

THE SOURCE

MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

SPRING 2017

Global Patient Access

Serialization: A Global Perspective and Outlook for the Future

Examining the Nuffield Ladder

Fighting for Access: 25 Years and Counting!



Enhanced Donor Compensation. Simplified.



Customized donor compensation solutions tailored to your unique requirements

Streamlines operations and increases efficiencies

Superior uptime

See how 3PEA can customize a donor payment solution for you



1700 W Horizon Ridge Parkway, Henderson, NV 89012 | Toll-free: +1 866 549 9126 | Direct: +1 702 453 2221 | Plasma@3pea.com

PaySign® is a registered trademark of 3PEA Technologies, Inc. in the United States and other countries.

© 2017 3PEA International, Inc. All Rights Reserved.



GLOBAL BOARD OF DIRECTORS

Bio Products Laboratory | John Perkins

Biotest AG | Bernhard Ehmer

CSL Behring | Karen Etchberger

Grifols USA | David Bell, *Chair*

Kedrion SpA | Paolo Marcucci

Shire | Matthew Walker

General Counsel | Paul Rosenthal, Esq.

PPTA STAFF

President & CEO | Jan M. Bult

**Sr. Vice President,
North America & Global Health Policy** | Julie Birkofer

**Vice President,
Source & International Affairs** | Joshua Penrod

**Vice President,
Global Medical & Regulatory Policy** | Mary Gustafson

Executive Director, Europe | Bruno Santoni

**Vice President,
Legal Affairs & Global Operations** | John Delacourt

THE SOURCE STAFF

Editor | William Murray

Managing Editor | Rachel Liebe

Requests for permission to reprint should be directed to the editor at thesource@pptaglobal.org.

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

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

©Plasma Protein Therapeutics Association 2014

DESIGN AND PRODUCTION

B. Creative Group | www.agencybcg.com

THE SOURCE is published quarterly by **PPTA**.
147 Old Solomons Island Road, Suite 100
Annapolis, MD 21401

PHONE: +1.202.789.3100

EMAIL: thesource@pptaglobal.org



SPRING 2017 Contents

3 IN MY VIEW

4 Serialization: A Global Perspective and Outlook for the Future

6 Examining the Nuffield Ladder

10 Fighting for Access: 25 Years and Counting!

12 Transmissible Spongiform Encephalopathies:
Past, Present, and Future

16 Living An Active Life With Alpha-1 Antitrypsin Deficiency
Interview with Frank Willersinn, M.D.

18 Prof. Dr. Marcell U. Heim, Chairman of the ARGE Board Retires

22 Patient Access within a Changing Landscape

24 INSIDE PPTA

24 Meet the PPTA Staff: Larisa Cervenakova, M.D., Ph.D.

25 Commemorating 25 Years of Saving and Improving Lives

27 UPCOMING EVENTS

28 GLOSSARY



Shape the future

With the cobas[®] 6800/8800 Systems

The future of donor screening is in your hands
With transformative performance, automation and flexibility, the **cobas[®]** 6800/8800 Systems are redefining molecular testing for donor screening. Experience unprecedented throughput, faster time to results, mixed batching and a consolidated menu—all with just 3 human touchpoints per run. Look into the future of molecular testing at www.cobas68008800.com



Not available in all markets.

cobas[®]

Life needs answers

COBAS and LIFE NEEDS ANSWERS are trademarks of Roche.
© 2016 Roche Molecular Systems, Inc.
Roche Molecular Diagnostics
4300 Hacienda Drive
Pleasanton, CA 94588
USA
www.molecular.roche.com

www.cobas68008800.com





In My View

BY JAN M. BULT, PPTA PRESIDENT & CEO

The challenges in the newly begun 2017 are no different than they were in 2016, but there is uncertainty about what will happen this year. Patient access to care remains the single most important issue. A lot of work needs to be done by all involved parties to ensure that patients have access to their (many times life-saving) therapies in all sites of service. There should be no exception. This should apply to all countries in the world.

Am I dreaming or living in Wonderland? Sometimes I cannot believe all the terrible things that are happening in the world that affect so many innocent people. I remember the concerns of our Brussels staff (while at the International Plasma Protein Congress in Barcelona) about their families after the airport and subway attacks.

The patients we care about are innocent because none of them asked to be affected with a genetic disorder and always have to “fight” for their legitimate treatment. When I mean fight I am talking about problems with reimbursement, coverage, availability and so on.

As I am writing this, there are many questions about what will happen in the United States with the Affordable Care Act (ACA) also known as “Obamacare.” The Republican Party has said many times that they want to repeal the ACA as rapidly as possible. Then the President states that he wants to keep certain parts—like the provisions that allow children 26 years old and younger to be included on their parent’s policy—and not exclude pre-existing criteria for newly insured persons. What about the elimination of the lifetime caps that patients fought so hard for? Is this going to stay or not? It may well be that when this column is published, we will know more, but today there is uncertainty. And that is unfair.

Persons with genetic disorders who rely on life-saving plasma protein therapies already have enough on their mind. They do not need additional worries.

We are going to have many elections in various countries such as France, Germany, The Netherlands, and more. Every time when an election is near, issues come up about health care and price of medicines. And every time we have to explain why our therapies are so different:

- Small patient populations
- Rare diseases
- Different starting material with additional regulations
- Complex, lengthy, and expensive manufacturing processes

The good thing is that we have done it so many times that the arguments are in our veins. The arguments have to be conveyed to the various policymakers and payers in the many countries where these debates are held. Nowadays it seems that it is everywhere.

The good thing is that many times we are successful. Be assured that we will continue to help. You can count on us! ●

Jan M. Bult, PPTA President & CEO



Serialization:

A GLOBAL PERSPECTIVE AND OUTLOOK FOR THE FUTURE

BY RAJAGOPALAN SUBRAMANYAM, MBA, PMP
GLOBAL SERIALIZATION SOLUTION PROGRAM MANAGER
CSL BEHRING

By 2020, the pharmaceutical supply chain will undergo a major disruption in the way inventory is handled. The hitherto stable batch management concept will undergo a sea change and we will end up managing a lot (batch) size of one.

Serialization regulations for prescription drugs (outbound finished product) promulgated by major markets is the reason for this change. The primary rationale for serialization regulations is preventing counterfeit product from entering the legal supply chain.

While different markets have different timelines for these regulations, the U.S. and EU have major deadlines coming up in the next three years.

For most pharmaceutical manufacturers, these two markets represent a large percentage of their sales. Therefore we will see a huge spike in activity, discussion, and effort in this area starting in 2017. Pharmaceutical companies that were sitting on the sidelines will realize that the U.S. date for serialization is less than a year away.

WHY NOW?

We are in an era where the technology is sufficiently mature to enable the tracking of billions of units. Vials, folding boxes, lowest saleable units, etc. all refer to one unit of a prescription drug, and this is the level where serialization must occur.

Batch sizes can be anywhere in the thousands to tens of thousands of units. For serialization, supply chain entities must have systems that can track and authenticate a single unit of product (identified by a unique, randomized serial number). Furthermore, for serialization to be effective, every transaction between supply chain partners must also be authenticated. The current information systems and integration protocols enable the handling of such large volumes of complex transactions. At such a large scale—billions of products, tens of billions of transactions, and thousands of supply chain partner integrations—compliance would have been impossible, say a decade ago.

BENEFITS OF SERIALIZATION

While the primary benefit of serialization (as established in the rationale for the regulations) is preventing counterfeits from entering the legal supply chain, there are other significant benefits. One benefit would be that manufacturers can track specific issues that may be encountered during manufacturing to a single unit, which would be identifiable through serial

numbers in addition to batch numbers. This will increase the ability of manufacturers to take action when necessary and to maintain confidence in and availability of products, in addition to increasing efficiencies within the process itself. These benefits would accrue to manufacturers in a serialized environment.

Furthermore, the vast amount of product movement data will generate significant analytics. Such analytics will help companies lower their cost of transportation and identify and eliminate bottlenecks in the supply chain. When linked with connected health care and social media, patient preferences and engagement with product will provide additional insights. Serialization, therefore, should be seen as an investment and not simply a cost of compliance.

CHALLENGES

Compliance with serialization regulations for the global supply chain is not without its challenges, even with the technology now available.

Equipment/Technology Challenges

With serialization, packaging equipment now has to function as software. This is a big challenge. Printing the correct number and barcode in the right place at high speed is still difficult. Equipment's capability to read the number, verify it, and approve the print quality at speed, while aggregating the numbers into the next bigger handling unit (case, shipper case, or pallet) is still evolving. Globally, more than 10,000 packaging lines need to be upgraded to be serialization-capable.

