

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SUMMER 2013

PLASMA PROTEIN THERAPIES

**Patient-Focused
Drug Development
Takes Shape**

**ACA
Implementation
Looms Large**

**Quality of Life
Provides Valuable
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2

IN MY VIEW

Industry Continues to Invest in Research

4

Patient-Focused Drug Development

PPTA Stakeholders Drive Initiative

8

ACA Implementation

What Does it Mean for Plasma Protein Therapies?

10

Health Related Quality of Life

A Crucial Concept for Patients with Rare Plasma Protein Deficiencies

12

Augmentation Therapy Turns 25

Treatment Helped Build the Alpha-1 Community

14

Meet Jerry Treichel

An Alpha-1 Pioneer

15

Mandatory Rebates

Politics, Economics and Pharmaceuticals in Germany

16

Plasma Donor Sets Record

Heinz Behrmann Recognized for 1300 Donations

17

JMF Pleads for SCID Screening

Vicki Modell Testifies before House Committee

18

Compensated Donors and Safety

Canadian Debate Guided by Emotion not Facts

20

IPFA/PEI Celebrates 20th International Workshop

Major Event on Blood Safety and Availability Calendar

22

INSIDE PPTA

Website Redesign

European Stakeholders Meet in Dublin

25

COMMITTEE SPOTLIGHT:

Health Policy Steering Committee

26

MEET THE PPTA STAFF

Mary Clare Kimber,
Manager,
Regulatory Policy

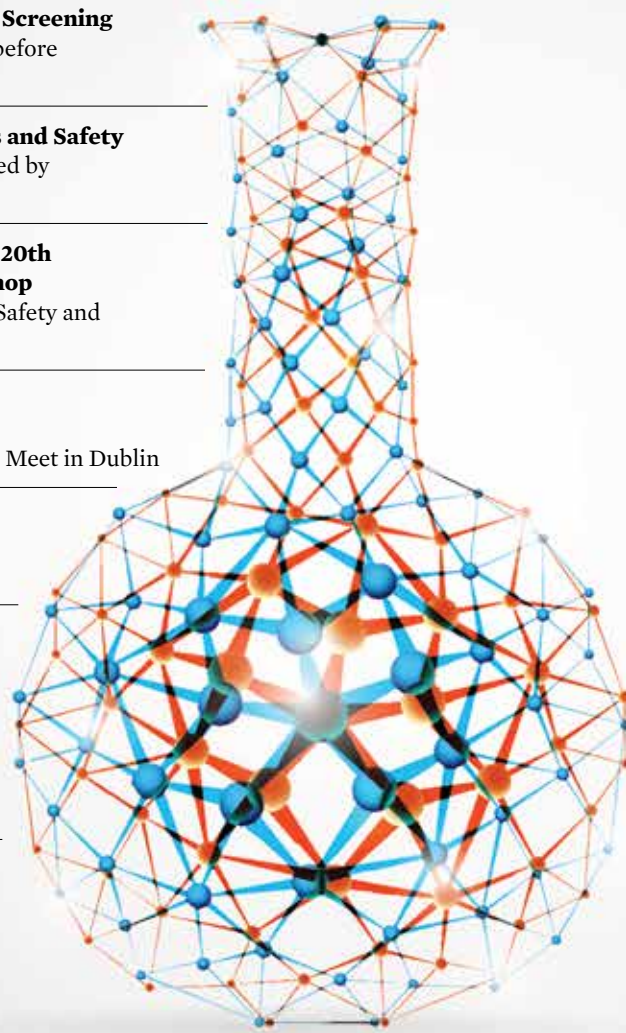
27

GLOSSARY

IN MEMORIAM

28

UPCOMING EVENTS
AND SYMPOSIA





IN MY VIEW

BY JAN M. BULT, PRESIDENT AND CEO

AS I AM WRITING THIS, we have just learned that a Phase III clinical study of immunoglobulin (IVIG) did not meet its co-primary end points of reducing cognitive decline and preserving functional abilities in patients with mild to moderate Alzheimer's disease.


It is not my intention to discuss details of this study but to give you some personal observations.

First: I am sad that a large patient population has to continue its search for a treatment of a debilitating disease that not only impacts the patient, but also the entire circle of family and friends. I can only hope that this study will contribute to new learning that one day will result in a treatment or cure.

Second: I am happy that other patient populations do not have to worry about the availability of immunoglobulins to

treat their (sometimes life threatening) conditions. Though I understood the concerns, I personally did not believe there would be a problem.

Third: I am proud that this study was done. It shows the commitment of our members to invest heavily into the further development of clinical use of plasma protein therapies. Developing clinical studies is a serious effort that is very time and resource consuming without any guarantee for success. There are many other clinical trials being performed with plasma protein therapies, not only in Alzheimer's but in many other fields as well.

I know that our industry continues to research, develop and market many plasma protein therapies that make a difference in many lives. 



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PPTA STAKEHOLDERS DRIVE PATIENT-FOCUSED DRUG DEVELOPMENT INITIATIVE

BY MARY CLARE KIMBER

PATIENT ORGANIZATIONS, including PPTA stakeholders, were instrumental in making a recent Food and Drug Administration (FDA) initiative, Patient-Focused Drug Development, (PFDD) a reality as part of the 2012 reauthorization of the Prescription Drug User Fee Act (PDUFA V). As early as September 2010, the FDA and stakeholders had been exploring the need for a venue in which the patient perspective could be discussed, outside of a specific product's marketing application review, where several programs already exist to facilitate patient representation.¹ FDA recognizes that patients have a unique and valuable perspective on analysis of the context of a regulatory decision—an important component of the Agency's benefit-risk assessment of a drug or biological product.² With that in mind, FDA included its PFDD initiative in its commitment to *"Enhancing Benefit-Risk Assessment in Regulatory Decision-Making"*, part of the Agency's PDUFA V Performance Goals and Procedures Fiscal Years 2013-2017. PDUFA V became effective with the passage of the Food and Drug Administration Safety and Innovation Act on July 9, 2012.

"The Alpha-1 Foundation and others have worked with the National Organization for Rare Disorders (NORD) and the National Health Council for several years to help the FDA bring more patient involvement to the drug development process,"



said John Walsh, Alpha-1 Foundation President & CEO. Kimberly Haugstad, Executive Director of The Hemophilia Federation of America (HFA) said, "HFA has worked with NORD, as a member of the greater rare disease community, on PDUFA for many years. We have participated in many joint letters with the American Plasma Users Coalition (A-PLUS), the National Hemophilia Foundation (NHF), the Committee of Ten Thousand (COTT), NORD, and others as well as having written many of our own comment letters, presented and spoken at various public meetings," She added, "Our view is that there is not

a single point that influences but a broad, ongoing advocacy voice that contributes to being heard at the FDA over a period of time." Through engagement with FDA, PPTA stakeholders have ensured that their patient communities will be among the 20 that are part of this five-year initiative.

Alpha-1 antitrypsin deficiency and Hemophilia A, Hemophilia B, von Willebrand disease, and other heritable bleeding disorders will each be the topic of a patient-focused drug development initiative meeting in FY 2014 or FY 2015 "We were thrilled to see that the FDA selected Hemophilia A, Hemophilia B, von Willebrand disease, and other heritable bleeding disorders as one of the disease areas of focus for the Patient-Focused Drug Development Program," said NHF CEO Val Bias. "While we have always worked closely with the FDA, this program provides a significant new opportunity for the

bleeding disorders community to discuss our own perceptions of risk and benefit. With so many hemophilia treatments in the pipeline, this is an ideal time for FDA to hear directly from people affected by bleeding disorders,” Bias added. “Walsh agreed, “Having Alpha-1 included in this exciting initiative is a tribute to the Alpha-1 community, and a gratifying result of our relationship-building efforts and the Foundation’s history of being patient-centered.” “We will participate in the upcoming public meetings as well as any comment opportunities and appreciate the opportunity to bring the patient community voice to this table,” Haugstad said. “We look forward to learning more about the initiative and will encourage FDA to include as many community members as possible in the process,” Bias added.

A second public process will be initiated to determine which four of the remaining 23 disease areas will be included in FY 2016 and FY 2017 initiative. The priority disease areas PPTA would like to be considered are thrombotic disorders, primary humoral immunodeficiencies, neurologic disorders treated with immunoglobulins, and hereditary angioedema.

