

THE SOURCE

THE MAGAZINE OF THE PLASMA PROTEIN THERAPEUTICS INDUSTRY

Fall 2010



All Roads Lead to the Patient

**PPTA Interview:
Peter L. Saltonstall,
President and CEO
of NORD**

**10 Years of the
Orphan Regulation
in Europe**

**Expert Recommendations
for Better Management
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relating to plasma protein therapies,
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THE SOURCE magazine may contain
statements of opinion on such issues.

These statements are those
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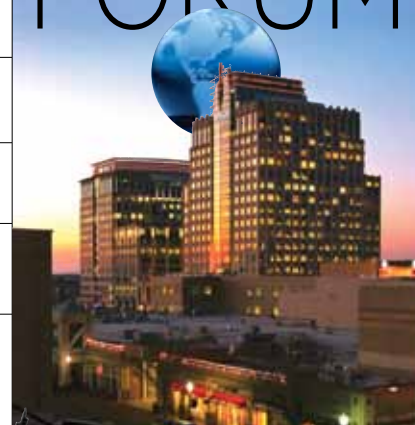
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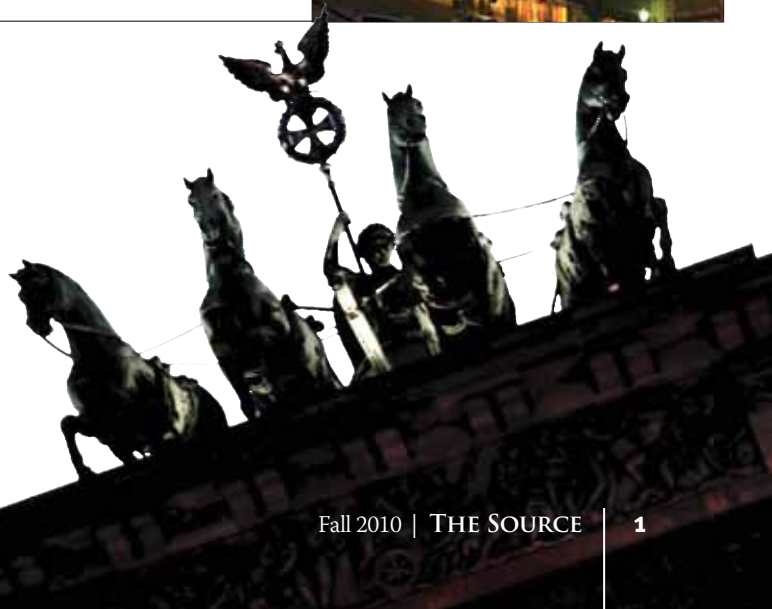


2010 PLASMA PROTEIN FORUM



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IN MY VIEW

WORLD CONGRESS OF HEMOPHILIA Highlights Progress in Care and Work to be Done



I AM WRITING THIS COLUMN a few weeks after my return from the World Congress of Hemophilia in Buenos Aires, Argentina. It was an amazing event in many ways. It was the week where the last games for the World Cup soccer were played in South Africa. My country, The Netherlands was playing Spain in the final. On the day of the final, you saw many Dutch participants dressed in the typical orange outfits, but also Spanish people with the red and yellow. The game was won by the Spanish team. In fairness, the Spanish team was stronger and more dominant, whereas the Dutch team had the better chances. We lost and it was another contribution to a bad day. That morning, I was a victim of an old exchange trick in a taxi that left me with fake money and, in the evening, my wallet was stolen, so I lost all my credit cards and licenses.

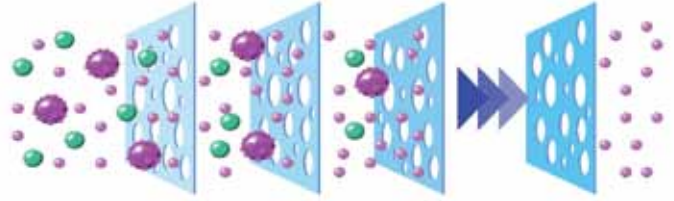
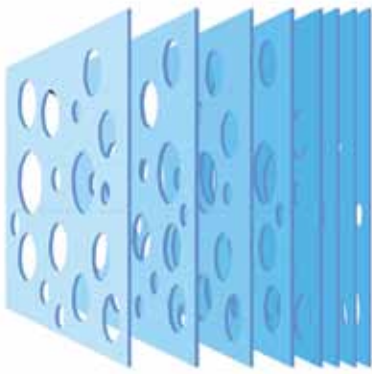
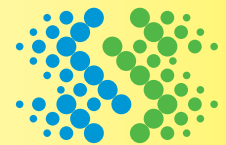
The Congress was impressive. Good scientific sessions and more than 4,300 participants. Mark Skinner and his team at World Federation of Hemophilia (WFH) did a very good job! I want to mention three things in particular.

First, just being at the Congress shows you the enormous progress that has been made in hemophilia care. The number of persons with crutches or in wheel chairs is reducing at every Congress. The number of children that walk around without any limitation is increasing and there is a demonstration of the benefits of early treatment. What a difference with the situation ten-to-20 years ago! I am proud of the treatment experts and the industry that has made this happen.

Secondly, I attended one session where an expert from Turkey presented on a series of about 150 children with treatment and complications after circumcision. As we all know, circumcision often is the first indication that a boy has hemophilia. In this presentation we heard about many complications, and that the main reason for circumcision in many countries is cultural and, therefore, family pressure is immense. When I asked the question why not say no to a family, when there is a medical reason not to perform the cultural or religious minor operation, I received a very vague answer and realized it made the presenter uncomfortable. I do understand that in many cases for a first boy it is difficult to anticipate hemophilia, except when there is a family history. But that is different for a second child. I respect cultures and religions, but I also respect a human life. I find it difficult to accept that family pressure is more important than medical risk.

Finally, I also met a woman, Cheryl, whose two stepdaughters have a rare Factor V deficiency. She wrote a book called: *Pooling Blood*. It gives a very good description of what this family had to endure with the medical world for their two daughters. There are very good doctors and nurses and there are others who, because of a limited understanding of this rare disease, can make life-threatening decisions. The lesson for me is that it is always good to listen to parents, they know their children best and certainly when there is a medical problem known to them. Thank you, Cheryl, for sharing your story with me. 🏈





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PETER L. SALTONSTALL

President and CEO of NORD

THE NATIONAL ORGANIZATION FOR RARE DISORDERS (NORD) has

championed individuals suffering from rare diseases, most of which do not have effective treatment, for nearly three decades, advocating for medical research and development, providing education to families, and working with government leaders to facilitate a path toward the development of treatments for all rare diseases. The following interview with Peter Saltonstall, the president and CEO of NORD, showcases the groups' roots and advocacy priorities and how it is encouraged by new thinking at the U.S. Food and Drug Administration (FDA) to support the development of orphan treatments, while knowing full well there is much more work to be done.

For those unfamiliar with NORD, please describe the organization, its mission and your background and role with the organization.