Regulatory Challenges

Different markets have different reasons for serialization. As a result, there are varying requirements from market to market. Interpreting regulations and then working with equipment suppliers for effective compliance is costly and time consuming. The U.S. regulation of 2017 calls for serialization with no aggregation requirement. This is proving to be quite difficult for managing serial numbers downstream. Already the big wholesalers are calling for aggregation starting in 2017. Handling returns of serialized product is another area where multiple pilots are underway for understanding how this will work.

Personnel Challenges

The industry has very few personnel who are skilled in this area of technology. Furthermore, serialization brings a whole new scale of change management—especially at the operations level. Operations personnel have been dealing with batch-managed product for several decades. In a batch-managed scenario, each unit within a batch is fungible, which is not so in a serialized world. Each serialized unit is different from another, even within a batch. If a serialized unit is pulled for sampling, or is damaged, then a whole new process will have to be followed. Unlearning years of habitual action and learning a new process may cause disruption in operations.

PROGNOSIS

While technology will aid serialization, serialization will also provide opportunities for breakthrough technologies far beyond what the regulators envision. Serialization provides application and use cases for innovative and sophisticated technologies such as blockchain, messaging standards, internet of things, technology integration, printing technology and many more. While full track and trace under the U.S. Drug Supply Chain Security Act is expected to be operational by 2023, technology advancements and business economics will provide effective anti-counterfeiting solutions well before that date. ●



Author Rajagopalan Subramanyam, MBA, PMP, Global Serialization Solution Program Manager, CSL Behring, presents "Prescription Drug Traceability at CSL Behring" during a panel on "Traceability under the Drug Supply Chain Security Act" at the PPTA Plasma Protein Forum on June 14, 2016.

EXAMINING

THE

NUFFIELD LADDER

BY JOSHUA PENROD, PPTA VICE PRESIDENT, SOURCE & INTERNATIONAL AFFAIRS

The practice of recognizing and saluting donors for their commitment to plasma donation—compensating them—is growing in importance. This is partly due to a burgeoning global awareness of the importance of treating rare diseases for which plasma protein therapies (PPTs) are developed and administered. Coupled with this increased awareness is a rise in diagnoses, which leads to an increase in the need for treatment. Inevitably, this has led to a greater need for plasma.

With increasing regularity, the work of the Nuffield Council is cited as foundation for justifying self-sufficiency policies that rule out compensation. The Nuffield Council is a UK-based independent think tank, supported by large foundations, which issues opinions on areas of biomedical interest and research, ranging from elder care to emerging technologies. Their work includes analysis of the balance between economic forces and medicinal products of human origin. With Nuffield, the analyses typically arise in the form of questions related to organ donation, medical research, and related fields. Nuffield's work is usually regarded favorably in many different countries and contexts, and must be taken seriously by anyone involved in the many sectors that attract Nuffield's interest.

Through the publication of the CreativCeutical report in 2015, the Nuffield Council's 2012 publication, "Human Bodies: Donation for Medicine and Research" gained greater recognition. The CreativCeutical authors focused on a small portion of a very lengthy and complex Nuffield document which contained considerations from widely disparate and distributed industries and practices throughout all of the health care industry.

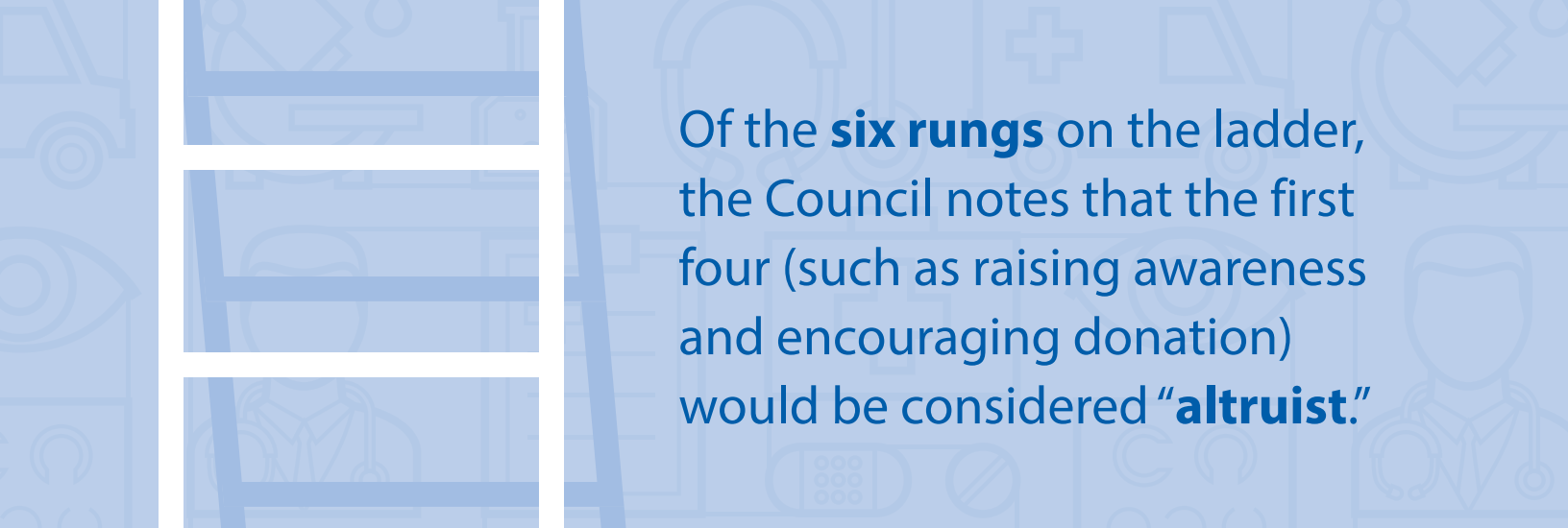
The 2012 Nuffield work is not the first generated by that organization focusing on ethical aspects of donation. Nuffield first grappled with these issues more than twenty years ago, in its 1995 report, "Human Tissue: Ethical and Legal Issues." The terms of reference for the 1995 work include recognition of the changing landscape of legal-ethical issues given advancements in basic medical research and technological change. The paper was written in the midst of crises involving variant Creutzfeldt-Jakob disease (vCJD) and property rights in genetic material. Blood and plasma were included as a part of Nuffield's analysis. Importantly, the Council in 2012 noted a critical distinction—and noted that the distinction was even wider than twenty years prior—between transfusable components and fractionated products. It specifically noted that blood donations for transfusion and blood products

derived from plasma have compelling differences in safety profile and usage, which have *nothing to do with compensation*. The report states that, for products such as PPTs, "quality and safety *are assured* not only by selection and screening of source materials, but also by the choice and control of manufacturing process." (12.27.2, emphasis added).

This is a highly important notation because, even in the middle of the 1990s, PPTs derived from a well-regulated process were seen as safe and efficacious. Since the 1990's, technology and safety have only improved, and the resulting products have become even safer. Even more to the point, this statement was made as a direct discussion of safety, during the peak of concern regarding vCJD in the United Kingdom. The most obvious point of importance is a glaring omission: that the practice of compensating the donor is not even mentioned.

The 1995 report also highlights the importance of transparency with regard to the procurement of donations; that is, a donor should have the knowledge of the disposition of his or her donation. This comports well with some of the more current thinking on the topic relating to informed consent. It recognizes the ethical considerations regarding how and why an individual decides to spend time and resources in supporting an activity, be it plasma donation, charitable contributions, or others. (James Stacey Taylor, *The Source*, spring 2014)

The second relevant Nuffield paper is the 2010 Consultation Paper, "Give and Take? Human bodies in Medicine and Research." This document helpfully notes that the term "donor" can be used, "whether or not [the donor] receive[s] *any form of compensation for doing so*." (31, emphasis added). The Nuffield Council also defines "volunteer" with the same proviso—compensation is irrelevant to the cast and definition of the term. Nuffield put the 2010 paper out in an effort to gain insight from interested parties. They cast their net quite wide, examining human clinical trials, cell and tissue donations, transplant policy, blood, donations to science and so on. The bulk of Nuffield's 2010 considerations focused on issues of autonomy,



Of the **six rungs** on the ladder, the Council notes that the first four (such as raising awareness and encouraging donation) would be considered “**altruist**.”

legal control, and property rights. Importantly, for the very specific context of the plasma collection industry, little was said about plasma per se and much of the same definitions from 1995 prevailed—acknowledgment of controversy with awareness of practical considerations given the different contexts around the uses of materials of human-origin.