On-going Dialogue between Stakeholders and FDA

At a September 29, 2010, FDA meeting with its stakeholders, FDA included PFDD as one of its 17 FDA proposals for enhancements to PDUFA V. PFDD was included in large part to address previous stakeholder concerns.³

Patient-Focused Drug Development

FDA proposed to ensure that patient perspectives are considered in regulatory decisions by hosting public meetings between review divisions and the relevant disease-area patient advocacy communities to review the armamentarium for specific indications or disease states and identify areas of unmet need. The goal of these systematic assessments is to improve the overall treatment

armamentarium, as well as, the efficiency and effectiveness of clinical development programs. As appropriate, FDA would develop new or improved guidance documents to assist sponsors in the clinical development of drugs for specific indications.⁴

FDA’s implementation of PDUFA V, including its commitment to “*Enhancing Benefit-Risk Assessment in Regulatory Decision-Making*,” is underway. In February, FDA published for public comment its draft “five-year plan to further develop and implement a structured benefit/risk assessment in the drug approval process.”⁵ FDA will, by the end of the fourth quarter of the Agency’s Fiscal Year 2013 (September 30, 2013), “begin execution of the plan to implement the benefit-risk framework across review divisions in the pre- and post-market human

FDA recognizes that patients have a unique and valuable perspective on analysis of the context of a regulatory decision—an important component of the Agency’s benefit-risk assessment of a drug or biological product.

drug review process” and will “update the plan as needed and post all update on FDA’s website.”⁶ In the plan, analysis of the context of a regulatory decision is embodied by the “Analysis of Condition” and “Current Treatment Options” sections of the benefit-risk framework.⁷ FDA’s patient-focused drug development initiative will inform these sections of the framework by obtaining the patient perspective on the condition and the current available therapies.

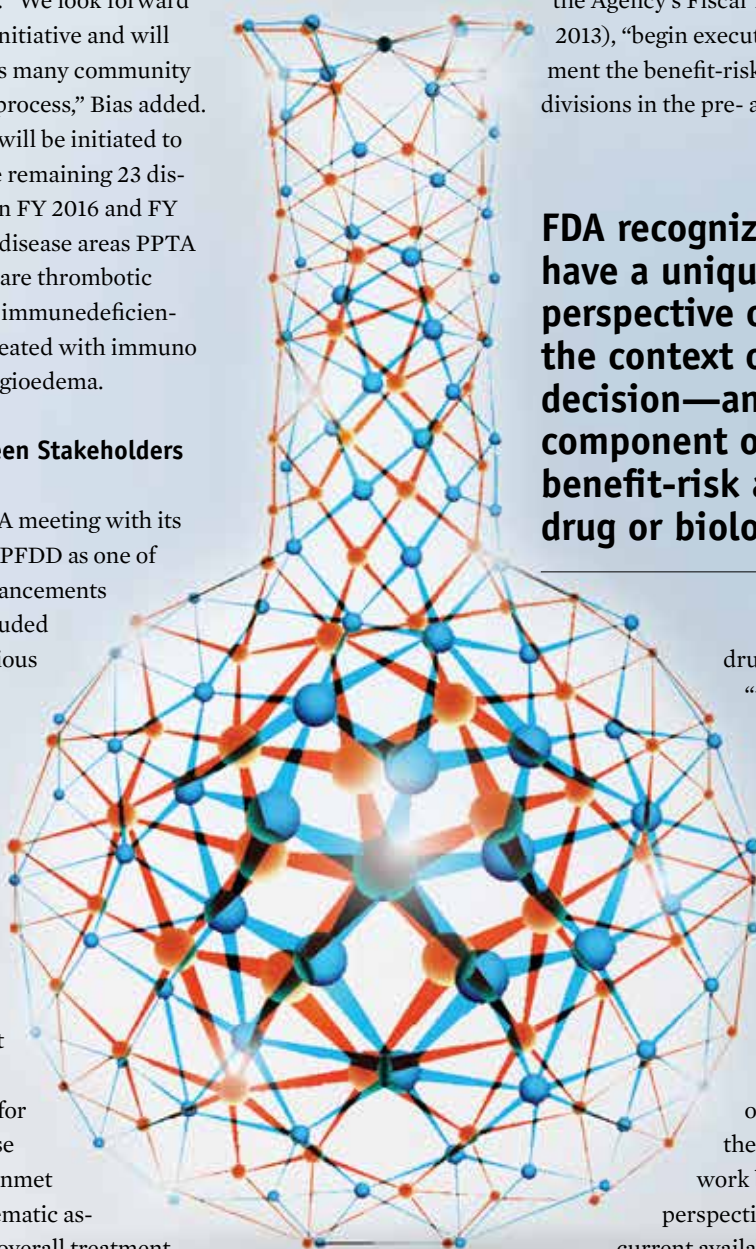


TABLE 1. PPTA Priority Disease Areas for Consideration
(excerpt of 39 preliminary disease areas)

September 2012 (39 preliminary disease areas listed)	April 2013 (16 of 20 disease areas selected)
Clotting disorders (e.g., hemophilia A (factor VIII deficiency) and von Willebrand disease)	Hemophilia A, hemophilia B, von Willebrand disease, and other heritable bleeding disorders. Meeting in FY 2014-2015
Thrombotic disorders (e.g., antithrombin deficiency and protein C deficiency)	Second public process will be initiated to determine remaining 4 disease areas (meetings in FY 2016-2017)
Primary humoral immunodeficiencies (e.g., common variable immune deficiency)	
Neurologic disorders treated with immune globulins (e.g., chronic inflammatory demyelinating polyneuropathy)	
Hereditary angioedema	
Alpha-1 antitrypsin deficiency	Meeting in FY 2014-2015

SOURCE: FEDERAL REGISTER

From Concept to Action


Over the period of PDUFA V, FDA will initiate a public process to nominate a set of disease areas that could benefit from a more systematic and expansive approach to obtaining the patient perspective on disease severity or unmet medical need. FDA will convene 4 meetings per year [Center for Drug Evaluation and Research] will host 17 meetings and [Center for Biologics Evaluation and Research] will host three meetings throughout PDUFA V) with each meeting focused on a different disease area [i.e. 20 areas during PDUFA V].⁸

On September 24, 2012, FDA published a preliminary list of 39 nominated disease areas for the patient-focused drug development initiative including clotting disorders, thrombotic disorders, primary humoral immune deficiencies, neurologic disorders treated with immune globulins, hereditary angioedema, and alpha-1 antitrypsin deficiency, as well as the criteria used for nomination. FDA invited public comment on the preliminary list through a public docket and at an October 25, 2012, public meeting where the Agency provided an overview of the initiative and discussion of the nominated disease areas.

FDA received over 4500 comments addressing over 90 disease areas from patients, patient advocates and advocacy groups, caregivers, healthcare providers, professional societies, scientific experts, pharmaceutical companies, trade associations such as PPTA, and others. In its comments, PPTA applauded FDA's listing of disease areas as potential candidates for the focus of public input, as well as the Agency's inclusion of rare diseases for which PPTA member companies provide lifesaving and life-supporting therapies, which the Association views as priority areas for consideration.

On April 11, FDA selected 16 of the 20 disease areas for the first 3 years of the 5-year initiative (remaining 4 areas for the last 2 years to be determined) (Table 1). Of note, in joint comments to the FDA docket on November 1, 2012, HFA, NHF, and

COTT suggested identifying hemophilia and von Willebrand Disease “as bleeding disorders rather than clotting disorders, since this is the more commonly used terminology”, a change that FDA adopted.

PPTA is encouraged by FDA's first steps to implement its PFDD initiative but recognizes that all involved, patients, the Agency, the Association, and others must remain vigilant. PPTA members are committed to ensuring the safety and availability of the medically needed, life-sustaining plasma protein therapies used by patients in the treatment of a number of rare and often genetic, chronic, and life-threatening diseases. PPTA will continue to support patients in their partnership with FDA in this important initiative. 

MARY CLARE KIMBER, *Manager, Regulatory Policy*

Endnotes

1. See FDA. (2013). Structured approach to benefit-risk assessment in drug regulatory decision-making: Draft PDUFA V implementation plan—February 2013: Fiscal Years 2013-2017, 13. Retrieved on May 8, 2013, from <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM329758.pdf>
2. Id.
3. See Id.
4. FDA. (2010). Stakeholder meeting on PDUFA V reauthorization, 3. Retrieved on May 8, 2013, from <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM222797.pdf>
5. See FDA. (2012). PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2013 through 2017, 24. Retrieved on May 8, 2013, from <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM329758.pdf>
6. Id.
7. See FDA. Structured approach to benefit-risk assessment in drug regulatory decision-making, 7 fig. 1.
8. FDA. PDUFA Reauthorization Performance Goals and Procedures, 25.



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WHAT DOES **ACA Implementation** MEAN FOR PLASMA PROTEIN THERAPIES?

BY BILL SPEIR AND CARRIE FIARMAN

THE PATIENT PROTECTION AFFORDABLE CARE ACT, commonly referred to as the Affordable Care Act (ACA), is a combination of Public Laws 111-148 and 111-152. The ACA was signed into law in March 2010 and puts in place comprehensive health insurance market reforms that are being implemented over the course of four years and beyond. While the details of these reforms are still falling into place, there are a few reforms in particular that patients who use plasma protein therapies will be paying close attention to as implementation continues into 2014.

Some reforms that are important to users of plasma protein therapies have already been implemented. Effective for health plan years beginning on or after September 23, 2010, young adults are now allowed to stay on their parents' insurance until they turn

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While many of these changes may be helpful to the patient communities who rely on lifesaving, unique plasma protein therapies, it is imperative to continue monitoring the rollout of these federal reforms to ensure they do not have unintended consequences on the rare disease community.