NORD provides a voice for the nearly 30 million Americans who have rare diseases. It was established in 1983 by patient leaders who helped get the *Orphan Drug Act* enacted. Since then, NORD has been providing leadership and unity to the rare disease community through programs of advocacy, education, research and patient services.

I joined NORD two years ago because, having a family member with a rare disease, I was fascinated by the opportunity to provide advocacy for this community. Also, I've been very involved in issues related to patient safety through my previous work, and representing patients and their families—who can't always speak for themselves—is important to me.

My charge from NORD's Board of Directors was to bring this organization, which had a wonderful history of advocacy and patient representation, to the next level so that it can cut through the background noise to make the patient voice heard even more effectively. This required rebuilding the infrastructure, adopting new technologies and addressing the need for greater diversity in revenue sources. We've made some good progress on all those fronts that will begin to be apparent over the next few months.

The U.S. Food and Drug Administration (FDA) recently held a public hearing, which NORD was instrumental in bringing about, seeking to hear more from the rare disease community. What would NORD like to see FDA do to address the growing needs of rare diseases patients that would address clinical, regulatory and reimbursement challenges?

One of the things that happened during my first year at NORD was that we hosted a Summit in Washington D.C. to bring together some of the best minds in this space to address two questions: How can we stimulate more innovative development of treatments for rare diseases? How can we ensure that patients have access to treatments?

We had a blue ribbon panel and speakers who included National Institutes of Health Director Francis Collins M.D, Ph.D, former Health and Human Services Secretary Tommy Thompson and several very senior-level people from FDA. Former FDA Commissioner David Kessler, MD, JD, served as moderator.

And what people said consistently was that we need to provide basic tools like patient registries and disease natural history studies for research, and we need to look at ways to de-risk the regulatory process. NORD believes that one way to do that would be for FDA to

develop a statement of policy related to rare diseases and orphan products.

Historically, even though orphan products are evaluated using the same standards of safety and efficacy that are required for other products, FDA has taken into account the limitations imposed by working with small patient populations when reviewing these products. However, there's no statement of policy to ensure that this is done consistently or in a predictable manner. NORD believes companies would be more willing to make the investment in research and development if FDA had such a written policy. This is one of the things the chairman of our Board of Directors talked about when he spoke on behalf of NORD at the public hearings on rare diseases FDA hosted this summer.

I should add that NORD is delighted that FDA created a new position, Associate Director for Rare Diseases, in the Center for Drug Evaluation and Research. NORD had been advocating for this. And, we also work very closely and well with FDA's Office of Orphan Products Development.

Can you tell us about NORD's work with the Rare Disease Caucus, how this fits into NORD's legislative agenda and what outcomes you hope to see from it?

NORD has been working very hard with our contacts on Capitol Hill to get the

NORD

National Organization for Rare Disorders

Caucus established. We think this is important because the Caucus can help focus Congressional attention on the fact that almost 6,800 rare diseases still have no treatment and that, when treatments exist, patients with rare diseases often can't access them because of reimbursement issues. The Caucus will help to ensure sufficient funding for research and incentives to get researchers interested.

How do you think the new federal health reform law will affect those with rare diseases and disorders seeking treatment or hoping new treatments will be brought to market?

NORD was actively involved in advocacy for health care reform. Some of the big issues for us were eliminating discrimination related to pre-existing conditions and discontinuing annual and lifetime insurance caps. These caps are a huge problem for people with certain rare diseases that require ongoing treatment. We see patients in their early 20s, or even younger, who are approaching their lifetime cap.

NORD was happy with the legislation that was ultimately enacted. Now, we're just watching carefully to make sure it gets implemented in the ways that were intended. ☞

JULIE A. BIRKOFER is PPTA's Senior Vice President, North America

Rare Disease Caucus Formed

U.S. Representatives Joseph Crowley (D-NY) and Fred Upton (R-MI) have formally announced the newly-established Rare and Neglected Diseases Caucus. As co-chairs of the Caucus, Crowley and Upton will focus on:

- Bringing Congressional attention to the 6,800 known rare diseases that currently have no approved therapies
- Ensuring sufficient funding for research and orphan product development
- Exploring ways to incentivize companies to create new drugs, biologics and humanitarian use devices
- Providing an opportunity for Members of Congress, families and advocacy groups to exchange ideas and policy concerns

A "dear colleague" letter went out in early July to encourage Members of Congress to join the caucus. The National Organization for Rare Disorders (NORD) was instrumental in advocating for its formation.

NORD's CEO Peter Saltonstall said, "NORD believes the Rare and Neglected Caucus is important because it will help focus attention on the 6,800 rare diseases that have no treatment, the need for research funding, and the need to incentivize orphan product development. The Caucus will also provide a forum where Members of Congress, advocacy groups and families can exchange ideas and policy concerns."

PPTA supports the goals of the Caucus and looks forward to its growth and influence to enhance patient access to therapies that treat rare diseases and disorders.



Joseph Crowley



Fred Upton

UNCERTAINTY REIGNS IN THE

BY SYBILLE BECK

BETWEEN 6 AND 15 BILLION EUROS, or \$7.7 and \$19.4 billion USD, is the expected deficit of the German Statutory Health Insurance (SHI) in 2011. The SHI (in contrast to the private health insurance) in Germany covers approximately 70 million people out of 82 million inhabitants of the country. The system roots in the social legislation introduced by Chancellor Otto von Bismarck in 1883. It is financed equally by employers and employees¹. Relatives without their own income are included in the insurance without contribution. Thus the fact that virtually everybody is covered by health insurance and has access to a well-developed and sophisticated health system (e.g. free choice of doctors, state-of-the-art facilities, reimbursement of virtually each marketed drug) is an inherent part of the social provisions in Germany and taken as a matter of course – which it is not and especially not in economically challenging times.

The expenditure of the SHI has increased steadily in recent years (see Fig 1 below) with the major cost centers being (based on figures from the German Ministry of Health from 2008): hospital treatment (32.7 percent), pharmaceuticals (18.2 percent), medical care (15.1 percent), SHI administration costs (5.2 percent) and dental treatment (5.0 percent).

Several governments have made it a priority to reform the German health care system and to put it on more financially solid grounds, but a really fundamental reform—whatever it may look like—is still missing. Since health care concerns everybody, hence, every voter and the stakeholder groups with

their vested interests are powerful on an economic and a political level, it is difficult for any politician to make changes without jeopardizing the next elections.