In returning to present day considerations, the Council published its most recent and largest of the reviews, mentioned above, in 2012. This report was initially focused on the UK environment, and one must read, at the very beginning of the document, Nuffield’s own cautionary words in interpretation: “Time and again, the report comments on how concepts in this area of donation and volunteering are understood in different ways, at different times, in different circumstances, and by different people...*What works in one context need not work in others.*” (p. viii, emphasis added). The authors of the paper therefore recognized the temptation to interpolate one’s own political goals atop the analysis contained in this lengthy and complex document. They also recognized the importance of nuance of interpretation in the face of ethical and conceptual complexity.

Much of the today’s discussion involving donor compensation policies today, such as that in the CreativCeutical report, focuses on the Nuffield’s thoughts on compensating donors for plasma focused on the so-called “Intervention Ladder,” presenting a model which seeks to define guidance and encouragement for policymakers considering strictures involving donation of human-derived medicinal products. Of the six rungs on the ladder, the Council notes that the first four (such as raising awareness and encouraging donation) would be considered “altruist.”

The Council recommends greater scrutiny on non-altruist “rungs” on the ladder, but does not contemplate a blanket prohibition. Instead, with regard to UK policy, the Council explicitly states that: “Where payment is currently made to such [plasma] donors, the same concerns . . . should be considered... In the case of plasma, for example, given the importance of the need for plasma, the difficulties in sourcing it at present in the UK because of the theoretical risk posed by vCJD, and the

highly regulated nature of the donor recruitment and quality systems, it would seem likely that those tests [passing concerns regarding donor treatment] would be met, *and hence that reward for donors in these circumstances would constitute an ethically vindicated rung 6 of our Intervention Ladder.*” (p. 196, emphasis added). Again, while Nuffield focuses on the UK, their analysis also makes apparent their own expectation that other contexts could also agree with theirs.

Adapting models to fit different circumstances does present novel solutions to intractable problems; the downside is that forcing a fit from one model may cause greater problems. It seems clear that many of those who would proscribe donor compensation would forget Nuffield’s own nuanced view. It is indeed interesting that Nuffield expressly used the compensated donation of plasma as an example of an ethically suitable practice. While Nuffield focused on the UK environment (and noted the UK’s dependence on United States plasma for further manufacture), the distinct nature of plasma was called out for mention; specifically, the safety processes used in manufacture, and the special situation of patients dependent on PPTs. Using the Nuffield work to examine these issues is of great utility. It is also crucial to understand the spirit of the Nuffield paper; this helps us recognize what the Council itself has said...especially when it applies to our industry and PPT patients.

In the final count, the Nuffield Council’s work points a clear path toward the acceptable ethical construct that our industry uses. The Council’s report contemplates the finished products, the needs of the patients, and the importance of donor safety and quality systems, among many other factors. While the document itself acknowledges the complexity of the area of medical products of human origin and much of its language is rightly cautious, the fact that our industry and its practices were singled out for identification as an ethical path is something that reminds the industry of the rightness of our mission. It will also give greater hope to patients around the world that more regulatory and health care systems will see their way to Nuffield’s conclusions and create structures in which donors are recognized and patients are fully treated. ●



THE WORLD'S CITI. IT'S WHEREVER YOU ARE.

Every day, in cities around the world, people are doing amazing things. They're creating, innovating, adapting, building, imagining. What about a bank? Shouldn't we be equally ingenious? Strive to match our clients' vision, passion, innovation? At Citi, we believe that banking must solve problems, grow companies, build communities, change lives.

Citi® Prepaid Cards has used our passion and innovation to provide the plasma industry with a secure, easy payment answer for donor compensation. Citi Prepaid remains committed to our clients, just as our clients are committed to helping people live healthier, happier lives through research, therapy and treatment.

To find out more about Citi Prepaid Cards, please visit citiprepaid.com



Fighting for Access: 25 YEARS AND COUNTING!

BY JAN M. BULT, PPTA PRESIDENT & CEO
KARL PETROVSKY, PPTA SENIOR MANAGER, HEALTH POLICY

In 1992, three visionary leaders in the plasma protein industry—Ralph Galustian (Bayer), Otto Schwarz (Immuno), and Guelfo Marcucci (Gruppo Marcucci)—decided to establish a specific Association focusing on plasma protein therapies (PPTs).

Robert W. Reilly became the first Executive Director of the newly formed International Plasma Products Industry Association (IPPIA). Because of legislative initiatives in Europe that were very important to our industry, it was decided to start Association activities in Europe.

In the fall of 1993, I received a phone call from Karl Petrovsky—who was working in the legal office of Immuno in Munich, Germany at the time—to attend a meeting in Zurich to discuss the creation of an Association specific to the PPT industry. Unfortunately, I could not attend because of important company issues. In December, I attended a meeting with EFPIA (European Federation of Pharmaceutical Industry Associations) who, until that moment, had a working group with limited activities in the PPT sector. At that December meeting, it was decided to establish a new Association in Europe. I remember the special atmosphere at that meeting. The head of EFPIA was not pleased.

In January 2004, the first meeting was held in London and one of the decisions that needed to be made was naming the newly established Association in Europe. Determining a name took a long time because the chosen name had to be different from EFPIA. That was the beginning of the European Association of the Plasma Products Industry (EAPPI). The first chairman was Knut Hansen (Immuno), who was an excellent industry leader. Other Board members were Jean-Marie

Vlassembrouck (Baxter), Roland Hagberg (Kabi), Bill Hartin (Alpha Therapeutics), Jack Ryan (Bayer), Roloff Johannsen (Behringwerke), Norbert Chariatte (Berna), Giovanni Rinaldi (Gruppo Marcucci), Manel Canivell (Gruppo Grifols), and myself, Jan M. Bult (Biotest).

It was an interesting experience, especially since Karl Petrovsky and I now work full time for the Association. Special credit needs to be given to Robert W. Reilly who moved to Europe with his wife, Ann, to do all the groundwork necessary to build the Association. It was not an easy task but they worked tirelessly to make it happen.

There was a special role for Knut Hansen, the first chairman of EAPPI. Knut Hansen was, at that time, the visionary head of Immuno's Legal and EU/International Department. Hansen was the rare combination of an outstanding, pragmatic attorney with a deep understanding of the plasma products industry sector, and a brilliant diplomat and negotiator. At that time, around 1992, our industry was dealing with the need to advocate for some changes to the very first Blood Directive 89/381/EC of 1989, which was developed as a consequence of the HIV tragedy in the '80s.

With the growing complexity of the issues, and the need for a very specific industry advocacy, Knut Hansen was among the key leaders who convinced his industry peers that this sector needed its own association. The first steps to create an industry association were done with Robert W. Reilly and the American Blood Resources Association's International Committee, of which Knut Hansen was a member. This is how Hansen decided in the fall of 1993 to ask Karl to call me. The rest is history. ●



Your Testing Service Solution

Services

- Infectious disease testing
- Nucleic acid testing (NAT)
- Immunohematology reference lab (IRL)
- Microbiology/sterility testing
- Specialty testing

About QualTex

- Multiple laboratory sites
- 24/7/365 testing schedule
- FDA registered
- EU GMP certificate of compliance
- German Health Ministry certification
- ISO 9001:2008 certified
- Active research and development