26 years old. In the past, insurance companies could search for an error or mistake on a customer's application and use this error to deny services when the customer got sick. The ACA put an end to that practice by making rescissions illegal. Insurance companies can no longer impose lifetime limits on insurance coverage, an essential benefit for those with lifelong chronic illnesses. Each of these reforms is vital and removes a barrier to patient access for those who often need on-going treatment.

As 2014 approaches, a number of additional insurance market reforms are scheduled to take place that aim to increase access to healthcare for all Americans, especially the chronically ill patients who depend on lifesaving plasma protein therapies. On January 1, 2014, the individual requirement to have insurance is scheduled to go into effect. Coverage will then be available to individuals and small businesses with up to 100 employees through the state-based Health Insurance Marketplace and the Small Business Health Options Program (SHOP) Exchanges, administered by a governmental agency or non-profit organization. Refundable tax credit advances and cost sharing subsidies will be available to certain eligible individuals to purchase insurance from the exchanges. In January 2014, the ACA will require insurance companies to issue and guarantee the renewability of health insurance policies regardless of one's health status or pre-existing condition. It will also prevent insurance companies from placing annual limits on the dollar value of coverage.

There is no doubt that the ACA, will increase access to insurance for individuals who rely on plasma protein therapies, but will the insurance be affordable? The ACA requires insurance companies to cover health benefits that they do not currently cover or that they offer in a limited capacity. This will increase premiums for the entire market. The total increase is expected to be more than 20%. However, for individuals who have chronic conditions, including individuals who rely on plasma protein therapies, the premiums under the ACA are expected to be significantly lower than what are offered in the current market.

In addition to lower premiums for individuals who rely on plasma protein therapies, the subsidies provided by the

government will lower premium costs for individuals as well. If an individual makes less than 400% of the poverty level (roughly \$45,000 for an individual), they qualify for premium assistance from the federal government. It is expected that the subsidies will make insurance more than 80% less expensive for some individuals receiving subsidies.

All the cost containment built into the ACA could have a negative impact on patient access to plasma protein therapies. The ACA will limit the insurance companies' ability to control costs. This will lead them to be more aggressive in areas where they can still impact costs. It should be expected that they will limit the number of specialty pharmacies which insured individuals may use to deliver their plasma protein therapies. It is also expected that they will become more restrictive regarding an individual's ability to get the specific plasma protein therapy they have been prescribed. To save money, experts predict patients will be limited to only a few products in a therapeutic class. This could result individuals receiving insurance, but being required to switch their specialty pharmacy or their plasma protein therapy.

Many of these reforms have the potential to be beneficial to users of plasma protein therapies; however, significant questions linger regarding implementation of the exchange and how individuals will participate in them. For example, it remains to be seen if certain necessary therapies will be covered under these new plans or if patients will have access to their specialist. While many of these changes may be helpful to the patient communities who rely on lifesaving, unique plasma protein therapies, it is imperative to continue monitoring the rollout of these federal reforms to ensure they do not have unintended consequences on the rare disease community. As regulations and guidelines are being released, PPTA remains committed to policies that preserve open access to all brands of plasma protein therapies in all therapeutic classes and in all sites of care in order to protect patient access. 🌐

BILL SPEIR, *Director, State Affairs*

CARRIE FIARMAN, *Manager, Federal Affairs*

HEALTH RELATED

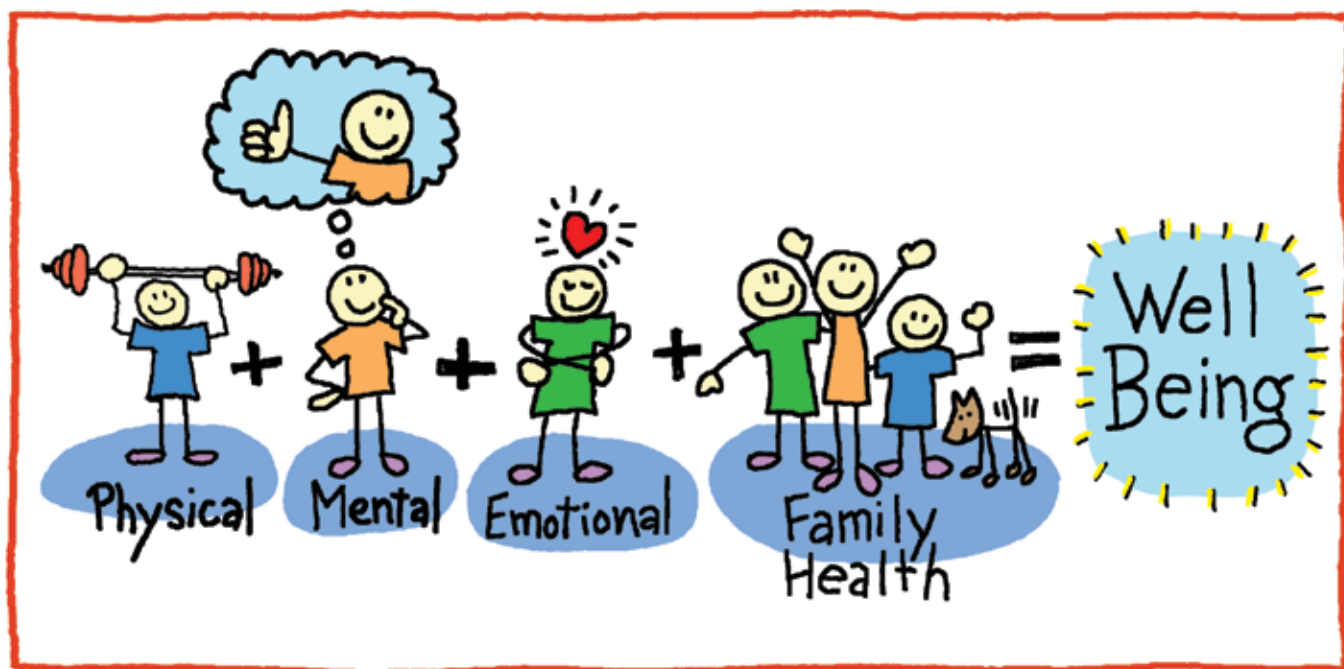
BY PROFESSOR ALBERT FARRUGIA

A Crucial Concept for Patients with Rare Plasma Protein Deficiencies

ACCORDING TO THE WORLD HEALTH ORGANIZATION (WHO), “Health is a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity”. Hence we encounter the concept of Health-Related Quality of life (HrQOL), which encompasses several aspects of health that are directly experienced by the person, including physical functioning, social and role functioning, mental health and general health perceptions. These concepts are crucial for patients with rare, chronic disorders such as people with hemophilia, immunodeficiency and other patients with plasma protein disorders.

Where Are We Now?

For many patients, the past thirty years of steady progression, enhancing the supply and safety of treatment products, has ensured that their lives are longer and better. With the enhanced life expectancy that the therapies provide, we now recognize that previously unknown problems can affect the patients. The early damage suffered through lack of treatment in e.g. hemophilia and PID can manifest in later life, leading to the need for more medical intervention. This obviously affects many aspects of HrQOL. The level to which this is recognized and treatment provided contributes to the variations we see in the use of plasma protein therapies. For example, the usage of Factor VIII has been studied relative to the prevalence of hemophilia in different countries (figure page 11). We observe that the prevalence increases as usage increases, up to a consumption of approx. 1 IU of Factor VIII per capita, and then the prevalence tends to level off. The



JOE SUTLIF

QUALITY OF LIFE

consumption of 1 IU per capita is what is estimated to be needed for a normal life-span for people with hemophilia. Consumption above this level reflects the capacity of a country to deliver a better HrQOL for patients through allocating Factor therapy for surgery, inhibitor tolerization and, above all, prophylaxis.

Measuring HrQOL

As the importance of HrQOL has increased, appropriate ways of measuring it have been developed. These methods all utilize a questionnaire of health related issues as their basis. They seek to quantitate HrQOL on a scale of one to zero, with one describing a state of perfect health and zero linked to death. The development of these questionnaires, called 'Utility Instruments' continues, in order to ensure sensitivity to different levels of HrQOL.

Why is HrQOL Important?

One value elicited from patients answering utility instruments is called a 'utility score'. These utility scores have many uses. One of the most important is their use in calculating the Quality Adjusted Life Year (QALY). One QALY may be defined as one year in perfect health, a useful measurement as it is the same for all conditions and for all patients. To calculate the QALY for a condition, one simply multiplies the utility score elicited for that condition by the number of years the condition prevails.