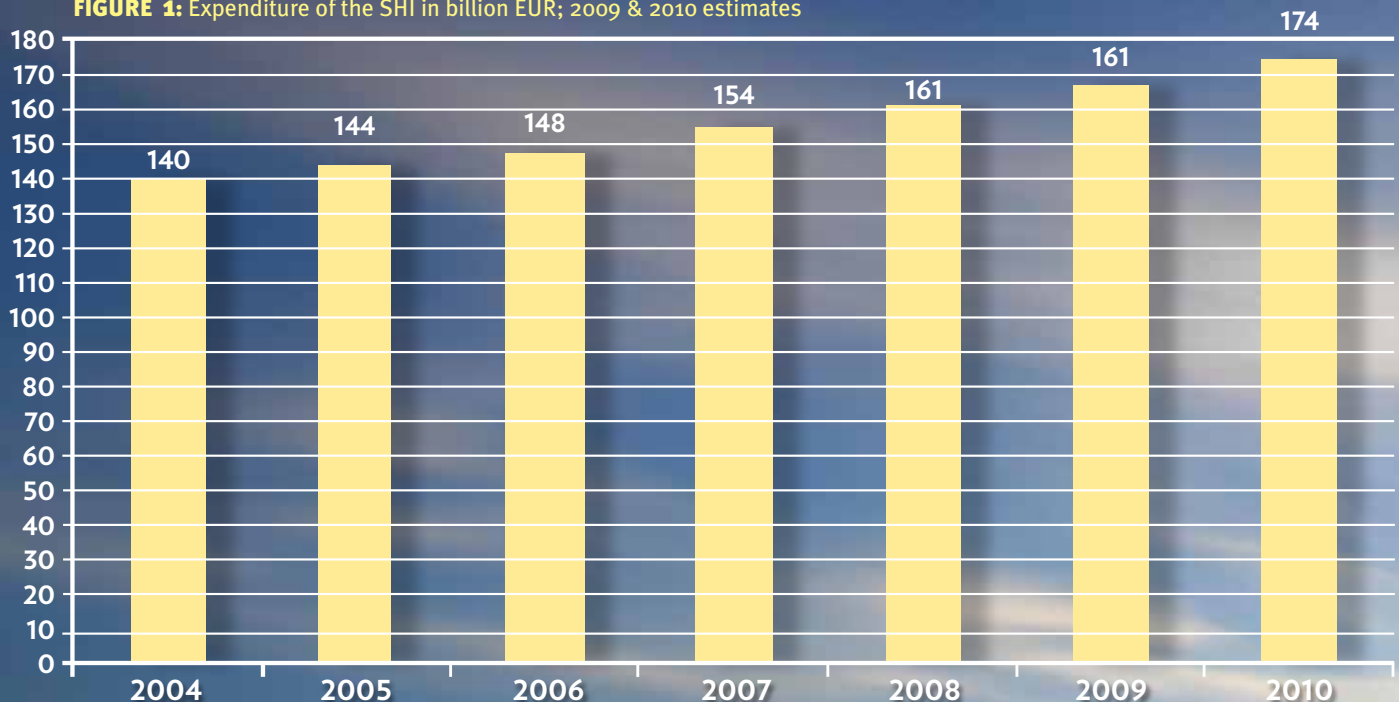
In light of the current situation – an economic recession, higher unemployment rates, decreased income, and an aging population – the government has to make changes to save on the expenditures and to find new ways of funding the SHI.

Saving on medical care, however, especially physicians, is a difficult undertaking on multiple levels: the income structure for physicians be it in hospitals or in private practices is low in Germany compared to other European countries resulting in a “physician flight” to countries such as the United Kingdom, where salaries are significantly higher. Furthermore, German physicians successfully pushed for improved work conditions including higher salaries by going on strike with backing in the population, and it is unlikely that significant cuts can be made in this area, although the Ministry of Health has plans to do so.

As a first measure to cut costs in the SHI, the German Health Minister Dr. Philipp Rösler published a legislative proposal in which he outlines both structural long-term plans and short-term measures to cut expenditures on pharmaceuticals. The short-term measures, which will come

¹14 percent are split equally between employer and employee, whereas additional 0.9 percent are payable by the employee only.

FIGURE 1: Expenditure of the SHI in billion EUR; 2009 & 2010 estimates



SOURCE: GERMAN MINISTRY OF HEALTH; SCHÄTZERKREIS

GERMAN HEALTHCARE SYSTEM

into force on August 1, 2010 and will also seriously impact the plasma industry with the rationale being that *“the pharmaceutical industry due to the economic and financial crisis only had to bear little losses compared to the overall economic development. The burden for pharmaceutical companies is therefore in relation to the objective to stabilize the expenditure of the SHI for a limited timeframe of three years and five months reasonable.”*

- Increase of the mandatory rebate payable on pharmaceuticals (without maximum price) distributed by the retail pharmacy from 6 percent to 16 percent
- Rebate based on price valid August 1, 2009
- Price moratorium until December 31, 2013

During the last meeting of the Health Committee of the German Parliament, an additional measure was introduced:

- All pharmaceuticals distributed by the hospital pharmacy to the outpatient clinic are subject to the mandatory rebate

The fact that the pharmaceutical industry in general does not have a good image in the general public makes it also an easy target with little objections to cost saving measures. “The last amendment also foresees possibilities for pharmaceutical companies to apply for an exemption of the rebates. These opportunities, however,

are only applicable for companies whose economic survival is in real danger and needs to be proved by certified numbers based on the 2009 financial result.

PPTA members have alerted representatives of the German Ministry of Health that in spite of cost containment measures other European countries, e.g. United Kingdom, Belgium and Greece, have granted exemptions for plasma protein therapies on the grounds of the special cost structure and treatment of rare diseases. PPTA and its members will continue to emphasize these intrinsic differences compared to other sectors of the pharmaceutical industry and how these are addressed by other governments to ensure availability of life-saving drugs for patients in need.”

SYBILLE BECK is PPTA's Senior Manager, Source Europe

Several governments have made it a priority to reform the German health care system and to put it on more financially solid grounds, but a really fundamental reform—whatever it may look like—is still missing.



On May 3-4, 2010,
the European
Medicines Agency
(EMA) held a
conference to
discuss the
10 years of the
Orphan Regulation
in Europe.

**Medicinal products for
treatment of orphan diseases
of a different nature share
a number of constraints,
such as few patients
in different countries
with different requirements
regarding clinical trials,
adding to the complexity
and high costs for the
development of
medicinal products
in general.**

10 YEARS OF THE ORPHAN REGULATION IN EUROPE

BY ILKA VON HOEGEN

THE OBJECTIVE OF REGULATION EUROPEAN COMMISSION (EC) NO 141/2000 is to provide the same quality of treatment for persons suffering from rare diseases as other patients. Incentives for research and development and for placing on the market of medicinal products for rare diseases (orphan medicinal products) and a community procedure should help to turn the objective into reality. This article gives a summary of the discussions among regulators, patients, physicians and industry. Have the tools been sufficient to achieve the goal? What is still needed to get there? What are the constraints and limitations?

Development of Products for Rare Diseases: Incentives, Reality and Future Directions

Medicinal products for the treatment of orphan diseases of a different nature share a number of constraints, such as few patients in different countries with different requirements regarding clinical trials, adding to the complexity and high costs for the development of medicinal products in general. Often there are also no specific provisions for reimbursement of orphan medicinal products. Consequently, manufacturers do not look at a solid basis that predicts appropriate reward of their investment. A better alignment between registration and reimbursement in the form of conditional reimbursement with ongoing data collection would encourage companies to commence the challenging development of an orphan medicinal product.