Locations

HEADQUARTERS – SAN ANTONIO
6211 IH 10 West
San Antonio, TX 78201

ATLANTA
4258 Communications Drive
Norcross, GA 30093



Transmissible Spongiform Encephalopathies: PAST, PRESENT, AND FUTURE

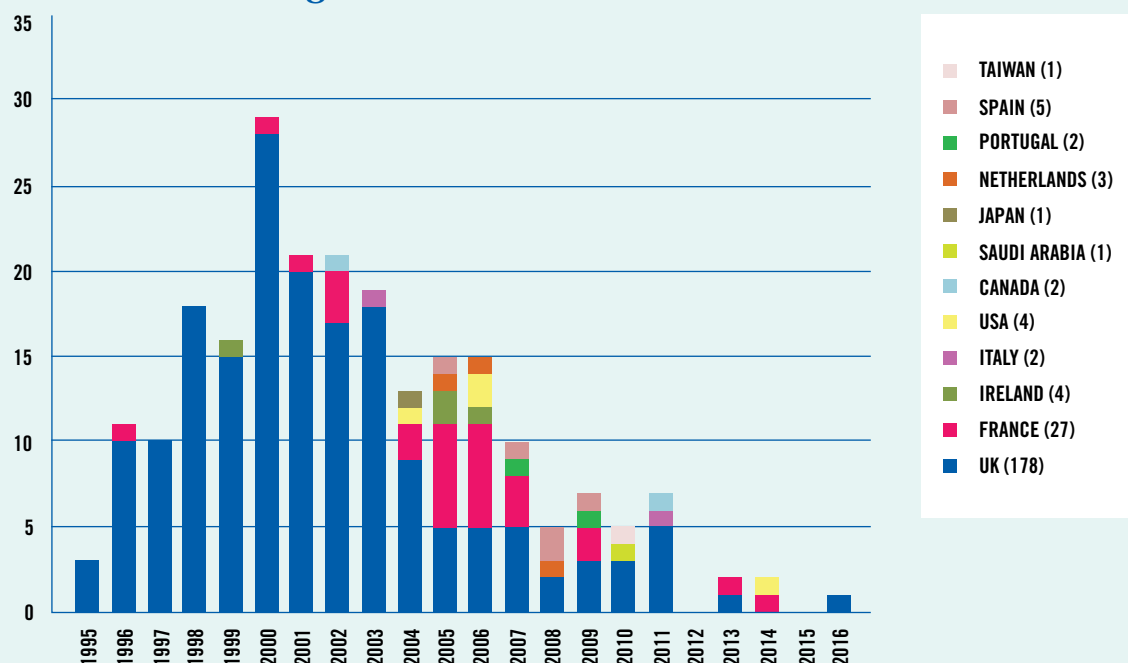
BY LARISA CERVENAKOVA, M.D., PH.D, PPTA MEDICAL DIRECTOR

The first issue of The Source magazine, released in September 2001, was dedicated almost entirely to transmissible spongiform encephalopathies (TSEs)/prion diseases, and their possible iatrogenic human-to-human transmission through blood and blood-derived therapeutic treatments. Special attention was given to variant Creutzfeldt-Jakob diseases (vCJD), the rare form of human TSE mainly occurring in the UK and linked to dietary exposure to products contaminated with prion isolate of bovine spongiform encephalopathy (BSE), known to the public as “mad cow” disease.¹ In the same year, the National CJD Research and Surveillance Unit in the UK reported, for the first time, a lower number of vCJD cases than in previous years since the first patients were diagnosed in 1995 (**Figure 1**).² At that time, no one could predict whether the declining trend in number of vCJD cases signaled a turning point in the epidemic, considering the enormous extent of the BSE epidemic and the probability of significant human exposure to the agent. An additional uncertainty surrounded the incubation period of TSE, which could vary from 4-20 years and in some cases can even exceed 50 years, as had been reported for patients afflicted with kuru—an almost-extinct condition in Fore people of Papua New Guinea

who were infected through practicing cannibalistic rituals.^{3,4} Long incubation periods had also been described in iatrogenic CJD patients to whom the disease was accidentally transmitted through treatment with prion contaminated pituitary-derived human growth hormone, or gonadotropin or dura mater transplant.⁵ Especially worrisome was the fact that vCJD affected younger individuals (the youngest patient was 12 years old) with a mean age at onset of 28 years. In contrast, sporadic CJD (sCJD) patients, afflicted with the most common form of TSE, develop the disease at significantly older age—in their ‘60s.

We needed to know what the future would hold and how we should prepare for the situation described by one epidemiological predictive model that provided the possibility of an epidemic affecting between one thousand and more than twenty thousand individuals in the UK alone.⁶ It was a time of uncertainty, in which the question of whether there were prions present in the blood of vCJD-afflicted individuals and in blood of healthy people who might be silently incubating the disease had yet not been answered. Since then, PPTA has repeatedly returned to the issue of prions in number of subsequent publications addressing human and animal TSEs, including BSE, vCJD, and sCJD. The most recent articles mentioning

Figure 1: vCJD Cases Worldwide



these conditions were published in the 2012 summer issue of *The Source* and included interviews with Jay Epstein, M.D. of the Center for Biologics Evaluation and Research, FDA and Glenda Silvester, Ph.D. of the Human Medicines Development and Evaluation Unit, EMA.⁷ By that time, complex issues had been addressed at regulatory and research levels, and many global decisions were proven to be justifiable in preventing a wide spread of vCJD epidemic. Coincidentally, since 2012 only two new cases of vCJD were diagnosed in the UK (as of Dec. 5, 2016), and two new vCJD patients were reported in France—the country with the next highest number of cases,⁸ and one new case was identified in the U.S. with strong evidence of possible exposure to BSE outside the country (**Figure 1**).⁹

From the time when the first four individuals died from vCJD in the UK in 1995, in total, 178 people in the UK, 27 people in France, and more than two dozen people around the world—mostly in European countries—were lost due to the same condition (**Figure 1**). Regrettably, we witnessed the probable transfusion transmissions of vCJD to four individuals in the UK who received non-leukoreduced red blood cell concentrates from three donors in the pre-symptomatic phase of the disease.¹⁰ The universal leukoreduction of blood and blood components was implemented in the UK, France, Canada, and many other European countries by the end of 1999 and in Germany in October 2001 to reduce the possibility of vCJD transmission through the white blood cells, which were identified at the time as the probable primary source of infection in blood.¹¹ In the United States, the universal leukoreduction has been recommended to the blood establishments by the FDA Blood

Products Advisory Committee and the Advisory Committee on Blood Safety and Availability in 1998 and 2001, respectively. The issue of whether this preventive measure alone was sufficient to eliminate the risks of vCJD transmission through blood transfusion is still open for discussion but, as of today, no vCJD cases related to transfusion of leukoreduced therapeutics have been identified.

In one person with hemophilia who died at the age of 73, the main diagnostic marker of TSEs, the misfolded disease-associated prion protein (PrPTSE), was found in one sample of many prepared from the spleen during retrospective pathological study.¹² This individual died from a non-neurological disease and there was no evidence of PrPTSE or pathological changes consistent with TSE in the brain—the main organ suffering from the consequences of the infection. The patient was heterozygous at a codon 129 methionine/valine (M/V) of the prion protein gene (PRNP), whereas almost all other vCJD patients had been methionine/methionine (M/M) homozygous at codon 129. The exceptions were reported in a recent vCJD patient who died in 2016 and in one of the four recipients of non-leukoreduced red blood cells, mentioned above, both had a PRNP codon 129 M/V profile. This latter person never developed clinical signs of neurological disease but the presence of PrPTSE was found in the patient's spleen and one lymph node.¹³ The codon 129 PRNP genotype has been shown to influence the incubation period and duration of the disease, as well as clinical and neuropathological features of TSEs and even biochemical prion protein characteristics. The PRNP codon 129 M/V vCJD cases deserve special attention

because of the retrospective study of appendix samples collected between 2000-2012 that was undertaken in the UK.¹⁴ The presence of PrPTSE was found in 16 out of 32,441 examined samples, yielding the estimate of approximately one in two thousand individuals potentially silently incubating vCJD. All three PRNP codon 129 genotypes (M/M, M/V, and V/V [valine/valine]) were represented within this group of sixteen samples, an indication that a person with any PRNP codon 129 genetic makeup can be susceptible to vCJD infection. The question now is whether the comparatively long incubation periods associated with a codon 129 M/V PRNP genotype, coupled with the estimated frequencies of silent vCJD infection, could contribute to a possible future reoccurrence of vCJD cases.

Regardless of the outcome, we can state that multiple preventive mandatory and regulatory measures put in place by government agencies have been extremely important in safeguarding plasma-derived and recombinant therapeutics from contamination with vCJD and other human TSE agents. In addition, the industry has been highly responsive to the threats of the vCJD epidemic and has carried out numerous studies to assess the clearance of prions during the manufacturing processes, as well as introduced industrywide additional steps for prion removal.¹⁵

Sporadic CJD, is believed to occur spontaneously without any known link to relevant environmental or iatrogenic exposure. More than 20 years of epidemiological follow-up observations conducted by the American Red Cross provide no evidence of sCJD transmission through blood transfusion.¹⁶ The Transfusion Medicine Epidemiology Review study that was initiated in the UK in 1997 and still continues, also reports no evidence of transfusion transmission of sCJD.¹⁷

In conclusion, the years of commitment and investment in research supported by an extensive record of publications and presentations at various scientific meetings documented the seriousness with which the industry addresses issues of patient safety. We hope that there will be no unexpected surprises stemming from transmission of zoonotic TSEs to humans, one of which—chronic wasting disease (CWD) of cervids—has not only spread through the North American continent affecting deer, elk, and moose, but also reached South Korea through commercially exported farmed animals. In the past year, CWD was first found in wild reindeer during the tagging and registration,¹⁸ and later in two additional reindeer and two moose by targeted surveillance in Norway (as of December 2016).¹⁹ Presently, we have no epidemiological evidence that CWD has ever infected humans, but as always, only continuing vigilance will ensure that no preventable human tragedies related to TSEs will occur in near future.