For example, let us say a person with hemophilia brought up on episodic therapy is asked about HrQOL at the age of twenty and returns a score of 0.7. Let us assume that this person will receive adequate episodic treatment for the rest of his life and will live for 75 years. For that period of time, he will accrue

$$0.7 \times 55 = 38.5 \text{ QALYs}$$

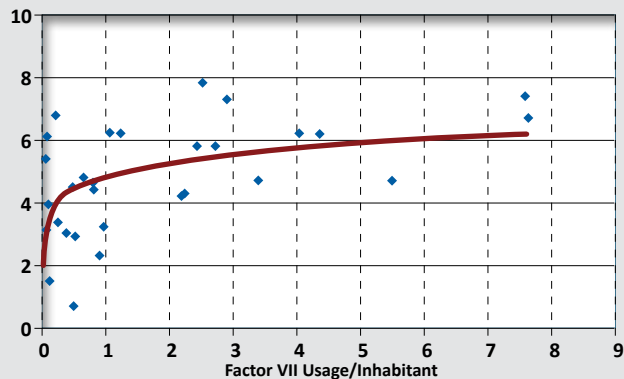
Let us now consider another patient who has been on prophylaxis from an early age and as hardly suffered from his condition. His utility score after questioning turns out to be 0.9. He will also live up to 75 years but he will accrue

$$0.9 \times 55 = 49.5 \text{ QALYs}$$

This kind of exercise is important because the calculation of the cost of a QALY is used by reimbursement agencies to determine whether a treatment gets reimbursed so that patients can access it. In the example above, the choice of prophylaxis versus

OBSERVED HEMOPHILIA: A prevalence and amount of Factor VIII used in 32 countries reporting to the World Federation of Hemophilia.

Prevalence of Hemophilia A per 100,000 inhabitants



Source: Evatt BL Haemophilia (2002), 8, 153-156.

episodic treatment leads to 11 additional QALYs over life. In most countries, if each of these additional QALYs were to cost more than \$50,000 to \$100,000, the treatment would be viewed as not sufficiently cost-effective and patients would find trouble in accessing it. I have described the case of hemophilia, but other conditions such as PID and AIAT deficiency are similarly positioned.

What can Patients Do?

As I have described, the elicitation of the crucial utility score is done through questioning patients. It is therefore crucial that patients are fully informed on the benefits of treatments which shape their HrQOL. Patients with chronic diseases are known to underestimate the gravity of their condition and to be reluctant to undertake new therapies when these are available—they do not want to 'rock the boat'. However, it is important for patients wishing to access better treatments to not underestimate their benefits on HrQOL, so that it is clear that the therapies are cost-effective and worthy of support.

At PPTA, we have undertaken collaborations with several patient organizations in order to develop utility measurements to inform cost-effectiveness analogies. One of these has been published (*Haemophilia 2013 Farrugia et al*) and others will be developed over the coming years. Centering the patient in these efforts is crucial, if quality of life is to be gained and retained. ☞

ALBERT FARRUGIA, PH.D, Vice President, Global Access

AUGMENTATION THERAPY TURNS 25 YEARS OLD

***Treatment helped build the
Alpha-1 community***

BY BOB CAMPBELL

IN 1962, Sten Eriksson was a 30-year-old resident physician on the staff of Malmo General Hospital in Sweden. He was also a research assistant to Carl-Bertil Laurell, head of the hospital's clinical laboratory.

Eriksson remembers a phone call.

"One day Laurell called me and said, 'Come up here, I want to show you something.' And what he showed me was a bunch of paper electrophoretic strips that he wanted me to check for subnormal concentrations of alpha-1 protein."

Electrophoresis is a technique, still used, that Laurell had perfected to study human proteins in the blood. Blood serum—the clear, pale yellow fluid left behind when blood clots—is applied to a special paper or gel. An electrical charge separates the proteins into five major categories, one of them being alpha-1 antitrypsin.

Laurell had noticed extremely small amounts of alpha-1 protein in some of the strips. The question was why this was so, and whether it was important.

Small Study, Huge Importance

So Eriksson and Laurell began a study. "I studied 1,500 unselected electrophoretic strips," Eriksson says. "I wanted to know how many cases of alpha-1 deficiency there could be."

The result was a 1963 article in the *Scandinavian Journal of Clinical & Laboratory Investigation* that became a landmark: the discovery of Alpha-1 Antitrypsin Deficiency (Alpha-1). The study, reporting on just five patients, three of them with

emphysema, gave Alpha-1 its name. With so few patients, Eriksson and Laurell did not make any definite conclusions. But they still came up with remarkably accurate suggestions about the nature of Alpha-1:

The deficiency seemed to be genetic—it ran in families; it had a "striking" connection to lung disease; and some people with Alpha-1 were able to maintain normal lung function. Many more scientific studies of Alpha-1 soon proved Laurell and Eriksson

correct in their description of Alpha-1 lung disease.

Alpha-1 is a genetic disorder characterized by low levels of or absent alpha-1 protein in the blood. Alpha-1 patients are at risk for severe lung disease as adults and liver disease at any age. Up to 3% of all people diagnosed with COPD may have Alpha-1. The condition can lead to severe disability and reduced life expectancy.

Therapy Played a Key Role

Some 25 years after the discovery of Alpha-1, a series of studies at the National Heart, Lung, and Blood Institute (NHLBI) led to a specific treatment: Augmentation therapy, which consists of intravenous infusions of concentrated alpha-1 protein, purified from the pooled blood plasma of healthy volunteers. The goal of augmentation is to increase the amount of protein in the blood and lungs to a level considered protective.

Approved by the U.S. Food and Drug Administration in December 2007, augmentation therapy became commercially available in 1988, and is now marking its 25th anniversary. Besides being a specific therapy for emphysema due to Alpha-1, augmentation became a key factor in helping to create a cohesive Alpha-1 patient community.





Sten Eriksson



John Walsh

“Augmentation therapy gave Alphas hope,” says John Walsh, president and CEO of the Alpha-1 Foundation. “It extends life and improves the quality of our lives.” Augmentation was made possible by the Orphan Drug Act, a 1983 law intended to help bring drugs to rare disease populations, Walsh pointed out.

And the Phase 4 trial of augmentation, which ran from 1989 through 1996, “is actually where the Alpha-1 community met

**“Augmentation therapy
gave Alphas hope.
It extends life and improves
the quality of our lives.”**

each other,” he says. “We met each other at clinical centers, got to know each other, kept in touch and formed support groups that led to the Alpha-1 Association in 1991. With 1,129 Alphas in that study by the National Heart, Lung and Blood Institute (NHLBI), we learned the importance of participating in clinical research and we mobilized ourselves around that theme.”

A healthy Alpha-1 research program was also made possible by augmentation therapy, Walsh says. “When the Phase 4 trial ended and the NHLBI told us they were ending Alpha-1 research, we decided the community had to take on the responsibility of funding research. So we created the Alpha-1 Foundation.”

Augmentation therapy also made it possible to create AlphaNet as “a social enterprise,” Walsh says. “In AlphaNet, we

could provide comprehensive disease management to Alphas that improved the quality of their lives. At the same time, AlphaNet’s fees for disease management developed a recurring source of revenue that supports the research mission of the Alpha-1 Foundation. It’s the ultimate in recycling our insurance dollars—to support research that is ultimately going to cure Alpha-1.”

Congress Marks 50th Anniversary

Alpha-1 delegates from 23 countries met in April at the 4th International Alpha-1 Patient Congress in Barcelona, Spain, to mark the 50th anniversary of the discovery of Alpha-1 by Laurell and Eriksson in 1963.

Professor Eriksson spoke at the opening of the Congress and was honored for his landmark scientific achievement.

The delegates considered awareness of Alpha-1 to be the most important need for the Alpha-1 communities in their countries. Access to care, especially augmentation therapy, was the second most important priority named. The need for an organized Alpha-1 community in many countries and the need for better communication between Alpha-1 communities worldwide were also major concerns of the Alphas at the Congress.

The Alpha-1 Foundation provided organizational and financial support for the Congress and the International Research Conference on Alpha-1 held in conjunction with the Congress.

As the Congress ended, Walsh suggested an international organization to help support communications between Alphas. He proposed to set up a website for the purpose, and promised to share the latest research information on augmentation therapy as soon as it becomes available. ☞

BOB CAMPBELL, *Alpha-1 Foundation, Communications Director.*
Adapted from Alpha-1-To-One magazine



An Alpha-1 Pioneer: **JERRY TREICHEL**

BY BOB CAMPBELL

AT 41, **JERRY TREICHEL** was an electrical contractor, a healthy outdoorsman, hunter and golfer. Hunting in the mountains of Nevada one day in 1983, he found himself too short of breath. “I couldn’t keep up with the other guys,” he remembered.

After he went back to his home in Las Vegas, his doctor sent him to a pulmonologist who diagnosed Alpha-1 Antitrypsin Deficiency the first time, which was nearly unheard of in 1983.* The doctor told Treichel there wasn’t much he could do about Alpha-1.

Then Treichel’s mother read about an Alpha-1 study at the National Institutes of Health (NIH) in Bethesda, MD. The study would test augmentation therapy—taking intravenous infusions of concentrated alpha-1 protein, purified from the pooled blood plasma of healthy volunteers. The goal was to increase the amount of protein in the blood and lungs to a level that was considered protective.