In view of the limited number of patients, a harmonized data collection on the European Union (EU) level, for example in data registries, is a prerequisite for the successful development of orphan medicines. All stakeholders agree that specifically in orphan indications more international registries following a standard methodology in data management and analysis are needed to ensure harmonized approaches and provide cross-border information. Not unexpectedly, a major constraint in the establishment of international registries is the lack of funding, which should be a joint initiative of all EU member states, rather than isolated initiatives in a few. In France, fundraising for rare diseases has been quite successful, but lacks the international dimension.

Financial incentives to manufacturers, such as fee waivers, free

regulatory advice or tax exemptions must be considered to improve patient access to orphan medicinal products. Today, there are no sufficient incentives on EU or national level. Centralized Health Technology Assessments (HTAs) could provide the right arguments to support the benefits of incentives.

For example, in the U.S. a 50 percent federal tax credit on a clinical trials expenditure, no Marketing Authorization Application (MAA) fees and a significantly larger program on grants for clinical development have led to an increase of development of orphan medicinal products in the past years. In addition, seven years of market exclusivity reassures manufacturers that their investment was well placed.

From a regulatory perspective, the European Medicines Agency's (EMA) role in the development of orphan medicinal needs to be enhanced in providing more support before and throughout the entire development of an orphan drug, thus reducing uncertainties in regulatory outcomes and accelerating time to approval of a marketing authorization.



Research for Rare Diseases: Translation into New Drugs for Rare Diseases—The Role of Academic Researchers

Government funding for research in areas of rare diseases is scarce, but scientists need sustained financial support for starting and continuing studies to increase understanding of the causes of rare diseases. Consequently, a partnership with industry is simply unavoidable. Rare diseases impose specific challenges on the partnership of researchers and industry, because experts are scarce and a conflict of interest may arise. Only a transparent collaboration with better definitions of the roles of the different players can help to avoid or reduce conflicts of interests. External experts involved in decision making processes could provide an unbiased view.

Researchers, whether in the academic setting or in an industry environment, are equally challenged by the specific needs for clinical trials in orphan indications. The limited number of patients requires an overarching, multicenter approach in many countries to enroll a sufficient number of patients. But often disharmonized national requirements get in the way. Also, the quality of evidence as the primary goal, is hampered by the limited number of patients. In many orphan indications the knowledge of the underlying mechanisms is often very scarce, making it difficult to design the correct endpoints in a clinical study. These constraints highlight the need for consolidated and standardized data collection and management.

Patients' View on the Regulation's Implementation. What is Still Needed?

Patients with rare diseases are often caught in the dilemma between the need to seek alliances with other patient organizations to promote awareness of rare diseases in general, against the needs of their own organization. Different patient groups may find themselves in competition for resources and funding. Only a well structured organization can address issues on the local, national, European and international levels. Particularly, groups of patients with rare diseases often have neither the experience nor resources to cover all levels. Consequently, a wider alliance of different groups is needed to bring their specific issues successfully forward.

Public awareness about the specific nature of rare diseases within the general public and among the educated stakeholders should be one of the key activities of patient groups. It cannot be emphasized enough that society as a whole benefits when treating rare diseases, instead of ignoring these groups of patients. It has to be noted that, although there are few patients suffering from the individual disease, there are more than 30 million people with orphan diseases in Europe. HTAs can be a helpful tool to provide supporting arguments. It is also accepted that better understanding of the underlying mechanisms in orphan indications might lead to spin-offs to indications for more common diseases.

Patient groups in need of orphan medicines would like to be more actively involved in the whole development process of their treatments, such as in development of regulatory frameworks at the national and European levels and in defining what is clinically relevant in the treatment of their conditions. Particularly in the area of clinical trial design, patient experience can be a valuable asset.


Conclusion

There is a general consensus that the orphan regulation has been a successful initiative to raise awareness and to promote treatment of

rare diseases, but there are still many areas in need of improvement. While therapy development for rare diseases has a number of intrinsic challenges in addition to those in more common conditions the biggest challenge in the future might be the lack of funding and provision of incentives due to the increasing limitations in health care spending in most EU member states.

If there is no funding in academic research to increase the understanding of the underlying mechanisms, how can potential treatment strategies be developed?

If a company cannot predict whether investing in the development of a new therapy will result in appropriate revenues, why would it embark on such a venture?

In conclusion, the European Union and its Member States need to continue their consolidated efforts in building the framework for successful development of orphan medicinal products, despite the unfavorable economic environment, in the interest of patients and society as a whole. 

There is a general consensus that the orphan regulation has been a successful initiative to raise awareness and to promote treatment of rare diseases, but there are still many areas in need of improvement.





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RUEDI WÄEGER

HONORED WITH

REILLY AWARD 2010

Recognizing his significant contributions to the plasma protein therapeutics industry, Dr. Ruedi Wäeger was honored by PPTA with the prestigious Robert W. Reilly Award at the 2010 Plasma Protein Forum in Reston, Virginia.

BY KARA FLYNN

Dr. Wäeger was honored by PPTA for his tremendous efforts to encourage industry to continue its momentum forward in all areas: improving patient access to therapies; ensuring the highest possible quality and safety of plasma therapies; working toward world-wide regulatory harmonization; improving the image, reputation and credibility of industry; and educating people about our therapies.

Each year, PPTA awards the Robert W. Reilly Award to recognize an individual who best exemplifies the leadership qualities of Robert W. Reilly, a leader of the plasma therapeutics industry association.

Dr. Wäeger studied biochemistry at the Swiss Federal Institute of Technology and has spent the past 30 years in the pharmaceutical and therapeutic protein industry. Currently, he sits on the Board of Talecris Biotherapeutics, Inc. Most



recently, he was President and Chief Executive Officer of Aventis Behring L.L.C., a global plasma protein therapeutics business which was acquired by CSL Ltd. in 2004 to form CSL Behring. Prior to this position, Mr. Wäeger was President and Chief Executive Officer of ZLB

Central Laboratories, Blood Transfusion Service of the Swiss Red Cross. Before that, he spent more than 20 years at Sandoz Ltd., where he had consecutive worldwide responsibilities for Strategic Research and Development Planning, Human Resource Management, and Marketing, including responsibility for three global product launches.

In accepting the award on Dr. Wäeger's behalf, Tom Lynch of Talecris Biotherapeutics, highlighted four groups of people to whom Dr. Wäeger wanted to dedicate his award including the employees of member companies who work day and night with great passion to collect safe plasma; plasma donors who want to help those patients who need plasma products; patients who support our industry and who regained confidence in our activities and products and the Association staff, who works hard and effectively—often behind the scenes—with patients and patient organizations, regulators, and governmental bodies to advance the success of the whole industry and the member companies. ☺



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formerly of Nabi Biopharmaceuticals

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Baxter BioScience/
BioLife Plasma Services

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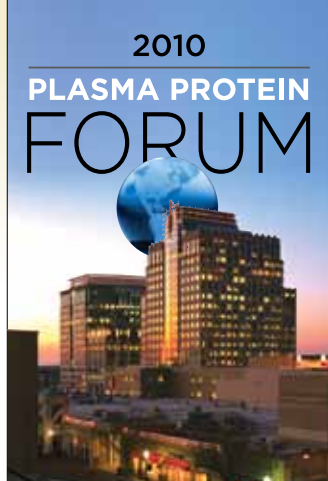
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formerly of
Bayer Corporation

2001
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Chairman of the Board, Grifols

2000
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Serlogicals Corporation

1999
JACK RYAN
formerly of
Bayer Corporation

1998
JOHN BACICH
formerly of
Baxter Healthcare Corporation



2010 PLASMA PROTEIN FORUM

Unites Leaders in Reston, Virginia

BY KARA FLYNN

PLASMA PROTEIN THERAPEUTICS INDUSTRY LEADERS gathered in Reston, Virginia for the annual Plasma Protein Forum held June 15-16. The event, which featured attendees from government agencies, patient advocacy groups, physicians, consumers and policymakers, drew close to 300 attendees who took part in the two-day meeting. Topics addressed at the conference included strategies to improve patient access, pandemic preparedness, the ethics of compensation, improving global access to therapies, health care reform and a special question and answer period with representatives from the U.S. Food and Drug Administration (FDA).