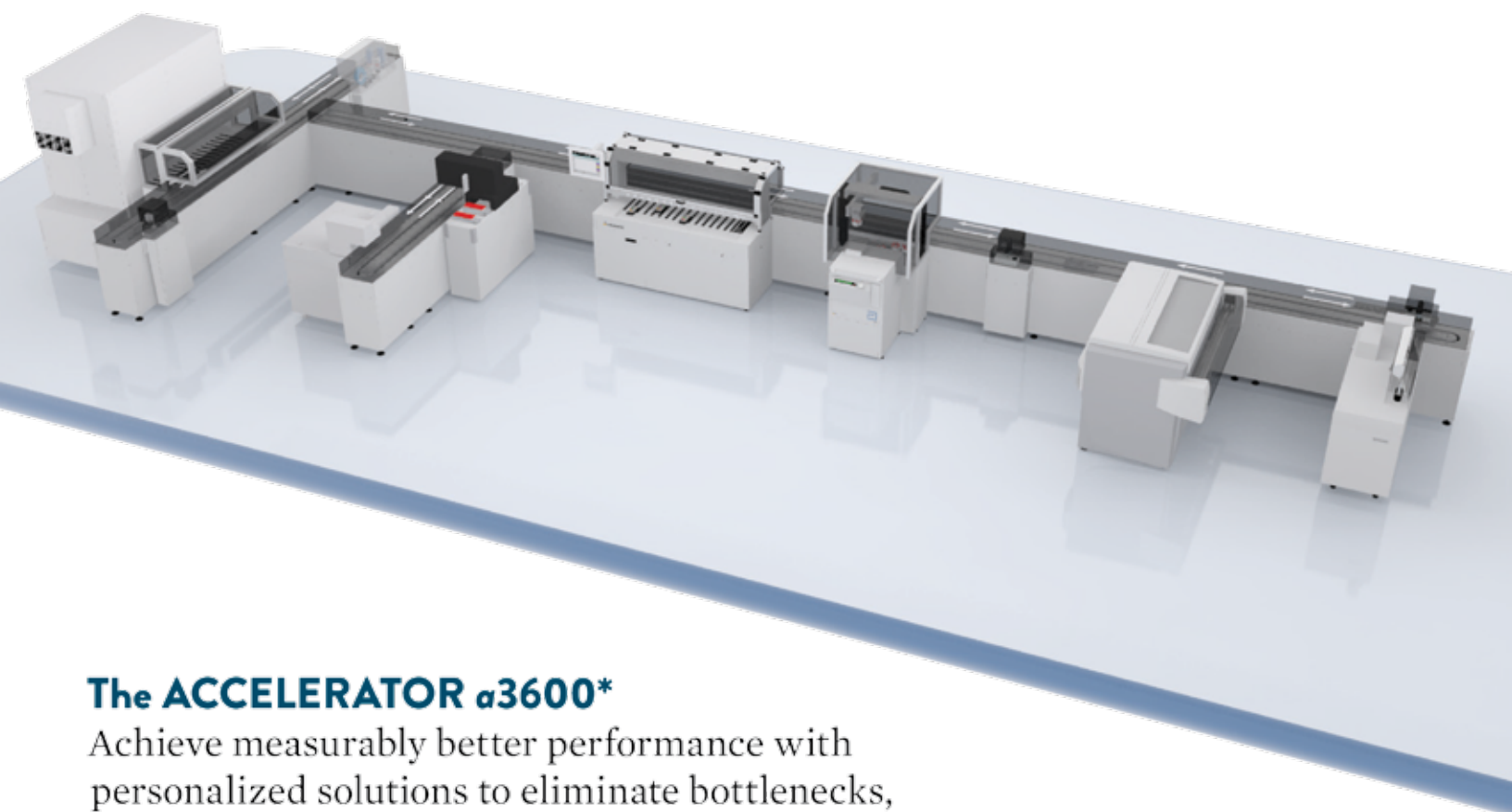
Acknowledgment: I am grateful to Jan M. Bult and Dr. Paul Brown for helpful comments on the manuscript. •

References:

1. The Source, September, 2001, pp.3-16.
2. Will RG, Ironside JW, Zeidler M, Cousens SN, Estibeiro K, Alperovitch A, Poser S, Pocchiari M, Hofman A, Smith PG. A new variant of Creutzfeldt-Jakob disease in the UK. *Lancet*. 1996; 347:921-5.
3. Cervenáková L, Goldfarb LG, Garruto R, Lee HS, Gajdusek DC, Brown P. Phenotype-genotype studies in kuru: implications for new variant Creutzfeldt-Jakob disease. *Proc Natl Acad Sci U S A*. 1998;95:13239-41.
4. Collinge J, Whitfield J, McKintosh E, Beck J, Mead S, Thomas DJ, Alpers MP. Kuru in the 21st century - an acquired human prion disease with very long incubation periods. *Lancet*. 2006;367:2068-74.
5. Brown P, Brandel JP, Sato T, Nakamura Y, MacKenzie J, Will RG, Ladogana A, Pocchiari M, Leschek EW, Schonberger LB. Iatrogenic Creutzfeldt-Jakob disease, final assessment. *Emerg Infect Dis*. 2012;18:901-7. doi: 10.3201/eid1806.120116.
6. Ghani AC, Donnelly CA, Ferguson NM, Anderson RM. Assessment of the prevalence of vCJD through testing tonsils and appendices for abnormal prion protein. *Proc Biol Sci*. 2000;267:23-9.
7. The Source, Summer 2012, pp. 20-23.
8. <http://www.eurocjd.ed.ac.uk/surveillance/data/1.html>
9. Maheshwari A, Fischer M, Gambetti P, Parker A, Ram A, Soto C, Concha-Marambio L, Cohen Y, Belay ED, Maddox RA, Mead S, Goodman C, Kass JS, Schonberger LB, Hussein HM. Recent US Case of Variant Creutzfeldt-Jakob Disease-Global Implications. *Emerg Infect Dis*. 2015;21:750-9. doi: 10.3201/eid2105.142017.
10. Checchi M, Hewitt PE, Bennett P, Ward HJ, Will RG, Mackenzie JM, Sinka K. Ten-year follow-up of two cohorts with an increased risk of variant CJD: donors to individuals who later developed variant CJD and other recipients of these at-risk donors. *Vox Sang*. 2016;111:325-332. doi: 10.1111/vox.12426.
11. Bassuni WY, Blajchman MA, Al-Moshary MA. Why implement universal leukoreduction? *Hematol Oncol Stem Cell Ther*. 2008;1:106-23.
12. Peden A, McCardle L, Head MW, Love S, Ward HJ, Cousens SN, Keeling DM, Millar CM, Hill FG, Ironside JW. Variant CJD infection in the spleen of a neurologically asymptomatic UK adult patient with haemophilia. *Haemophilia*. 2010;16: 296-304. doi: 10.1111/j.1365-2516.2009.02181.x. Epub 2010 Jan 12.
13. Peden AH, Head MW, Ritchie DL, Bell JE, Ironside JW. Preclinical vCJD after blood transfusion in a PRNP codon 129 heterozygous patient. *Lancet*. 2004;364: 527-9.
14. Gill ON, Spencer Y, Richard-Loendt A, Kelly C, Dabaghian R, Boyes L, Linehan J, Simmons M, Webb P, Bellerby P, Andrews N, Hilton DA, Ironside JW, Beck J, Poulter M, Mead S, Brandner S. Prevalent abnormal prion protein in human appendixes after bovine spongiform encephalopathy epizootic: large scale survey. *BMJ*. 2013;347:f5675. doi: 10.1136/bmj.f5675.
15. Cai K, Gröner A, Dichtelmüller HO, Fabbri F, Flechsig E, Gajardo R, von Hoegen I, Jorquera JI, Kempf C, Kreil TR, Lee DC, Moscardini M, Pölsler G, Roth NJ. Prion removal capacity of plasma protein manufacturing processes: a data collection from PPTA member companies. *Transfusion*. 2013;53:1894-905. doi: 10.1111/trf.12050.
16. Dorsey K, Zou S, Schonberger KB, Sullivan M, Kessler D, Notari E 4th, Fang CT, Dodd RY. Lack of evidence of transfusion transmission of Creutzfeldt-Jakob disease in a US surveillance study. *Transfusion* 2009;49:977-84. Epub 2009 Jan 5.
17. Urwin PJM, Mackenzie JM, Llewelyn CA, Will RG, Hewitt PE. Creutzfeldt-Jakob disease and blood transfusion: updated results of the UK Transfusion Medicine Epidemiology Review. *Vox Sanquins* 2016;110:310-316.
18. Benestad SL, Mitchell G, Simmons M, Ytrehus B, Vikøren T. First case of chronic wasting disease in Europe in a Norwegian free-ranging reindeer. *Vet Res*. 2016;47:88. Doi: 10.1186/s13567-016-0375-4.
19. <https://www.bds.org.uk/index.php/documents/125-cwd-update-from-norway-dec-16/file>



A HEALTHIER COMMUNITY THRIVES WITH A HEALTHIER BLOOD AND PLASMA SUPPLY.



