International Plasma Federation Association (IPFA) gave their respective perspectives on the supply of plasma protein therapies in Europe.

Another session focused on research carried out on donor motivation and in particular on blood donations and kidney donors for transplants. Dr. Mario Macis presented the results of several studies conducted in the USA and Argentina on blood donor motivation. The findings of the studies showed that economic incentives have a positive effect on donations by existing donors, with no crowding out of quantity or quality. Social recognition also motivates donors; however it will depend on the circumstances. Willem Weimar M.D., professor of

Internal Medicine at Rotterdam University, discussed the current European framework for kidney transplants from living donors and provided more information on the incentives and misconceptions about kidney donations from living donors.

To illustrate the increased need for PPTs, Dr. Volker Wahn of the Charité Hospital in Berlin reported on successful efforts to increase the PID diagnosis rate in Germany. Despite progress, it is estimated that as much as 90% of people with PID go undiagnosed.

IPPC closed with a session on clinical developments. Of particular note was a study by Dr. Antonio Paez of Grifols who leads the Alzheimers Management by Albumin Replacement (AMBAR) project. The study has proven effective with subjects showing better than expected cognitive results two years after receiving albumin. Although much work remains to be done, the study shows promise in addition to other therapies being developed to treat Alzheimer's. 🌐

LAURA SAVINI, *Manager, National Affairs*

LISA LOVULLO, *Senior Manager, Communications*

UPCOMING REGULATORY WORKSHOPS

▶ **PLAN TO ATTEND PPTA'S WORKSHOP,**

Industry's Commitment to Safe and Healthy Donors, on June 10th, from 1:00 pm to 5:00 pm. The workshop will explore industry efforts to maintain the health and safety of individuals who donate Source Plasma. The workshop will describe the Source Plasma donor population, from donor demographics and donation frequency, to donor eligibility requirements and industry response to rare donor adverse events. Workshop attendees will go inside today's plasma centers to learn how industry compliance, with not only mandatory government regulations but also voluntary PPTA standards (International Quality Plasma Program (IQPP) and Quality Standards of Excellence, Assurance and Leadership (QSEAL)), help to foster a healthy donor base and a safe donation experience. The Food and Drug Administration (FDA) is expected to outline the history of its Source Plasma donor eligibility requirements and its current thinking regarding changes to a proposed rule published in 2007. FDA then is expected to present its analysis of donor adverse outcomes. The workshop will conclude with a session on industry donor surveillance highlighting successes, current practices, and future PPTA initiatives and will provide ample opportunity for attendee interaction. Attendees can engage with FDA representatives as well as PPTA staff and members, including the Chairs of the Source Board of Directors and the Regulatory Policy and Compliance

Steering Committee and members of the Medical Policy Committee. There is no fee to attend this workshop. You may register online at www.pptaglobal.org/pptaregistration/home.aspx.

▶ **THIS FALL, PPTA WILL JOIN** FDA and the National Institutes of Health (NIH) to co-sponsor a public workshop on immune globulin (IG) products and hemolysis. Hemolysis is a known but uncommon side effect of administration of intravenous IG (IVIG) that has been documented in the literature (see, e.g., Padmore, *Transfusion and Apheresis Science* 46 (2012) 93-96, doi: 10.1016/j.transci.2011.11.004) and is reflected in IVIG package inserts. Industry and regulators understand that high doses (≥ 2 g/kg, single administration or divided over several days) and non-O blood groups may be risk factors related to the development of hemolysis but that the role of an individual patient's underlying inflammatory state in increasing risk of hemolysis is uncertain. Workshop attendees will seek to identify appropriate risk-mitigation strategies by exploring risk factors in recipients (e.g. pathogenesis, epidemiology) and products (e.g. plasma, manufacturing).

Expected attendees include FDA representatives from the Office of Blood Research and Review and the Office of Biostatistics and Epidemiology, global regulators, and academicians. FDA will publish workshop notice and logistics in the Federal Register.

For additional information on these workshops, please contact Mary Clare Kimber, Manager, Regulatory Policy, PPTA at mckimber@pptaglobal.org or 443-433-1112. 🌐

COMMITTEE SPOTLIGHT



Federal Affairs Steering Committee

PPTA works with a talented team of federal government relations and health policy experts from each of its North America member companies to help guide and shape the direction of the Association's federal policy priorities. The overarching goal of the Federal Affairs Steering Committee (FASC) activities is ensuring patient access to all plasma protein therapies (PPTs) in all sites of care. The FASC monitors and engages with Members of Congress and their staff in legislative proposals and on key federal agency rulemaking.

Chief among the priorities is that Medicare, the federal government health insurance program for Americans over the age of 65 or who qualify as disabled, reimbursement of plasma protein therapies is sufficient to sustain patient access. Preserving the current Medicare Part B reimbursement rate of average sales price (ASP) +6 % in the physician office, which is the current statutory requirement, is of paramount and perennial concern, as are other potential entitlement reform policies aimed at reducing spending in the Medicare program,

The FASC not only focuses on Medicare policy, but also on working with members of Congress to provide information about the value of source plasma collection, plasma protein therapies (PPTs) and the rare diseases treated with the lifesaving therapies produced by PPTA member companies. With nearly one-third of the members of the House of Representatives in office for two years or less, it is important that PPTA cultivate relationships with lawmakers and to inform members about the uniqueness of

plasma protein therapies and how they are manufactured including differentiating industry from chemical pharmaceuticals, and key concerns regarding access to PPTs. This ongoing outreach helps the industry enhance its footprint on Capitol Hill, furnishes insight on the direction of policy proposals under consideration, and gives PPTA the opportunity to foster champions for key issues important to PPTA members and the patient community.