Anne Pariser, M.D. and Nisha Jain, M.D. of FDA presented the Forum's keynote address.

Presenting the key note address, Anne Pariser, M.D., associate director for rare diseases at FDA and Nisha Jain, M.D., chief of the clinical review branch of the Office of Blood Research and Review at FDA discussed the new prominence that rare diseases are receiving with the establishment of a newly created position within the Center of Drug Evaluation and Research (CDER), that will ultimately lead the development of the agency's first time drug center policies for reviewing and approving rare treatments. The two speakers discussed the synergy within FDA that will help patients cope with rare diseases and manufacturers seeking to

bring orphan products that can treat them to market.

Another Forum highlight came during a discussion of the current economic troubles facing policymakers in the U.S. Congress and the Administration. Jim Nussle, president and chief executive officer of The Nussle Group and a former director of the Office of Management and Budget and a Republican Member of the U.S. House of Representatives addressed the budget process and the challenges that legislators face when reducing health care spending. In addition, Mr. Nussle discussed the delicate balance between cost containment measures and maintaining patient access.

A dialogue on "Global Access to Plasma Protein Therapies" with renowned speakers including Shigeaki Nonoyama, M.D., Ph.D., professor and chairman of the department of pediatrics at the National Defense Medical College in Japan; Jordan Orange, M.D., associate professor of pediatrics at the University of Pennsylvania's School of Medicine; Kenneth Chapman, M.D., professor of medicine



Jim Nussle, president and chief executive officer of The Nussle Group

and co-Chairman of the Collaborative Respiratory Research Program at the University of Toronto and Donna DiMichele, Deputy Director at the Division of Blood Diseases and Resources, National Heart, Lung and Blood Institute addressed the care of patients needing access to plasma protein therapies. Participants discussed areas of their work such as medical education, generation of clinical evidence and diagnosis which are needed to ensure and enhance access. Prof. Albert Farrugia, PPTA's senior director, global access moderated a vigorous session of comments on which areas can be improved in order to increase awareness of rare disease states.



Global Access Panel Participants pictured from left to right: Dr. Donna DiMichele, Dr. Kenneth Chapman, Prof. Albert Farrugia, Dr. Jordan Orange, and Dr. Shigeaki Nonoyama.



Donors, Patient honorees attend Recognition Ceremony at the Forum.

PPTA Recognizes Donors, Patient Representative with Recognition Awards

Plasma donors and a patient representative were honored by PPTA in a special recognition ceremony intended to express appreciation for their dedication to increasing awareness of the serious, chronic, genetic diseases treated by plasma protein therapies and helping others during the Plasma Protein Forum in Reston, Virginia on June 15.

Honorees included Raymonde Marius, an anti-D donor who has donated plasma for over 40 years, Samuel and Alvin Collier, brothers who have donated plasma for over 10 years and Christine Libertino, vice president and advocacy chair at the Hemophilia Foundation of Maryland, who has been tireless in her efforts to improve access to plasma protein therapies.

"We honor these outstanding individuals for the commitment they have shown to saving and improving lives," said PPTA President Jan M. Bult.



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PPTA Represents Rare Diseases Treated with Protein Therapies at FDA Meeting

Industry heard from two officials at the U.S. Food and Drug Administration (FDA) at the Plasma Protein Forum in mid-June regarding the agency's coordinated and patient-centered approach to rare diseases. The theme of rare diseases continued later than month when PPTA took part in an FDA public hearing focused on regulation and treatment of rare diseases. Mary Gustafson, vice president of Global Regulatory Policy presented information about the rare diseases treated with plasma protein therapies, the challenges with bring plasma-based therapies to market and regulatory solutions that could improve patient care and access. The National Organization for Rare Disorders (NORD) was a driving force in encouraging the agency to hold the public meeting (see interview with NORD CEO on page 4).


Common themes included the need for regulatory flexibility and FDA internal changes to effect flexibility with guidance documents outlining review expectations; need for patient-meaningful clinical endpoints and appropriate biomarkers; more expertise on rare diseases in the review of the orphan product; transparency in the review and orphan drug designation process; and an understanding that the rare diseases are more alike than not with review practices across therapeutic lines. The outcome of this public meeting will be considered by a FDA committee established under a recent law to review the agency's review practices as they relate to articles for rare diseases.



On the health care reform front, Carrie Budoff Brown, a reporter with the publication *Politico*, discussed how she covered health care in the year leading up to the passage of health reform legislation and provided an insider's unique anecdotes from covering President Barack Obama's campaign and how the news media and political reporting has changed over the years. Her insight into today's accelerated news cycle and the need for 24-7 coverage to provide information to the masses on the internet, proved to be popular with audience members who asked a number of questions about what it takes to cover a broad topic such as health care when you're dealing

with multiple, complex issues and organizations.

The 2010 Plasma Protein Forum's success was due in a large part to the support provided by sponsors and exhibitors. Their contribution and participation helped make this year's Forum and Exhibit Hall a truly interactive event. PPTA extends its sincere thanks to the Plasma Protein Forum sponsors and exhibitors.

Save the date for next year's Plasma Protein Forum at the Hyatt Regency Reston in Reston, Virginia, June 14-15, 2011. 