The **ACCELERATOR a3600***

Achieve measurably better performance with personalized solutions to eliminate bottlenecks, improve productivity and reduce labor costs. Contact your Abbott representative to learn more about innovative solutions that can transform the health of your blood and plasma supply.

ABBOTTDIAGNOSTICS.COM

CHOOSE TRANSFORMATION™

*The ACCELERATOR a3600 is an automated system that can be used in the lab for pre- and post-analytical sample processing.



FRANK WILLERSINN, M.D.

Founder and President of Alpha-1 Plus Belgium
Member of the Board of Directors of the Alpha-1 Foundation, Miami
Member of the Alpha-1 Global Steering Committee

Living An Active Life With Alpha-1 Antitrypsin Deficiency

INTERVIEW WITH FRANK WILLERSINN, M.D.

BY DR. IRINA ODNOLETKOVA, PPTA DIRECTOR, HEALTH ECONOMICS & OUTCOMES

Like a lot of others, I was diagnosed by chance in 1998, when my alpha-1 globulin levels were tested due to a mistake in the laboratory request form. Without special symptoms, I did not go to the doctor immediately. All I knew was that AATD had a genetic origin.

Q What is Alpha-1 antitrypsin deficiency?

Alpha-1 antitrypsin deficiency (AATD) is a rare genetic disorder that causes defective production of alpha-1 antitrypsin. Depending on the form and degree of deficiency, it may lead to lung disease, liver disease, or skin disease. Among the rare diseases, AATD is one of the most common conditions. But not everyone with AATD develops a clinically significant disease. Probably about a tenth of the alphas require plasma protein augmentation therapy. The different genotypes will lead to different serum levels of alpha-1 antitrypsin (A1AT). Most patients with clinical disease are homozygous SS or ZZ. They have the lowest serum levels of A1AT.

Q How were you diagnosed with AATD?

I was a heavy smoker for more than 15 years and I had chronic bronchitis. Like a lot of others, I was diagnosed by chance in 1998, when my alpha-1 globulin levels were tested due to a mistake in the laboratory request form. Without special symptoms, I did not go to the doctor immediately. All I knew was that AATD had a genetic origin. But at that point, I was much more preoccupied with adopting a new way of life after I lost my arm two years earlier, a consequence of a severe and rare form of streptococcal infection. Statistically there was no chance of survival. I was brought in in a coma, and my heart even stopped beating once. By a miracle, I survived but lost my infected arm. Since then I experience every day as a gift.

Q What moved you to start patient advocacy work?

Ten years after the diagnosis, when I started with alpha-1 antitrypsin augmentation therapy, I wanted to learn more about AATD and find other people with this rare disease. Together, we contacted Members of the European Parliament (MEPs) and explained to them that the problem of AATD is not sufficiently addressed in Belgium. After this meeting, the MEPs reached out to the Belgian Minister of Health, and we realized that better care may be achieved through a dialogue with health care policymakers. In 2013, I founded the Alpha-1 patient association in Belgium, which was made up of eight members with AATD and gave us an identity and a voice with policymakers.

Q How do you see the mission of your patient association?

The mission is to improve the health and quality of life of other alphas. The founder of AlphaNet and COPD [chronic obstructive pulmonary disease] Foundation in the U.S., John W. Walsh, was a great source of inspiration. Currently, I am involved in patient advocacy at different levels, like Alpha-1 Global, which is run by eight volunteers. We support national associations through developing information and advocacy tools to improve early diagnosis, education of specialists, establishment of treatment centers, etc. We find patients and patient associations throughout Europe and help to develop new associations in countries where they don't currently exist. Along with a group of top experts in Europe, we are also actively involved in drafting EU recommendations for AATD. The upcoming 6th Alpha-1 Global Patient Congress will be held in Lisbon, Portugal on April 5-8, 2017 (see <http://www.alpha-1global.org/> for more information).

Q What have you been able to achieve?

In more than 30 countries in Europe, there is no reimbursement for AATD treatment. Through educating physicians in countries such as Romania and Poland, and supporting them with diagnostic tools, we identify local alphas and bring them together. They can find national patient associations and we help them in their advocacy work. Bringing them in contact with other well-established national structures—such as rare disease foundations supporting immunodeficiency and hemophilia—is important to build their health policy experience. Similarly, we set up Alpha-1 Switzerland, which started with twelve enthusiastic alpha-1 volunteers and now, six months later, they have 35 members.

Q What are the main challenges in your advocacy work?

The main challenge is that every country requires a different approach. To start, all work needs to be done in the national language and there are 24 official languages in Europe. Furthermore, each country has its own health care system and policy for rare diseases, which creates inequalities. For example, in the Netherlands, the replacement therapy is reimbursed to people with severe AATD (phenotype OO), in Belgium the reimbursement stopped several years ago, leaving those alphas diagnosed after June 2010 without treatment. We have taken several actions against this discrimination and will continue this work. Also, in the UK, no reimbursement was granted by the National Institute for Health and Care Excellence. The budget impact of AATD in the UK would be high, indeed, with about 1,300 patients waiting for treatment and an annual treatment cost per patient of approximately 80,000 € (about \$85,400). However, patients and clinicians don't give up their struggle and hope.

Q What is your perspective on the plasma sector?

The plasma protein products industry has a great potential to open new markets, both geographically and in terms of new indications, because AATD also has an influence on other metabolic balances.

I believe that more targeted (personalized) treatments will be possible when the underlying causes of diseases are better understood. An important challenge for the future is to ensure more plasma collection in Europe. Currently, most of the plasma is coming from the U.S., which worries me as a patient with European residence. In Europe, a solution must be found to motivate young people to donate plasma. It will require a re-thinking of the communication campaigns to make clear to people how important their contribution will be for saving other people's lives. ●



Prof. Dr. Marcell U. Heim, Chairman of the ARGE Board Retires

BY ALEXA WETZEL, PPTA ASSISTANT DIRECTOR, SOURCE EUROPE

Prof. Dr. Marcell U. Heim, Head of the Institute of Transfusion Medicine and Immunohematology and Professor at the Medical Faculty of the Otto-von-Guericke-University in Magdeburg (Saxony-Anhalt, Germany), retired in late 2016. The institute, which provides the University Hospital with sufficient blood and plasma, was directed by Heim for the past 22 years.



Prof. Heim was born in Frankfurt (Hesse, Germany), went to secondary school in Austria, and finished his university studies and Ph.D. in Munich. His career started in Munich and in 1993—a couple of years after the German reunification—he was offered the opportunity to move to Magdeburg, in the Eastern part of Germany, and to manage the Institute of Transfusion Medicine and Immunohematology at the University Hospital. His role was to ensure a sufficient supply of blood and blood components, including plasma and plasma protein therapies.

However, Heim has become famous in Germany for a different kind of donation. He is the initiator of a regional bone marrow donation registry. In 1995, when the parents of a local leukemia patient were looking for a suitable donor, he succeeded in motivating 21,000 people from the region of Saxony-Anhalt to undergo a blood test and created the first registry. Today this registry contains more than 33,000 names and the Association for Bone Marrow Donation in Saxony-Anhalt has succeeded in helping patients from around the world.

His expertise, engagement, and friendly nature also contributed in increasing the number of committed blood and plasma donors for the Institute.

Heim also has many other passions, one of which is cooking. He always jokes that if he hadn't become a physician he would have been a well-known chef. He owns more than 1,000 cookbooks and has even written a couple.

His other trademark is his Citroën 2CV ("Duck"). The Duck was the first car he owned and since then has never wanted to have any other car. He still proudly drives his Duck even if it is very cold in the winter.

He is and has always been very engaged with different associations. For the past ten years, he was also the chairman of the board of the German Arbeitsgemeinschaft Plasmapherese (ARGE) e.V. (German Working Group on Plasmaphereses).

The ARGE was founded in 1997. The unique aspect of the ARGE is that it brings together the four different parties that are involved in plasma collection in Germany: the German Red Cross organizations; state owned and community hospitals; independent collectors; and the industry.

The mission of the ARGE is to foster sufficient plasma collection for the manufacturing of high-quality plasma protein therapies but also to ensure donor and product safety. The ARGE has, on many occasions, provided scientific support for plasmapheresis. Its contributions to the Guidelines of

the German Physician Chamber and the support of the SIPLA (Safety of long-term intensive plasmapheresis in donors) studies are some examples.

In 2015, more than 2.2 million liters of plasma were collected by ARGE members. This included source plasma, recovered plasma, and plasma for therapeutic purposes.