Treichel applied, was accepted and began his study infusions in January 1986. The U.S. Food and Drug Administration approved the first augmentation therapy, Prolastin,* and Treichel began to receive the commercial infusions in January 1988.

This augmentation therapy celebrates 25 years on the U.S. market this year and Jerry Treichel has been receiving the therapy for 27 years.

Treichel has been an Alpha-1 support group leader and patient advocate for many years. He recently gave a talk on his



This augmentation therapy celebrates 25 years on the U.S. market this year and Jerry Treichel has been receiving the therapy for 27 years.

experiences to a group of employees of Grifols. He reunited there with Karen Keogh, who was the specialty representative in Las Vegas in 1988, the year the therapy was launched.

Treichel turned 71 on Feb. 24, 2013. He needs supplemental oxygen when he’s sleeping and on airplanes. His pulmonary test scores have been stable for many years. “I don’t know if I would still be here today if not for this therapy,” he says. ☞

*Today, Alpha-1 patients have multiple therapy options.

BOB CAMPBELL, *Alpha-1 Foundation, Communications Director.*
Adapted from Alpha-1-To-One magazine



MANDATORY REBATES CONTINUE TO BE AN ISSUE IN GERMANY

BY STEFAN GRAFENHORST

FINANCIAL CRISES ARISE REGULARLY and they quite often lead to economic recessions. In most cases, recessions cause unemployment, which in turn, results in fewer employees paying into social insurance schemes. Consequently cost pressure on social welfare services increases. Governments must respond accordingly and often do so with austerity policies.

In 2008, Europe faced a financial crisis and its governments were consumed by cost reduction strategies, especially in the healthcare and pharmaceutical sectors.

Facing a weak economic forecast in 2010, the German government decided that manufacturers of patented medicines needed to contribute to the sustainable funding of statutory health insurances to decrease their cost pressures. Since August 2010, manufacturers of patented medicines which do not belong to the fixed price group of drugs incur a 15% mandatory rebate. A further price moratorium threatens that the mandatory rebate is paid on the basis of prices negotiated before August 1, 2009 rather than current prices. The decision to cut prices and introduce a mandatory rebate was made under the assumption that there would be a funding gap for the statutory health insurances of approximately 11B€ in 2011. The goal of the price intervention was to control the increased spending by the German statutory health insurances. The Social Code Law V (§130a Section 4) which establishes the regulative frame for the rebate regime foresees an annual review of the regulation and its impact by the Federal Health Ministry.

But unlike other European Union member states such as Italy, Spain, Portugal or Ireland, the German economy recovered quickly from the 2008 financial crisis. The current unemployment rate in Germany is at a comparable low of 7.3 % and the total number of employees is at 41.42M people¹ which is more than 2008. The positive economy has led to massive surpluses of statutory health insurances in 2012. According to the Federal Health Ministry, the statutory health insurances made a surplus

of 5.07B€ in 2012.² The Ministry states that the total reserves of the statutory health insurances are 15.2B€. Given this, the argument that statutory health insurances expenses are not in line with income is no longer valid as economic development has made cost pressures vanish. Therefore it comes as a surprise that the Federal Ministry refused to skip the increased mandatory rebate during their annual review at the end of 2012. The economic situation in Germany can no longer be used as a justification for this government intervention even though the government argues that cyclical risks in the Euro zone still persist. Overall it seems unjustified that drug producers alone contribute to the consolidation of the statutory health insurances.

At least, the existing 16% mandatory rebate regulation will expire by December 31 of this year and will decrease to the pre-2010 level of 10% for patented medicines. Coincidentally Germany will hold federal elections in September this year. Whether the current coalition remains in power or a new government coalition takes over, the issue of the mandatory rebates will quickly be on the agenda. Any new regulation should carefully consider the implications for manufacturers of drugs for rare diseases. Cost-saving policies should not threaten patients' access to the therapies they need and also should not undermine the industry's ability to invest in research and development in order to develop therapies that can save the lives of people. ☞

STEFAN GRAFENHORST, *Senior Manager, Germany*

Endnotes

1. See "Statistisches Bundesamt" at: <https://www.destatis.de/DE/ZahlenFakten/GesamtwirtschaftUmwelt/Arbeitsmarkt/Erwerbslosigkeit/ErwerbsloseILOKonzept/Aktuell.html>
2. See press release of the Federal German Health Ministry at: http://www.bmg.bund.de/fileadmin/dateien/Pressemitteilungen/2013/2013_01/130307_PM_Finanzentwicklung_der_gesetzlichen_Krankenversicherung_2012.pdf

GERMAN DONOR SETS RECORD WITH 1300 DONATIONS

BY ALEXA WETZEL

Helping patients with rare diseases, his personal well-being and regular medical checkups motivate Heinz Behrmann.

THE CSL PLASMA CENTER in Kiel celebrated Heinz Behrmann's record-breaking 1300 plasma donations October 11, 2012. At age 59, Behrmann's 34 years of commitment to donating plasma is impressive. He has been a dedicated donor at CSL Plasma center in Kiel (Schleswig-Holstein—Germany) who recognized his contribution with a certificate and gift basket.

In 1978, at the age 25 Behrmann donated plasma for the first time. One of his friends was a nurse in a plasma center and had explained the need for plasma donations. During his first donation, he was so nervous that he almost fainted, but he was not discouraged by the experience and continued to donate.

Behrmann is motivated by helping patients with rare diseases, his personal well-being and regular medical checkups. In 1986, he began long-distance running and started to practice for marathons. In 1991 he achieved a personal best time of 2:50:41. He frequently, runs as much as 20 km on the days he donates.

A donor's blood pressure and heart rate are measured, as well as other medical screenings prior to each donation to ensure that only healthy donors are



Heinz Behrmann

allowed to give plasma. These regular examinations prior to each donation allow Behrmann to stay healthy and in good shape and avoid overtraining and related injuries.

Behrmann still runs marathons but he is no longer chasing record times. He focuses primarily on the quality and the fun of running. He has completed over 252 marathons and ultra-finishes and since 1997 has worked as a volunteer to organize the annual "Three Lighthouses Run" in Kiel.

Behrmann plans to continue donating plasma and saving lives of others as long as his health permits. He notes that with age health becomes more and more important. Patients whose well-being depends on these therapies manufactured from human plasma rely on the generous donations made by committed individuals. Behrmann keeps a couple of brochures about plasma donation in his office, hoping to encourage others to donate. ☺

ALEXA WETZEL, *Junior Manager, Source Europe*

JEFFREY MODELL FOUNDATION PLEADS FOR SCID SCREENING

BY CARRIE FIARMAN

ON MARCH 13, VICKI MODELL, Co-founder of the Jeffrey Modell Foundation (JMF) testified in front of the House Labor, Health and Human Services (LHHS) Appropriations Subcommittee in support of \$2M in funding for the Health Resources and Services Administration (HRSA) Genetic Services Branch. This vital funding would help save the lives of the one in 30,000 babies born each year with Severe Combined Immunodeficiency (SCID), a rare genetic disorder resulting in little or no immune system.

SCID represents a severe defect in T- and B- lymphocyte development resulting in marked susceptibility to severe and complicated infections. These white blood cells are necessary for normal immunity. The onset of infection usually occurs in the first six months of life. SCID is considered to be the most serious of the primary immune disorders.¹

Babies born with SCID appear normal at birth, but are at high-risk for developing life-threatening infections. Hospitalizations in the pediatric intensive care unit and the costs of care in the first year of life can be from \$2 to \$4 million for a baby with SCID. If identified within the first three-and-a-half months of life, a bone marrow transplant has a better than 95% success rate to cure this fatal disease. New and inexpensive screening technology is more than 99% accurate and only costs \$4.00 per baby.²

Modell testified that states have begun to adopt screening initiatives, but cannot do it alone without the federal



Vicki Modell

This vital funding would help save the lives of the one in 30,000 babies born each year with Severe Combined Immunodeficiency (SCID).

government's help. While additional funding was not allocated in H.R.933, which funded the federal government through the end of the fiscal year (FY) 2013, the emotional appeal from JMF

was heard by Sen. Tom Harkin (D-IA), Chairman of the Senate Appropriations Subcommittee on Labor, Health and Human Services, and Education. Chairman Harkin offered an amendment to H.R. 933 that included the \$2M request from JMF, but it failed to pass. Because the amendment made additional changes to the Labor, Health and Human Services, and Education accounts, critics argued that inclusion of this amendment would have jeopardized the legislation's chances on final passage. ☞

CARRIE FIARMAN, *Manager, Federal Affairs*

Endnotes

1. Jeffrey Modell Foundation. (2013). Severe Combined Immunodeficiency. Retrieved May 3, 2013, from <http://www.info4pi.org>
2. Vicki Modell (Co-founder, Jeffrey Model Foundation). "Public Witness Testimony before the House Appropriations Committee, Subcommittee on Labor, Health and Human Services."