KARA FLYNN is PPTA's Director of Global Communications



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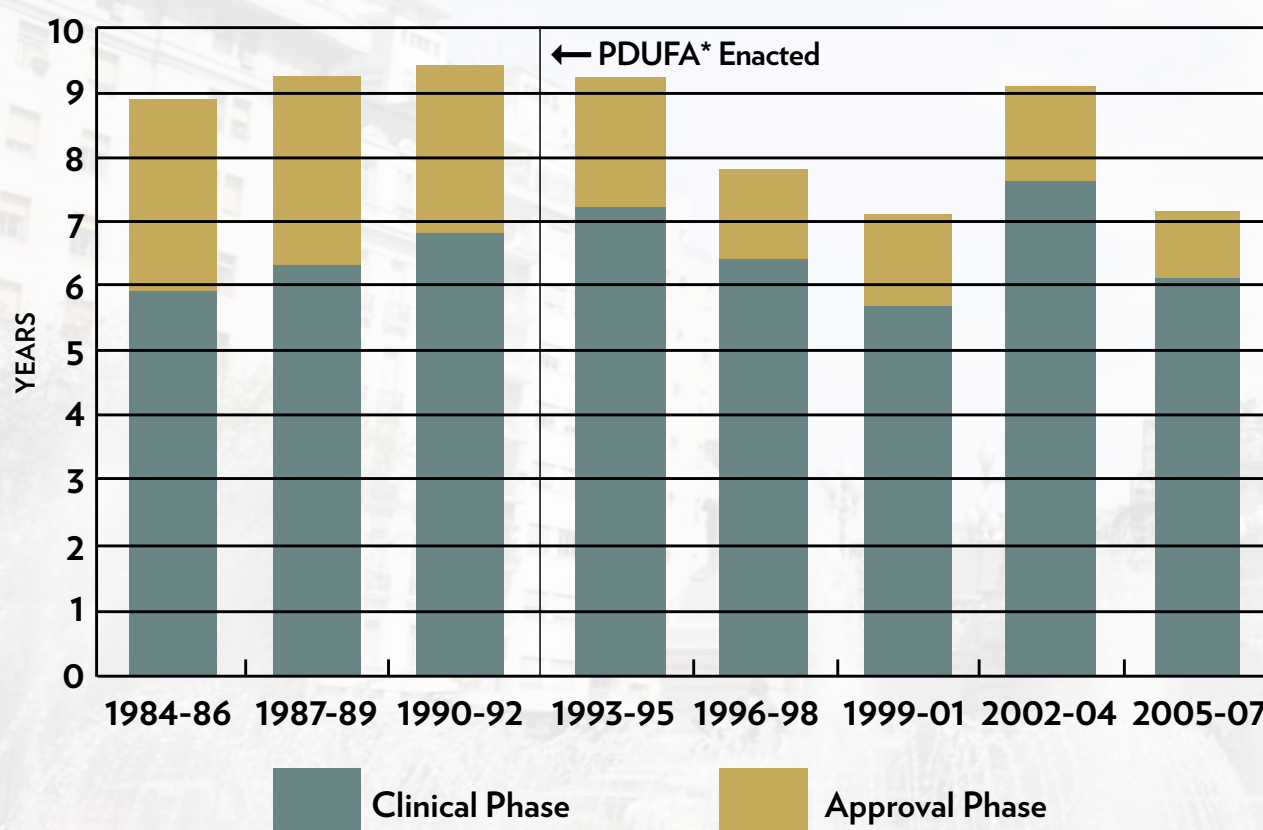
FOR PEOPLE WITH RARE DISORDERS

JUSTICE NOT DOGMA

I AM WRITING THIS A FEW DAYS AFTER participating in the Congress of the World Federation of Hemophilia (WFH) in Buenos Aires, Argentina. As always, seeing the bleeding disorders community come together with their treaters and the industry was a great experience, although mixed in some ways. It was great to observe how the availability of treatment products has emancipated these vulnerable patients. Mixed in with this good news, however, was the sobering realization of how much more needs to be done.

Which brings me to the story of Cheryl D'Ambrosio, whom I met at the Congress. Cheryl's story can be found on www.poolingblood.com, and I encourage you to learn about her journey caring for her two now-adult stepdaughters with coagulation factor V deficiency, a disorder of the clotting system affecting one in 1 million people. I was affected and inspired by Cheryl's story of her struggle for recognition and treatment of her girls' condition. People with Factor V deficiency need, like in all congenital bleeding disorders, replacement of the missing factor if they are not to suffer life-threatening bleeds. The disorder has been identified and understood in the medical community for a very long time, and the best form of treatment would be, as is the case with hemophilia, a concentrate of Factor V. There are no such products available, and Cheryl's girls,

FIGURE 1: Drug development and approval times



like all of these patients, depend on the transfusion of plasma for treatments. Hearing Cheryl's tale of her struggle against ignorance and constant impediments to access for the treatment reminded me of the situation faced by individuals with hemophilia 40 years ago. Transfusion of plasma, with its large volume, its propensity to result in allergic reactions and its risk of pathogen transmission, is, clearly not the preferred therapy. All these undesirable properties would be avoided with the development of a concentrate. The isolation of Factor V from plasma is not too difficult—I myself have had occasion to do it



We salute Cheryl D'Ambrosio, a woman of style and courage.

in the distant days of when I was a coagulation researcher. Why has this simple chemistry not given rise to a product?

Some might speculate that the reason lies on the rarity of the condition. Research suggests that Factor V deficiency affects one in 1 million individuals. Yet there are inherited protein deficiencies, which are equally rare, where products exist. For example, concentrates

of fibrinogen for the treatment of hereditary fibrinogen deficiency have been approved by regulators, and hereditary fibrinogen deficiency is rarer than Factor V deficiency. This naturally causes us to ask, "What makes the development of drugs such a difficult and lengthy process?" Clearly, it is expensive to develop medicines, involving as it does much research and development. However, the longest—and therefore costliest—phase of drug development in the so-called clinical phase, during which the drug is tried out—or trialed—in patients to ensure that it is safe and that it works, or is efficacious. [Figure 1] Such "clinical trials"



are, effectively, experiments in people to ensure safety and efficacy in the drug before it is approved for general use in the patient population.

The science of clinical trials has evolved over the past 50 years, and it has reached its pinnacle in the so called “randomized clinical trial” or RCT. In an RCT, the target patient population is split into two groups, one of which receives the drug, another of which receives an inert agent—the placebo. This “random” allocation of the treatment ensures that any contributors to the observed affect are solely the result of the drug, and do not ensue from particular features of individual patients, doctors, etc.

Now, RCT’s are absolutely the best way of assessing a drug’s efficacy when the number of patients is large and when the mechanism of the drug’s action is relatively uncertain. Under these conditions, an RCT can be designed, which will allow an answer to be generated on the drug’s efficacy. Such an RCT will generally need a substantial number of patients, will take a long time to conduct, and will therefore cost a lot of money. Many of these limitations are not too problematic for mass-produced pharmaceutical drugs intended for large numbers of patients—think statins or anti-hypertensives (such as erectile dysfunction drugs).

For biological drugs used to treat rare disorders such as coagulation factor deficiencies, the use of RCTs is very

Figure 2: Parachutes— should their use be subject to randomized trials?

Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials

Gordon C S Smith, Jill P Pell

Abstract

Objectives To determine whether parachutes are effective in preventing major trauma related to gravitational challenge.

Design Systematic review of randomised controlled trials.

Data sources: Medline, Web of Science, Embase, and the Cochrane Library databases; appropriate internet sites and citation lists.

Study selection: Studies showing the effects of using a parachute during free fall.

Main outcome measure Death or major trauma, defined as an injury severity score > 15.

Results We were unable to identify any randomised controlled trials of parachute intervention.