One strength of the ARGE has always been the organization of its annual Congress in late November. This Congress is one of the only continuing medical education opportunities in the German-speaking area that is geared exclusively to staff from plasma collection organizations.

Last year's Congress, which took place in Magdeburg, Germany was Professor Heim's farewell.

Now that he is retired, he has plenty of new projects. One is to write a book on the year after the German reunification. His purpose is to illustrate all the exciting things that happened 25 years ago, in the Eastern part of Germany. His fascination with East Germany is related to his grandparents, as they used to live in Brandenburg where he visited them regularly until 1965, when the Berlin Wall was erected.

PPTA and ARGE have worked together on many projects throughout the past 15 years.

PPTA would like to take this opportunity to thank Prof. Heim for the outstanding collaboration and contributions to the industry. ●



An example of a Citroën 2CV ("Duck").

His other trademark is his Citroën 2CV ("Duck"). The Duck was the first car he owned and since then has never wanted to have any other car.

Collect with confidence and optimize operational efficiencies

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



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

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

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

© 2016 Haemonetics Corporation. Haemonetics, Haemonetics The Blood Management Company, and DMS are trademarks or registered trademarks of Haemonetics Corporation in the USA, other countries, or both. 02.2016 USA. COL-AD-000185(AA)

Next Generation DMS™ — our new plasma management software solution



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

→ **To learn more about our full range of products, programs, and services,** contact your Haemonetics Account Manager or visit www.haemonetics.com





AFFORDABLE CARE ACT (ACA)

Patient Access within a Changing Landscape

BY TOM LILBURN, PPTA SENIOR DIRECTOR, GOVERNMENT RELATIONS
BILL SPEIR, PPTA SENIOR DIRECTOR, STATE AFFAIRS

Access to plasma protein therapies for patients in the U.S. has been relatively certain and predictable throughout the past decade. Efforts by PPTA, member companies, and stakeholders have resulted in continued reimbursement by both federal and state governments for a wide range of economic situations and private insurers have also provided coverage. The life-saving nature and non-interchangeability of these biologic products has set them apart from many of the more controversial drug therapies that have raised concerns within the health care system, often because of triple digit price increases.

However, the environment that we find ourselves in now is different and challenging. The U.S. is passing from a two-term administration that embraced federal government entitlement to provide health care to as many patients as possible to a yet-to-be-defined market-driven system. Leaders in Congress and the White House have promised to abandon the Affordable Care Act (ACA) and replace it with their own plan, but cannot agree on what that plan should contain. Most Americans like some of the ACA, such as prohibiting exclusion because of pre-existing

conditions, extending coverage to children until age 26 on their parent's plans, and eliminating lifetime caps on costs.

However, the exchanges brought costs individuals could not afford, such as the rising deductibles costs and huge monthly premium increases. Very few who bought health care insurance through the state exchanges understood that there could be no caps or protections against rising costs, although they were mandated by law to purchase it.

The largest health care providers, like United Healthcare and Aetna, ended participation in the ACA due to unsustainable losses. This often left patients with no choice in the exchanges or no bargaining power along with forced changes in doctors and hospitals. Access to therapy then became a challenge. Changes in government programs also added to the uncertainty. Recently proposed rules in Medicare—which intended to cut payments for drugs, penalize physicians that prescribe biologics, and restrict access to just a few products—were scrapped soon after the November election when it could not be approved before the new administration takes office.

State Medicaid programs will change under the Trump Administration, but just how they will change is the question. The ACA created a new eligibility group of adults without dependents. More than 14.6 million Americans were able to enroll in Medicaid as members of this “New Adults Group” when 31 states and the District of Columbia expanded Medicaid eligibility. If the ACA is repealed, will they lose their coverage? Will the Republican governors who supported expansion in their states, including Vice-President Mike Pence, allow these individuals to lose their Medicaid eligibility?

Indiana’s Medicaid expansion program could serve as an example of changes to come to the entire program. Seema Verma was vital in creating the program and she has been nominated by President Trump to lead the Centers for Medicare and Medicaid Services. The Indiana plan requires the enrollees in Indiana’s Medicaid expansion program to pay premiums and co-pays for services. She has set up the program to make it more like commercial insurance.

One commercial insurance strategy that should be watched for is a restricted formulary. A restricted formulary provides insurance beneficiaries with access to only a few therapies in a therapeutic class. Currently this is not allowed in Medicaid and as a result plasma protein deficient individuals have access to all plasma protein therapies in Medicaid. This is because of a federal rule that allows patients access to all pharmaceuticals if the manufacturers pay a federal rebate. This access could change if the Trump administration seeks to repeal this provision of federal law and attempts to negotiate lower pharmacy costs through a restricted formulary where Medicaid recipients have access to only one or two therapies in a therapeutic class. In fact, the federal government could do this for all their health benefit programs including Medicare, Medicaid, and the Children’s Health Insurance Program. An administration that promises to provide better health care at less cost through a competitive market system will be a challenge. The health care system in the U.S. has experienced a slowing of growth since a 2002 high of more than eight percent to a more moderate five percent per annum rate in 2015. But even then almost 10 percent of Americans reported that they delayed or did not get care because of cost and this is in a system where over 94 percent of patients are covered by some form of health care insurance or government subsidy.

For a majority of patients, access continues to be tied to the availability of reimbursement, whether through employer sponsored plans, Medicare, or Medicaid. The possibility that the new Congress and Administration will scrap the entire exchange-based system in favor of market



The possibility that the new Congress and Administration will scrap the entire exchange-based system in favor of market driven approaches like health savings accounts, accountable care organizations, and even state block grants can only add to interruptions in access.

driven approaches like health savings accounts, accountable care organizations, and even state block grants can only add to interruptions in access. Vigilance in pursuing reimbursement policies that provide access for patients through chosen providers will require a constant effort with decision-makers during these uncertain times. ●

Inside PPTA

MEET THE PPTA STAFF

Larisa Cervenakova

MEDICAL DIRECTOR, M.D., PH.D.



Q How long have you been with the Association?

I joined PPTA in November 2016 but it feels like I have been with the organization significantly longer.

Q What do you focus on in your role as Medical Director?

Since I joined PPTA very recently, I am still in the process of learning from my colleagues at PPTA. So far, it has been a great experience! For the future, in my role as a Medical Director, I would like to contribute to our common goals by bringing plasma protein therapeutics to places in which there is a demand for these life-saving products and to the patients who are not adequately treated or not treated at all.

Q Tell us about your background.

I graduated with a Medical Doctor degree in 1981 from the Pirogov Russian National Research Medical University in Moscow, with a specialization in biochemistry. That same year I moved to live in the former Czechoslovakia. One year later, I joined the Drug Research Institute in Modra where I spent five years enjoying my favorite subject, immunology, while investigating the effect of new drugs on the immune system. This experience extended to the studies of the altered T-cell immune responses in patients with subacute sclerosing panencephalitis, a chronic form of measles, at the Institute of Preventive and Clinical Medicine in Bratislava (Slovak Republic). This research led to a Ph.D., which I received from the Comenius University in Bratislava in 1992. Next, I joined the Laboratory of Central Nervous System Studies at the National Institute of the Neurological Disorders and Stroke, which is part of the National Institutes of Health (NIH). I was an International Fellow supported by the J.E. Fogarty International Center working in Dr. D. Carleton Gajdusek's lab. Dr. Gajdusek is a Nobel Laureate for the discovery of "kuru", the disease

that occurred in certain tribes of aboriginals of Papua New Guinea practicing ritualistic cannibalism. In 1991, I spent four months working in this laboratory under the supervision of Drs. Lev Goldfarb and Paul Brown and successfully hunting for mutations in the prion protein gene of patients with familial forms of transmissible spongiform encephalopathies (TSEs)/prion diseases.

I joined the Plasma Derivatives Department at the American Red Cross (ARC) in 1997 and worked on multiple projects related to the safety of the blood supply and plasma-derived products. With the help of my colleagues, we were able to develop a strong program addressing the transmissibility of variant Creutzfeldt-Jakob (vCJD) through blood transfusion in experimental models. During that time, I met many of the experts on the PPTA Pathogen Safety Steering Committee. I also had the opportunity to work directly with TSE-afflicted families, which I consider to be one of the most important and fulfilling experiences in my career.

Q What's your favorite city in the world?