COMPENSATED

BY JOSHUA PENROD AND SONIA BALBONI

SOURCE PLASMA DONOR COMPENSATION should be separated from discussions about product safety. For the better part of two decades, there have been no reported transmissions of Hepatitis C, Hepatitis B, or HIV in any plasma product manufactured by a PPTA member company. Despite this lengthy record of safety, presumption and misinformation sometimes prevail when questions relating to compensated donation arise. Recent stories in the Canadian media have brought this to light. Many in the industry and our observers have taken note of recent public policy debates in Canada relating to the potential opening of plasma centers. The focus of the media reports has been donor compensation, with some critics assailing the possibility that the Canadian government could be disregarding the lessons on safety learned many years ago.

Nothing could be further from reality.

As is well-established through the peer-reviewed literature, exhaustive licensing and inspection procedures, and implementation of voluntary industry standards programs, the plasma protein therapeutics industry has demonstrated extraordinary leadership in helping ensure the highest possible levels of safety. Donor screening and selection, testing, physical assessment, computerized management, voluntary industry standards, production processes, and pathogen inactivation have all combined to create therapies of uncommon safety and high impact.

In Canada, if safety is consistently and truly the issue, that question has been well-settled for many years and the critics need only look to the volumes of data and publications underscoring the facts. The conversation would be better served if,

instead, the issues themselves were discussed forthrightly and openly. To that end, we invite conversation about donor compensation and the practices of the industry.

Beyond the questions about safety, however, are politically-driven goals that actually do great harm to patients. Late last year, in a publication by the European Blood Alliance and republished in the America's Blood Centers newsletter, the World Health Organization again made headlines with its proclamation insisting on the "principle" of altruistic donations. The reasons given in the proclamation include safety and ethical concerns.

The ethical concerns have been discussed at length in many different publications, including a recent article in *The Source* (see Penrod and Farrugia, Winter 2012). Just as in the



DONORS AND SAFETY

conversation about safety, volumes can be written about the debate over donor compensation—in fact, volumes already have been written. Without attempting to re-hash the debates, or again write what was already shown, we can say that our industry's detractors, or those who do not fully understand our industry, often conflate these concerns, creating a cloud of uncertainty. Sadly, the result is that worry and misunderstanding triumph over patient care. The results may occur in several forms, including international trade barriers; self-sufficiency policies targeted toward erroneous ends, and generalized concerns about safety.

For instance, political preferences can lead to obstructions to international trade, which may be cloaked by safety discussions. The true reason behind an obstruction could actually be a hasty reaction to public perceptions on the practice of compensated plasma, or even a regulatory or statutory deployment favoring protection of a domestic industry. It is sometimes difficult to isolate one rationale behind these measures, but it is simplified by knowing that whatever the reason, patients suffer.

Hand in hand with trade barriers comes a rationalization in the form of an avowed self-sufficiency policy. While from the public health and national security standpoint, a self-sufficiency policy makes sense for blood components with short shelf lives, the complexity of the global plasma protein therapeutic industry makes such policies result in availability issues. In turn, this increases government and payer pressure on diagnosis, substituting a political and economic goal instead of true

patient need. When one speaks of ethics and systems favoring patient care, one must consider the need of patients and empirical medical findings.

As discussed above, viral safety has been and continues to be a top focus for the industry. Donor selection, examination, screening, assessment, and testing processes reduce risk by orders of magnitude. Companies participating in PPTA's International Quality Plasma Program (IQPP) have the added assurance of a third-party audit system and compliance with the Viral Marker Standard, the Qualified Donor Standard, the Community-Based Donor Standard, the Donor Education Standard, and others. All of these steps take place prior to the plasma even entering further production; during the fractionation process, robust and sophisticated pathogen inactivation steps occur which eliminate further risk. Companies adhering to the PPTA Quality Standards for Excellence and Leadership (QSEAL) further benefit.

The industry has earned a strong safety record for its robust practices including screening, testing and manufacturing. Concerns about compensation remain; however the practice will prevail as we continue to recognize the effort that it takes to be a committed, regular donor. To *not* recognize such commitment would be nonsensical. We are proud of our donors' dedication and for that reason compensation will remain a vital part of our industry. 🌐

JOSHUA PENROD, *Vice President, Source*

SONIA BALBONI, *Manager, Source and Standards*

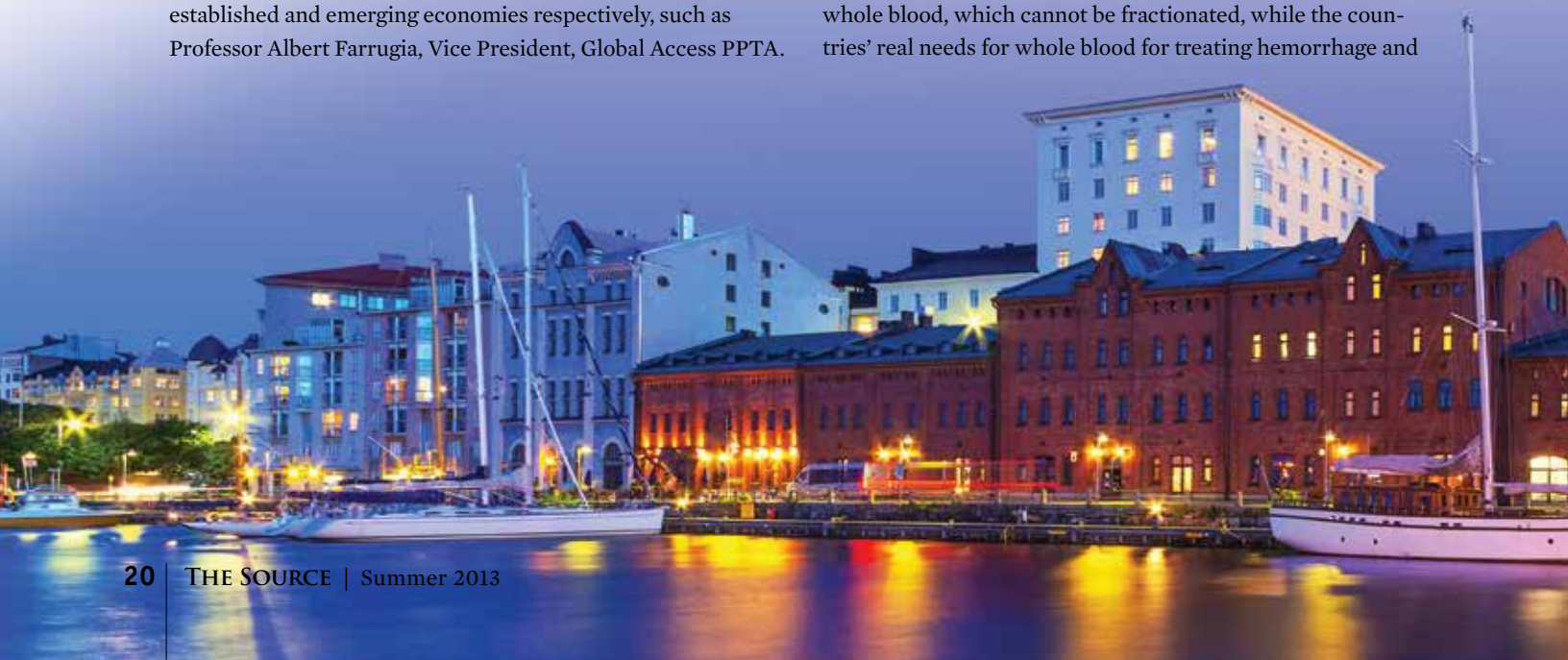
IPFA/PEI CELEBRATES 20TH INTERNATIONAL WORKSHOP

BY ILKA VON HOEGEN, PH.D AND PROFESSOR ALBERT FARRUGIA, PH.D

THE INTERNATIONAL PLASMA FRACTIONATION ASSOCIATION (IPFA) and Paul Ehrlich Institute's (PEI) annual International Workshop on *"Surveillance and Screening of Blood Borne Pathogens"* is justifiably one of the major events on the blood safety and availability calendar. Over the past 20 years, it has achieved a reputation for scientific excellence and engaging debate, and its faculty draws from the main key opinion leaders in blood and plasma product safety and related issues. The event welcomes people with diverse views and rapidly makes its full proceedings and presentations available in the public domain, sharing and preserving valuable insights on key issues.

This year's 20th anniversary event was held in Helsinki to commemorate the first meeting in that very city in 1993, featured not only the traditional reviews and assessments on pathogen safety and the threats from established and emerging agents, but also covered, as in previous events, other topics of wider interest. This year's particular focus on availability and potential use of plasma in developing countries produced a vibrant session which included two speakers each from the established and emerging economies respectively, such as Professor Albert Farrugia, Vice President, Global Access PPTA.

Speakers strongly advocated that the path of the rich countries is not necessarily the right one and should not be imposed on economically restricted countries. While some medium income countries have developed blood component therapy policies and are fractionating suitable recovered plasma into plasma protein therapies, low income countries are barely able to meet the basic health needs of their population. In the meantime, imposed policies are resulting in the removal of plasma from whole blood, which cannot be fractionated, while the countries' real needs for whole blood for treating hemorrhage and



malaria are imperiled. One of the results of this misguided policy is that a lot of plasma that could be fractionated is discarded.