Conclusions As with many interventions intended to prevent ill health, the effectiveness of parachutes has not been subjected to rigorous evaluation by using randomised controlled trials. Advocates of evidence based medicine have criticised the adoption of interventions evaluated by using only observational data. We think that everyone might benefit if the most radical protagonists of evidence based medicine organised and participated in a double blind, randomised, placebo controlled, crossover trial of the parachute.

accepted intervention was a fabric device, secured by strings to a harness worn by the participant and released (either automatically or manually) during free fall with the purpose of limiting the rate of descent. We excluded studies that had no control group.

Definition of outcomes

The major outcomes studied were death or major trauma, defined as an injury severity score greater than 15.⁶

Meta-analysis

Our statistical approach was to assess outcomes in parachute and control groups by odds ratios and quantified the precision of estimates by 95% confidence intervals. We chose the Mantel-Haenszel test to assess heterogeneity, and sensitivity and subgroup analyses and fixed effects weighted regression techniques to explore causes of heterogeneity. We selected a funnel plot to assess publication bias visually and Egger’s and Begg’s tests to test it quantitatively. Stata software, version 7.0, was the tool for all statistical analyses.

Results

Our search strategy did not find any randomised controlled trials of the parachute.

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problematic. Indeed, when the disease is so clearly understood and associated with a specific protein deficiency, such as hemophilia, the use of RCT can be viewed as not only impractical but unethical. A famous article some years ago pointed out that the use of parachutes has never been validated through an RCT. (Figure 2). Prof. Wolfgang Schramm (see profile on page 24) has remarked that forcing hemophiliacs to undergo RCT's for clotting factors would be a similar situation. And yet, this is precisely what some extremist advocates of RCTs would have patients do. Despite the impossibility of generating statistical evidence of efficacy with the small patient numbers possibly into rare disorders, some advocates of evidence-based medicine, a principle that I passionately support, insist on RCTs for treatments for these patients. Recently, some of these people achieved media publicity by insisting that RCTs for the treatment of alpha-1 antitrypsin deficiency (AAT) with AAT concentrate showed no benefit for these patients. The PPTA's response to this allegation is on the

For biological drugs used to treat rare disorders such as coagulation factor deficiencies, the use of RCTs is very problematic. Indeed, when the disease is so clearly understood and associated with a specific protein deficiency, such as hemophilia, the use of RCT can be viewed as not only impractical but unethical.

Association's website at www.pptaglobal.org. The allegation is spurious and harms these patients' prospects of getting reimbursed treatment, which has been shown in ways more appropriate than RCTs to be effective. There are many ways to show efficacy for treatments—sticking to the dogma of "Just RCTs" harms patients.

And so, I continue to muse on Cheryl D'Ambosio and her

family and hope that their courage will be rewarded with justice. The justice of a safe and efficacious treatment for Factor V deficiency. I noted during the WFH Congress the development of a Factor X concentrate by a British company. And a product for fibrinogen deficiency is already licensed in the U.S. These two diseases are as rare as Factor V deficiency. Hopefully, one day someone will stand up for Cheryl's Girls. (<http://www.mygirlsblood.com/>)

PROF. ALBERT FARRUGIA is PPTA's Senior Director, Global Access



PROFESSOR WOLFGANG SCHRAMM

University Hospital of Munich

1. How would you describe the treatment situation of German hemophilia patients as compared to other EU Member States?

In Germany, hemophilia patients receive the best treatment with eight units of clotting factor concentrates per capita. The same level of treatment can be found in Sweden. In other European countries, patients have more barriers to good treatment, for example, in Great Britain the tendering process has a restrictive effect on access to care. The worst situations can be found in Eastern Europe, for example in Romania, Bulgaria and other countries in the region, where patients often have no adequate access to care because of the difficult financial situation of these countries. In the international comparison, the U.S. is positioned in the middle of the treatment range with ca. five units per capita.

The second Wildbad Kreuth Initiative on “Optimal clinical use of blood components,” which was held under the auspices of the Council of Europe’s *European Directorate for the Quality of Medicines (EDQM)* in 2009, concluded in the recommendations that countries should strive to achieve a minimum treatment level of two units per capita.

2. Why is the percentage of use of plasma-derived clotting factors as opposed to recombinant therapies different in Germany than in other countries?

There is indeed a difference to other countries in the percentage of use of plasma-derived and recombinant clotting factor concentrates which is about 50 percent plasma-derived and 50 percent recombinant therapy. In prophylactic treatment the relation is about two third use of recombinant and one third of

plasma-derived factor use. The difference of the overall numbers is probably due to the more common immunotolerance treatment in Germany. Many hemophilia treatment centers prefer plasma-derived clotting factors for immunotolerance treatment because of the von Willebrand Factor (vWF) content of these products.

3. Health technology assessment (HTA) has a growing influence on decision-making processes. Would an HTA on hemophilia treatment support the current treatment regime in Germany, or would you expect that the outcome would have a negative impact?

In the 10 years between the first (1999) and the second (2009) Wildbad Kreuth Initiative, a tremendous number of new publications, new trends in treatment patterns, and a growing focus on economic issues have changed the environment. HTAs have an increasing impact on decisionmakers, particularly when HTAs become a legal requirement. So far the German Institute for Quality and Efficiency in Health Care (IQWiG) has not yet considered plasma protein therapies, but it can be expected that more and more people will look into the direction of these therapies. The focus will be less on the well accepted need for prophylaxis in children (Evidence level 1a), but on prophylaxis and immunotolerance treatment in adults. The German Guidelines on Hemotherapy as well as the conclusions of the 2009 Wildbad Kreuth initiative recommend considering each patient individually and adapting the therapy to the specific needs of the patient. Opinion leaders have divergent views on

the best treatment regime and, therefore, a general recommendation for treatment is not available. Adults who need prophylactic treatment need to be identified, for example those who are under physical exertion or are in specific stress situations.

4. What would you answer to a person who states that on demand treatment of hemophilia patients would be sufficient?

The answer to this question is definitely no. These times are long gone. The earlier treatment is started the better, particularly in children. There are new treatment protocols starting with low doses. On demand treatment in adults could be envisaged, as I said before, depending on the situation of the individual patient. It has to be kept in mind that there is still limited knowledge on the treatment pattern or what dosage should be used.

5. In §116b SGB V, a German regulation and the associated guideline it is stipulated that a physician treating hemophilia patients should be qualified as a hemostasiological experienced physician. Do you believe that all physicians treating hemophilia patients have this qualification?

Hemophilia treatment should only be performed by Experts in Hemostasis, which is an additional qualification a doctor can acquire in specific courses in Hemophilia academies that are available Europe-wide. Sweden is leading in providing the specific expertise. The courses provide a basic education for best practice in hemophilia care. I am proud of the fact that the term hemostasis was first used in 1953 by my former mentor, Prof. Marx, who had founded the German Society for Thrombosis and

Hemostasis (Former DAB) in 1956. At the same time, he founded the German Hemophilia Society (DHG), which is thus the oldest hemophilia society worldwide. Since then hemostasis has become a distinct training unit in medical education.

6. §116b SGB V supports the treatment of hemophilia patients in treatment centers, however, more and more treaters can be found in private practices. Should the government reinforce treatment in centers to ensure high quality treatment?