I consider myself a "nomadic" person and it is difficult for me to pinpoint just one city which I like the most. Each city has its own character, flavor, smell, people, and memory. I can fall in love just with one street or a square or a building or a park or a church or a piece of art or music that resonates with the place. If I close my eyes, I see all these places at the time of the year when I visited them. I can vividly smell the standing waters of the canals in Venice, the wooden structure of the Holyrood Palace in Edinburgh, the fresh

snow falling on the streets, squares, bridges, and fountains of St. Petersburg, and the hot air and dog presence on the streets of Erice in Sicily. I can hear the bells of the Pražský orloj, a medieval astronomical clock in Prague, and the vocal conversation in Italian on the Spanish Steps in Rome. If I think of Vienna, it is Mozart, Strauss, and Klimt and an apfel strudel with a single shot of espresso accompanied by a glass of water. I enjoy standing on the pier in Palanga, Lithuania that extends into the Baltic Sea and feeling the strong northern winds, which take your breath away.

Q What characteristic do you most admire in others?

Honesty, compassion, and trustworthiness.

Q Tell us something that may surprise us about you.

I like to express feelings and ideas by writing poetry in Russian and enjoy surprising my friends with rhythms on special occasions. I still cannot achieve such proficiency in English.

Q Who's been an inspiration to you in your life?

My writing inspiration has always been Dr. Paul Brown, who never judged my English writing skills even when pieces needed significant editing. My inspiration for endurance and self-motivation is Dr. Lev Goldfarb, who helped me to settle in the U.S., provided unselfish mentorship, and entrusted

me with the invaluable samples from patients with various neurological diseases, for molecular genetic analysis. Many of you may remember Dr. Bill Drohan, the Chief of the Plasma Derivatives Department at the ARC, who was an example of a strong leader and a human being ... he even remembered the names of each individual's spouses and children! I can continue with the list of people who were inspirational, supportive, and had a significant impact on my life, but I am afraid that this writing would then become an elaborate memoir. Many of you in the industry became my life-long colleagues and friends and I am grateful to you.

Q How did you get involved in this industry?

My five years of work on transmissible spongiform encephalopathies at the NIH were ending at the time when the epidemic of vCJD started in the United Kingdom. The uncertainty about the vCJD threat to the blood supply brought me to the J.H. Holland Laboratory of the ARC to address some of the questions that the plasma and blood industry faced. I have read almost every issue of The Source magazine from its first publication and kept on file many issues of the magazine. I call it a "wheel of life" that I was listed as a speaker of the PPTA meeting in Rome in 2002 and now I am a "happy camper" within this industry. ●



COMMEMORATING 25 YEARS OF SAVING AND IMPROVING LIVES

JUNE 13, 2017

This year, PPTA celebrates 25 years of saving and improving lives. In recognition of this, we will be hosting a black tie gala in Washington, D.C. on the evening of June 13, 2017 at the Mellon Auditorium in Washington, D.C. Registration will be in conjunction with the 2017 Plasma Protein Forum and will be open on a limited "first-come, first-served" basis. Watch the PPTA website for more information in the coming months. ●

One Company. Two Solutions.



**Haematologic
Technologies
Inc.**

Products and services for hemostasis research

- Purified blood coagulation factors
- Antibodies
- Factor deficient plasmas
- Customized blood collection tubes
- R&D assay services

HTI is a manufacturer of highly-purified, native plasma proteins and associated products involved in the hemostatic system. Development of assays for research or destined for cGMP testing is also offered.



haemtech.com



**Haemtech
Biopharma
Services**

Testing and services for protein biotherapeutics

- Thrombin generation assays
- Immunogenicity testing
- Stability & release testing
- Host cell protein mitigation
- Immunoassay development

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



haemtechbiopharma.com

Upcoming Events

CONFERENCES & SYMPOSIUMS

March

- 31 – Apr 4** 5th African Society for Immunodeficiencies (ASID) Biannual Congress
Livingstone, Zambia

April

- 6 – 9** Hemophilia Federation of America (HFA) Annual Symposium
Providence, R.I., United States
- 20 – 21** Spanish Hematology Meeting
Buenos Aires, Argentina

May

- 6** Guillain-Barre Syndrome (GBS) Foundation International Regional Conference
Baltimore, United States
- 16 – 17** International Plasma Fractionation Association (IPFA)/Paul-Ehrlich-Institut (PEI) 24th International Workshop on Surveillance and Screening of Blood-borne Pathogens
Zagreb, Croatia

June

- 13** PPTA 25th Anniversary Gala
Washington, D.C. United States
- 13 – 14** PPTA Plasma Protein Forum
Washington, D.C., United States
- 15 – 17** Immune Deficiency Foundation (IDF) 2017 National Conference
Anaheim, Calif., United States
- 22 – 25** European Hematology Association (EHA) 2017 Congress
Barcelona, Spain
- 23 – 25** 26th Annual Alpha-1 National Education Conference
Chicago, United States

July

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

August

- 24 – 26** National Hemophilia Foundation (NHF) 69th Annual Meeting
Chicago, United States

September

- 11 – 12** IPFA/Blood Centres of America (BCA) 3rd Global Symposium on The Future for Blood and Plasma Donations
Atlanta, United States
- 11 – 14** European Society for Immunodeficiencies (ESID): Autoimmunity & Inflammation in PID; Beyond the Paradox
Edinburgh, United Kingdom
- 23** Guillain-Barre Syndrome (GBS) Foundation International Regional Conference
Chicago, United States

October

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

November

- 8 – 10** International Primary Immunodeficiencies Congress (IPIC): Focus on Clinical Care and Diagnosis
Dubai, United Arab Emirates
- 8 – 10** 10th World Federation of Hemophilia (WFH) Global Forum on Research and Treatment Products for Bleeding Disorders
Montreal, Canada

GLOSSARY OF TERMS

A1AT – ALPHA-1 ANTITRYPSIN

AATD – ALPHA-1 ANTITRYPSIN DEFICIENCY

ACA – AFFORDABLE CARE ACT

ARC – AMERICAN RED CROSS

ARGE – ARBEITSGEMEINSCHAFT PLASMAPHERESE E.V.
(GERMAN WORKING GROUP ON PLASMAPHERESES)

ASID – AFRICAN SOCIETY FOR IMMUNODEFICIENCIES

BCA – BLOOD CENTRES OF AMERICA

BSE – BOVINE SPONGIFORM ENCEPHALOPATHY

COPD – CHRONIC OBSTRUCTIVE PULMONARY DISEASE

CWD – CHRONIC WASTING DISEASE

EAPPI – EUROPEAN ASSOCIATION OF THE PLASMA
PRODUCTS INDUSTRY

EFPIA – EUROPEAN FEDERATION OF PHARMACEUTICAL
INDUSTRIES AND ASSOCIATIONS

EHA – EUROPEAN HEMATOLOGY ASSOCIATION

ESID – EUROPEAN SOCIETY FOR IMMUNODEFICIENCIES

GBS – GUILLAIN-BARRE SYNDROME

HFA – HEMOPHILIA FEDERATION OF AMERICA

IDF – IMMUNE DEFICIENCY FOUNDATION

IPFA – INTERNATIONAL PLASMA FRACTIONATION ASSOCIATION

IPPIA – INTERNATIONAL PLASMA PRODUCTS
INDUSTRY ASSOCIATION

ISTH – INTERNATIONAL SOCIETY ON THROMBOSIS
AND HAEMOSTASIS

M/M – METHIONINE/METHIONINE

M/V – METHIONINE/VALINE

MEP – MEMBERS OF THE EUROPEAN PARLIAMENT

NHF – NATIONAL HEMOPHILIA FOUNDATION

NIH – NATIONAL INSTITUTES OF HEALTH

PEI – PAUL-EHRlich-INSTITUT

PPT – PLASMA PROTEIN THERAPY

PPTA – PLASMA PROTEIN THERAPEUTICS ASSOCIATION

PRNP – PRION PROTEIN GENE

sCJD – SPORADIC CREUTZFELDT-JAKOB DISEASE

SIPLA – SAFETY OF LONG-TERM INTENSIVE PLASMAPHERESIS
IN DONORS

TSE – TRANSMISSIBLE SPONGIFORM ENCEPHALOPATHY

V/V – VALINE/VALINE

vCJD – VARIANT CREUTZFELDT-JAKOB DISEASE



BE THE 1TM

Join Cristiano Ronaldo and Abbott to incorporate the **BE THE 1TM** program into your donor recruitment campaign. Please contact your local Abbott representative for details. **BE THE 1TM**.

Sign up to donate at BeThe1Donor.com





Aurora Power and Productivity



Aurora Plasmapheresis System

Aurora helps optimize plasma center efficiency, collects low cell count plasma, and enhances the experience for both operators and donors.

- Intuitive touch screen display lets operators manage and monitor each collection
- Donor display provides collection progress and icon prompts to engage donors throughout the procedure
- DXT Relay enables accurate data collection, remote procedure setup, and paperless documentation

Find out more today at:

www.fresenius-kabi.us/aurora



**FRESENIUS
KABI**

caring for life

Refer to the instructions for use for a list of warnings and precautions associated with the device.