For those countries with a regulatory system in place to ensure that plasma is suitable for fractionation, contract fractionation is an established tool. Eventually, domestic fractionation can be established. Some medium income countries such as Brazil or Iran are in the process of such a transition from contract to domestic fractionation. But often domestic fractionation is not a viable option, because of the amount of available plasma is too small and the financial implications of establishing plasma supply and a fractionation capacity are prohibitive.

“Provision of blood and Blood Components are a national Responsibility. Production of plasma derived medicinal products is an international or global issue” was stated by Dr. Jean Emmanuel, Head of the Zimbabwe Blood Service and former head of the Blood Transfusion area of the World Health Organization (WHO). The World Health Assembly 2010 Resolution aims to encourage countries to develop national blood systems and to procure plasma suitable for fractionation into plasma protein therapies. Of course, it was anticipated that undersigning developed countries and regions including the European Union (EU) would assume their share of the responsibilities. Such undertakings are now at risk because of the global economic climate, while funded projects appear to have a tendency to impose Western norms on emerging countries. Somewhat ironically, when the Resolution was adopted the EU was in the process of finalizing the EU GMP Annex 14 which only allows contract fractionation by EU based manufacturers when the plasma complies almost exactly with EU requirements.

One of the most enlightening presentation was

delivered by Mike Busch, M.D., who gave an overview of issues addressed at workshops since 2004. It is interesting to see how technology has evolved to control either existing challenges to the safety of blood and plasma protein therapies or to respond to arising threats such as West Nile Virus (WNV) or variant Creutzfeldt-Jakob Disease (vCJD). At every workshop developing world initiatives and the specific needs of developing countries were addressed. There is a fundamental difference between the basic needs of developing countries versus the level of sophistication in developed countries, and consequently, one cannot apply the same rules. An interesting observation is that globalization often implies that the concerns of developing countries may also become a headache for the developed world as pathogens have become global travelers. In response, developing countries adapt their arsenal of policies and research capacity to understand the biology of the pathogen and develop methods for their detection and removal/inactivation. Thereby and almost unintentionally in the effort to protect their national blood systems developed countries assist developing countries in responding to their specific challenges to protect public health.

Justifiably, the question arises whether all the initiatives over the years, including the WHO Global Collaboration on Blood Safety, Safe Blood for Africa and others or, indeed, the very World Health Assembly 2010 Resolution have had a measurable impact on the blood systems of developing countries or whether observed positive developments have been in these countries irrespective of, and sometimes perhaps despite of, the international network. ☞

ILKA VON HOEGEN, PH.D., *Senior Director, Quality and Safety*

ALBERT FARRUGIA, PH.D., *Vice President, Global Access*



REDESIGNED WEBSITES OFFER EXPANDED INFORMATION AND ADDED BENEFITS

BY LISA LOVULLO

IN ORDER TO BETTER MEET THE INFORMATION NEEDS of members, policymakers, the media and the general public PPTA has completed a major redesign of the Association's website and the Donating Plasma websites. The project was undertaken to better represent the full scope of the Association's work, employ state-of-the-art web technologies and search engine optimization strategies to maximize traffic.

PPTAglobal.org features a modern design, improved navigation, significantly expanded content, video enhancements and more. Particular effort has been made to balance and align content between Europe and North America. The Association's work in Asia and other parts of the world have been added.

A new Topics section defines the Association's six strategic goals: Patient Access, Plasma, Regulatory Policy, Image and Credibility, and Global Access. In addition, these are supported by an archive of official health policy documents. Health Policy

documents are sorted by Europe, U.S. Federal and U.S. State. Each provides a description of the advocacy issues in which the Association is engaged and position papers, statements and other official documents are categorized within topical area.

One of the more noticeable content additions is in Regulatory Policy. This tool is organized by geographical region and documents are sorted by regulatory agency or authority. Considerable effort has gone into defining the scope and importance of the Association's regulatory work. New areas on Pathogen Safety and Emergency Preparedness complement the regulatory content.

All meetings and events have been consolidated into one area including the International Plasma Protein Congress, the Plasma Protein Forum, Regulatory Workshops, International Plasma Awareness Week, and Sponsored Symposia. An enhanced events calendar provides increased functionality.

Other major enhancements include a new Membership




Area, an Advocacy Toolkit and the Plasma Products Literature Databases: Albumin, IVIG, Alpha-1 Antitrypsin and Clotting Factors. These databases provide a valuable research for clinicians and researchers working with plasma protein therapies.

Content experts throughout the Association have contributed to the site's development and will continue to consult to ensure that the site remains accurate, up-to-date, and a dynamic resource.

The Donating Plasma websites (in English, German and Czech) have also been redesigned to provide a better experience and more simplified information to both plasma donors and prospective donors. The concept was to design sites that could transcend cultures, language and educational levels by answering four questions: Who? How? Why? and Where? Site development was done in consultation with the Source Division and SIPC Committee leadership. The sites reinforce branding being used at collection centers throughout the world including the IQPP logo, a consumer friendly display version of

the IQPP standards, a worldwide database of collection centers and the global campaign for the International Plasma Awareness Week.

Finally, the PPTADeutschland site is also being redesigned and will launch this summer and complement the design and navigation of the global website. PPTA believes these websites sites reflect positively on our Association, our member companies and meet the needs of our various constituencies.

The sites are supported in the following browsers: (IE8+, Chrome15+, Safari5+, Firefox5+ and Opera10+). 

LISA LOVULLO, Senior Manager, Communications

PPTA Websites

pptaglobal.org
donatingplasma.org
dieplasmaspende.de
dieplasmaspende.at
darovaniplazmy.cz
pptadeustchland.org

PPTA Auxillary Sites

find-id.net
nddr.com
patientnotificationsystem.com
statepatientaccesscoalition.com

EUROPEAN STAKEHOLDERS CONVENE IN DUBLIN

BY LAURA SAVINI

 **REPRESENTATIVES FROM EUROPEAN ORGANIZATIONS** met with PPTA members and staff at the International Plasma Protein Congress (IPPC) held in Dublin, Ireland to discuss current and future priorities and to identify opportunities for collaboration.

Patient Organizations

Representatives from patient organizations provided an overview of their priorities. The majority are focused primarily on raising awareness and improving diagnosis and access to treatment.

HAEI International, the International Organization for C1 Inhibitor Deficiencies held its first congress in May 2012. One of their main goals of the organization is to define the prevalence of the condition. They also celebrated HAEI Day Initiative on May 16.

The Guillain-Barré Support Group is also focused on advocacy and awareness. They take part in GBS/ CIDP Foundation International's work to facilitate patient access in India, Pakistan and Africa.

Access to treatment is also a priority for patients suffering from primary immunodeficiency disease. Representatives from the International Patient Organization for Primary Immunodeficiency (IPOPI) outlined issues such as lack of product choice, unstable product supply due to market withdrawal and



limited access to treatment in multiple European Union (EU) countries. IPOPI is also working towards getting a resolution from the European Parliament on newborn Severe Combined Immunodeficiency (SCID) testing. The World Federation of Hemophilia is also focused on patient access and diagnosis through its "Close the Gap" campaign. This action marks the Federation's 50th Anniversary and looks at how to introduce, improve and sustain care in various regions of the world.

WFH noted that even in Europe, patients still experience in certain circumstances some difficulties in obtaining treatment.

PPTA Europe presented an overview of its priorities and work. Activities supported by PPTA focus on increasing awareness of conditions related to plasma protein therapies, for example such as FIND-ID, a German physician-led network that aims at raising awareness and increasing the diagnosis of

INSIDE PPTA

primary immunodeficiency in Germany, a country with a particularly low prevalence rate compared to neighboring countries.

PPTA also supports projects that generate further evidence for the diagnosis and treatment of plasma protein related conditions, such as the registry of the European Society for Immunodeficiency which gathers information on more than 1600 patients in 28 countries. The registry, started in 2003 and managed by the University Medical Centre in Freiburg, Germany, collects both epidemiological and demographic data. The next steps for the registry will be to collect additional data such as quality of life and clinical protocols.

Moreover, the PPTA staff closely monitors developments in the European health policy environment that have an immediate impact for PPTA members such as developments related to the “Blood Directive”. The European Commission is currently carrying out a study on the availability of blood, blood components and plasma derivatives to European patients. Following exchanges with the European Commission, it appears that this study will be used as a basis to evaluate whether Directive 2002/98/EC setting the standards of quality and safety for the collection, processing and distribution of blood and blood components will need to be revised.

Furthermore, the Association carries out work on health technology assessments (HTAs) and also monitors their impact on European Health Policy. During the event, Professor Albert Farrugia made a compelling case for engaging with patient groups to generate data when HTAs are used to inform decision-makers on therapeutic choice. HTAs are used widely in taxpayer funded systems in Europe, Australia and Canada to justify barriers to access. In Europe, for example, there has been increased cooperation among Member States with regards to HTAs, in particular through the EUnetHTA initiative and its work with the EMA.