PPTA and the FASC also work together on industry comment letters to agencies such as the Centers for Medicare and Medicaid Services (CMS), the Health Resources and Services Administration (HRSA), the Internal Revenue Service (IRS) and the Health and Human Services Administration (HHS) on regulatory issues of concern to the industry. Further, the group discusses legislative policy initiatives that may affect access to therapies and develops messaging to convey the value of the lifesaving treatments. ☞

KYM KILBOURNE, *Director, Federal Affairs*

The overarching goal of the Federal Affairs Steering Committee (FASC) activities is ensuring patient access to all plasma protein therapies (PPTs) in all sites of care.

U.S. FEDERAL AFFAIRS STEERING COMMITTEE

Patrick Collins
CSL Behring

Joe Gibbons
Kedrion

Dennis Jackman
CSL Behring

Gavin Lindberg
Grifols

Mallory O'Connor
Grifols

Jed Perry
Baxter Healthcare

Meredith Zerbe
Baxter BioScience

Kym Kilbourne, Carrie Fiarman
PPTA Liaisons

STAFF

ALEXA WETZEL

Junior Manager, Source Europe

How long have you worked at PPTA?

I have been working at PPTA since May 2010. I was recently promoted to Junior Manager, Source Europe and have three primary responsibilities: representing the interests of European plasma collector's within the Association and to national and European organizations. I work with the Arbeitsgemeinschaft Plasmapherese e.V. (ARGE). This German association represents the common interests of plasma collectors from the industry, German Red Cross Institutions, independent collectors and community based collectors.

I am also involved in the European project FIND-ID. This initiative strives to increase awareness for primary immunodeficiencies and to build a network between general practitioners and specialized treatment centers for an earlier diagnosis of this disease and adequate treatment.

Tell us about your background.

I was born in the eastern part of Belgium in a city called Eupen which is the German speaking region. Belgium is home to three linguistic groups, Dutch (59%), French 40%), and German- (1%). I obtained a Bachelor Degree in Marketing and Master's Degree in Translation Sciences. I had the chance to spend a semester abroad in Rome, Italy and have always been fascinated by languages. I am bilingual in German and French, speak fluent English and have a working knowledge of Italian and Dutch.



What is your proudest professional achievement?

My proudest professional achievement was the successful organization of the last two Annual ARGE Congresses. These congresses are always held in late November and are attended by over 200 participants. The aim of the ARGE Congresses is to provide continuing medical education for plasma collection staff in Germany. It offers a unique platform

for exchange among different types of plasma collectors. PPTA has supported ARGE for many years now. It was a great pleasure for me to take over the organization of this important event.

What is most rewarding about working in this industry?

Knowing that we help people with rare and life-threatening diseases improve their lives. ☺

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GLOSSARY OF TERMS

| | | | |
|---------------|--|---------------|--|
| ACA | Affordable Care Act | IDF | Immune Deficiency Foundation |
| CBER | Center for Biologics Evaluation and Research | MMA | Medicare Modernization Act |
| CMS | Centers for Medicare and Medicaid Services | MedPAC | Medicare Payment Advisory Committee |
| HHS | Department of Health and Human Services | OOFD | Office of Orphan Product Development |
| EHB | Essential Health Benefits | ODA | Orphan Drug Act |
| ESID | European Society for Immune Deficiencies | PID | Primary Immunodeficiency Disease |
| HRSA | Health Resources and Services Administration | SPAC | State Patient Access Coalition |
| FDAISA | Food and Drug Administration Safety and Innovation Act | SMART | Strengthening Medicare and Repaying Taxpayer |

EVENTS

UPCOMING CONFERENCES
& SYMPOSIUMS

2013

| | |
|----------------------------|---|
| April 18–21 | WFH 13th International Musculoskeletal Congress 2013 Chicago, Illinois |
| April 22–25 | 19th Annual International Society for Cellular Therapy Meeting Auckland, New Zealand |
| April 23–24 | IPFA/PEI 20th International Workshop on Surveillance and Screening of Blood Borne Pathogens Helsinki, Finland |
| April 24 | Frontiers in Critical Care Amsterdam, the Netherlands |
| May 20–24 | HEMATOLOGY 2013 - IV International Workshop on Hemophilia La Habana, Cuba |
| May 20–24 | IX Latinamerican Meeting on Hematology, Immunology and Transfusion Medicine La Habana, Cuba |
| May 23–26 | 8 th C-1 Inhibitor Deficiency Workshop Budapest, Hungary |
| June 1–4 | Euroanaesthesia 2013 Barcelona, Spain |
| June 2–5 | 23rd Regional Congress of the ISBT, Amsterdam, The Netherlands Amsterdam, the Netherlands |
| June 5–9 | 3rd African Society for Immunodeficiencies (ASID) Congress Sun City, South Africa |
| June 10–14 | 6th Annual Emerging Technologies in the OR and Great Fluid Debate Lake Buena Vista, Florida |
| June 11–12 | Plasma Protein Forum Reston, Virginia |
| June 29– July 7 | XXIV International Society for Thrombosis and Haemostasis Congress Amsterdam, the Netherlands |
| September 24–27 | 46th Annual Meeting of the German Society for Transfusion Medicine and Immuno-Haematology Münster, Germany |
| October 4–6 | European Haemophilia Consortium conference Bucharest, Romania |
| October 12–15 | AABB Annual Meeting Denver, CO, USA |
| October 13 | Source Business Forum (PPTA Members Only) Denver, Colorado |
| November 7–8 | 1st International Primary Immunodeficiencies Congress Estoril, Portugal |
| December 1–4 | 24th Regional Congress of the ISBT Kuala Lumpur, Malaysia |
| December 7–10 | American Society of Haematology Annual Meeting New Orleans, Louisiana |

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