Finally, PPTA European members actively involved in charting a course for the first ever International Plasma Awareness week, (October 13-20). This event is designed to raise awareness about the source plasma collection, recognize the contributions made by donors and increase understanding about importance of plasma protein therapies for patients.

In conclusion, it is evident that both PPTA and patient organizations are still very much focused on advocacy, patient access to treatment and improvement of diagnosis. The Association intends to maintain fruitful dialogue with patient groups. ☞

LAURA SAVINI, *National Affairs Manager*

CAPITOL HILL FLY-IN



PPTA HOSTED A RECEPTION the evening before the Association’s Capitol Hill Fly-In where Rep. Kevin Brady (R-TX) and Rep. Doris Matsui (D-CA) were honored for their leadership and commitment to patient access to plasma protein therapies. The reception was attended by numerous Congressional staff, PPTA members and stakeholders.

Correction

In the last issue of The Source, the university affiliation of Erik E. Berntorp, M.D., Ph.D, the winner of this year’s Hilfenhaus Award was incorrect. The correct affiliation is Lund University, Malmö Centre for Thrombosis and Haemostasis, Skåne University Hospital, Malmö, Sweden

COMMITTEE SPOTLIGHT

BY ALBERTO GIUMMARRA

HEALTH POLICY STEERING COMMITTEE (HPSC)


The PPTA Health Policy Steering Committee (HPSC) was established with the intention of marshaling the public affairs expertise of PPTA Europe and its members to accomplish PPTA priorities, with a particular focus on patient access, free trade and image and credibility.

During the last decade the PPTA HPSC has seen its tasks and responsibility grow in parallel with the growing influence of EU institutions in health policy. It contributed to the success of several PPTA European activities. In particular, the HPSC contributed to “*EU Call for Action on Rare Plasma Related Disorders*”. This project was launched in 2009 and today it is supported by more than 20 Members of the European Parliament committed to on rare diseases and patient access.

Committee members have an established an excellent network in Brussels, are well seasoned in EU advocacy and possess a deep understanding of blood and plasma policy in Europe. They represent the interests of Association with the European Parliament the EU Commission and Member States permanent representations. Through its regular outreach meeting program, the HPSC has been able to raise awareness about patient access to treatment inside EU institutions and to deliver PPTA policy messages on key EU health legislative dossiers to the majority of the highly influential policy makers on blood policy.

The Committee is also involved in PPTA Global Access Health Technology Assessment activities and (HTA) models for plasma protein therapies. It considers HTA a critical component of the EU health policy agenda and expects HTA's to be increasingly important at EU level due to the implementation of the EU cross-border healthcare Directive. The HPSC has developed a position paper, summarizing the findings of the PPTA Global Access team. It will continue to expand its understanding of HTA issues, working alongside the PPTA experts. In addition, the committee is working on the recognition of PPTA Europe as stakeholder into the EU network for Health Technology Assessment (EUnetHTA) which brings together all main stakeholders and public agencies involved in HTA at both EU and national level.

Today, the HPSC is fully engaged in the development of a PPTA strategy for the Blood Directive revision. It recently developed a position paper detailing the current issues faced by European patients in accessing plasma protein treatments

and the impact that the revision of Directive 2002/98/EC could have on the European plasma protein sector. Also, its efforts are focused on providing strategic advice and on implementing this strategy to advocate the industry position. 

ALBERTO GIUMMARRA, *Junior Manager Health Policy*

HEALTH POLICY STEERING COMMITTEE

Karl Petrovsky
Baxter (Chair)

Ruediger Gattermann
CSL Behring (Vice-chair)

Giovanni Rinaldi
Kedrion

Peter Pustoslemsek
Biotest

Isabel Henkel
Grifols

Alberto Giummarra,
Laura Savini
PPTA Liaisons

STAFF

MARY CLARE KIMBER

Manager, Regulatory Policy

How long have you served at PPTA?

In July, I will begin my fourth year of service at PPTA. When I joined the Association three years ago, I brought my perspectives as “attorney,” “teacher,” and “mathematician,” as well as “child,” “sibling,” and “spouse.” Since then, I have welcomed my first child, now 1½ to the world and have the added perspective as “parent.”

What do you focus on in your role as Manager, Regulatory Policy?

As PPTA's Manager, Regulatory Policy, I liaise with and represent the Association before the regional authorities that regulate the plasma protein therapies industry, primarily the Center for Biologics Evaluation and Research in the U.S. Food and Drug Administration, an agency of the U.S. Department of Health and Human Services. My work includes identifying and prioritizing key regulatory policy issues; advocating for rational policies that impact industry; monitoring, assessing, and guiding development of new and potential regulatory policies; and developing regulatory initiatives and alternatives to advance stakeholders' interests.

Tell us about your background.

I have not ventured far from the east coast of the U.S., living as far north as Providence, Rhode Island, where I earned a B.A. in Mathematics from Providence College, and as far south as Williamsburg, Virginia, where I earned

a J.D. from America's oldest law school, William and Mary. My roots are in the upper Chesapeake Bay in Maryland, a two-hour drive from Washington, D.C., where my family has run a sightseeing cruise boat business for the last 20+ years. After working on and around the water during my teenage years, I moved after college to the mathematics classroom, and after law school to the negotiating table. I ultimately settled in the D.C. area, where my legal work shifted to Federal agency litigation and research for approximately five years immediately prior to my joining PPTA.

What is your proudest professional achievement?

I am most proud of my role in helping to organize two PPTA Regulatory Workshops since joining the Association. Held annually in conjunction with the PPTA Plasma Protein Forum, the workshops reflect Association member commitment to ensuring the safety and availability of medically needed life-sustaining plasma protein therapies, as well as staff commitment to helping members ensure timely, compliant regulatory practices through industry education. A joint effort of the Source Plasma and blood communities, global regulators, and other biologics professionals, our 2012 Regulatory Workshop “US/EU Quality/Compliance Challenges and Solutions” included regulator praise for industry compliance/quality efforts, Association member case studies on quality tools to reduce human



errors, and interactive presentations by FDA and industry on resolving conflict during and after inspections. On June 10 our t 2013 PPTA Regulatory Workshop “Industry’s Commitment to Safe and Healthy Donors” explored on-going industry efforts to maintain the safety and health of individuals who donate Source Plasma.

What is most rewarding about working in this industry?

Seeing the connection between donor and patient and looking forward to the continued development of therapeutic possibilities of plasma proteins to treat rare diseases. ☺

IN MEMORIAM: JEAN-MARC SPIESER

IT WAS WITH GREAT SADNESS that the European Directorate for the Quality of Medicines and Healthcare announced that Jean-Marc Spieser, Head of the Department for Biological Standardisation, Official Medicines Control Laboratories Network and HealthCare passed away on April 1, surrounded by his family. Dr. Spieser was praised for being an enthusiastic Head of Department and an extraordinary colleague, peer and supporter. Dr. Spieser, was remembered by PPTA staff, who had the pleasure to interact with him over the course of the years, for always being open to dialogue with manufacturers to assure a continued supply of plasma protein therapies. Dr. Spieser attended several International Plasma Protein Congresses as both speaker and moderator, and always provided a valid contribution in the discussions between stakeholders. One of his biggest achievements was to establish the Official Control Authority Batch Release (OCABR) procedure for Human Biologicals: Vaccines, blood and plasma derivatives. This procedure assured that patients would access plasma protein therapies with the highest margin of safety in the European Member States and beyond. Dr. Spieser was also vital in the development of the European Pharmacopeia, a legally binding guidance for manufacturers of medicinal products. Dr. Spieser was also friendly and a pleasure to spend time with; he will be missed. 🌍



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GLOSSARY OF TERMS

A-PLUS	American Plasma Users Coalition
COTT	Committee of Ten Thousand
FDA	Food and Drug Administration
HFA	Hemophilia Federation of America
HPSC	Health Policy Steering Committee
HTA	Health Technology Assessment
NHF	National Hemophilia Federation
NORD	National Organization for Rare Disorders
OCABR	Official Control Authority Batch Release
SCID	Severe Combined Immunodeficiency
WHA	World Health Assembly

EVENTS

UPCOMING CONFERENCES & SYMPOSIUMS

2013



Denver, Colorado

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 12 – 13	9th Annual Symposium on Primary Immunodeficiency Diseases Newport Beach, CA, USA
October 13	Source Business Forum (PPTA Members Only) Denver, Colorado
October 26 – 27	NACLIS VI International Conference for primary immune deficiency disease Kuala Lumpur, Malaysia
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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Senior Vice President of Global Clinical Research & Development
USA, USA



MR JAN M. BIELE
President and CEO
Plasma Protein Therapeutics Association (PPTA)



DR RAVI S. KUMAR
Chief Executive Officer
Plasma Protein Therapeutics Association, Bangalore, India



DR THOMAS BURDOLPH
Professor
Soochow Medical University, Taipei, Taiwan

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