The 1999 Wildbad Kreuth initiative has clearly requested that comprehensive care centers (CCC), with at least 40 regular patients with severe hemophilia, primarily treat hemophila. This request was reiterated in the 2009 initiative. Also the Expert Report of 2001 issued by the Expert Committee of the German government stipulated that otherwise financial problems might play a role and the quality of treatment could be impaired. In conclusion, treatment in hemophilia care centers is essential. Treatment in private practices outside these care centers is possible, but should be performed only in cooperation with the CCC center. The government is responsible for ensuring appropriate treatment and the payers should only reimburse those treatments at the care center or under its supervision.

In the recommendations of the 2009 Wildbad Kreuth Initiative, home treatment with coagulation factor concentrate should be encouraged in patients with severe hemophilia, who are under the supervision of their care center.


7. The second Wildbad Kreuth initiative was highly appreciated by participants in that it provided a forum to discuss issues related to blood and plasma protein therapies on a European level and to agree on consensus recommendations on state-of-the art treatment. Would you recommend holding this conference on a bian-

nual basis to provide governments and decision-makers with science based support for their decisionmaking processes?

The initiative should certainly be repeated in a regular interval, but maybe not every other year. It is extremely important that the event is taking place under the auspices of the European Directorate for the Quality of Medicines (EDQM) with contribution of the national competent authorities, such as the Paul Ehrlich Institute. It is undoubted that there is a need for an ongoing exchange between stakeholders to further optimize the use of blood components and plasma protein therapies. A survey among the participants of the 2009 event showed that the majority, but not all participants use international and/or national guidelines in daily practice and consider them useful. In some countries, the health care system does not collect quantitative information, such as national registries, about the use of these products. The recommendations of the initiative clearly identified the need to establish and maintain registers of patients with hemophilia and related disorders including information on the outcome of treatment in each country. Other priorities were the establishment



of a network of CCCs in each country providing treatment to at least 40 patients each. Gathering pharmacovigilance information and harmonization of guidelines including advice on treatment patterns are also highly recommended.


Finally, it is recommended that patients with rare bleeding disorders should be treated with specific coagulation factor concentrates. The development of orphan drugs for the treatment of such patients should be encouraged. 

*ILKA VON HOEGEN is
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Expert Recommendations for

BETTER OF PID



This exciting new development is the latest of a series of efforts linked to the European Parliament to increase the profile of this rare condition, for which only one-in-three patients, it is estimated, is correctly diagnosed and receives proper care.

THE EUROPEAN INSTITUTIONS

and European Union (EU) Member States have greatly strengthened their efforts in recent years to increase support for patients with rare diseases across Europe. Member of the European Parliament (MEP) Mr. Jorgo Chatzimarkakis has for several years been raising the profile of primary immunodeficiency diseases (PID) and other rare diseases. In 2009, he called together an Expert Group for Primary Immunodeficiency Diseases comprising physicians, researchers and patients, and it quickly became the driving force behind the *Expert Recommendations for Better Management of Primary Immunodeficiency*. This working document emphasizes the need to take action at both the European and Member State level to raise awareness and increase the diagnosis rate of PID and to improve access to treatment.

MANAGEMENT IN EUROPE

BY LAURA SAVINI

This exciting new development is the latest of a series of efforts linked to the European Parliament to increase the profile of this rare condition, for which only one-in-three patients, it is estimated, is correctly diagnosed and receives proper care. These efforts began in 2004 with a workshop on PID organized by the European Parliament's Scientific Technological Options Assessment Panel (STOA), at which Professor Ann Gurdulf, of Stockholm's Karolinska Hospital, presented the results of a study conducted on PID, Quality of Life and Health Service Costs. Professor Gurdulf's study clearly demonstrated that immunoglobulin (Ig) therapy was a cost-effective treatment option for patients with PID.

In 2006, the International Patient Organization for Primary Immunodeficiency (IPOPI) with the help of funding from the European Commission hosted a consensus conference at the Paul-Ehrlich-Institut in Langen, Germany. This conference brought patients, physicians, academics, nurses and the industry together for the first time to identify and develop public health strategies for PID. The conference concluded with a set of recommendations and a report addressed to the EU Member States. It provided concrete, straightforward suggestions on how to raise awareness for PID, combat PID under-diagnosis and improve access to treatment. These documents also stressed the divergences in PID diagnosis and disease management standards across Europe.

In 2008, MEP Dr. Miroslav Mikolasik organized two working lunches at the European Parliament, one on Plasma Proteins in the Treatment of Rare Diseases



Jorgo Chatzimarkakis
Member of the
European Parliament

and another on How to Improve Care for Plasma Related Rare Diseases, co-hosted by Mr. Chatzimarkakis. These events introduced several MEPs and some European officials to the specificities of plasma protein therapies and plasma-related rare diseases. Patient association representa-

tives and physicians highlighted the most critical issues faced by patients, such as poor access to therapy and cost-benefit assessments, inequalities in diagnosis and therapies, and a lack of awareness and education. Patients and physicians alike proposed a number

EXPERT GROUP

Chair of the European PID Expert Group

Jorgo Chatzimarkakis

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of Member States measures to improve patients' conditions.

Then, in 2009, the Council of the European Union adopted recommendations on rare diseases, which Mr. Chatzimarkakis felt were an encouraging step forward. However, he recognized that they did not adequately take into account the specialist steps needed to tackle specific conditions present within the broad range of rare diseases. Hence, in the same year he established the PID Expert Group to align scientific and clinical knowledge with targeted political action. This culminated with a broad set of recommendations to be used for political action at the European level. This Recommendations Paper describes in clear and accessible language both PID itself and the quality of life of patients affected by it. It provides a background on PID awareness actions implemented across Europe, and gives concrete recommendations on how to increase diagnosis rates and improve care for PID patients. Finally, it looks at the cost-effectiveness of Ig treatment for PID patients, making reference to a Jeffrey Modell Foundation study, which estimates that each undiagnosed patient costs the United States Health Care Services \$102,736 USD versus \$22,696 USD for each diagnosed patient. These recommendations urge governments not to take a short-sighted approach to healthcare spending, but to make a social investment in giving patients with PID the opportunity to live a healthy life and make an active contribution to society.

The Recommendations Paper was officially launched at an event held at the European Parliament in March 2010, where members of the PID Expert Group presented various sections of the document to MEPs and European Officials. The document has now been printed and is currently being distributed to a variety of recipients at both the European and Member State level.

The PID Expert Group's next step is to tailor these recommendations to the specific needs of each Member State, by both translating the document into local languages and adapting the recommendations to focus on one or two main priorities for a specific country. The first target country will be Germany, starting at the end of 2010.

These are very encouraging developments for PID patients across Europe, which clearly warrant further monitoring over the coming months. To conclude with Mr. Chatzimarkakis' words, "this Recommendations Paper is an excellent start that I hope will lead to a better management of care for PID at the EU and Member State level [...] this paper is a perfect example of how all voices can and should be heard in providing guidance for policy making." 

LAURA SAVINI is PPTA's National Affairs Assistant, Europe

INSIDE PPTA

PPTA NEWS FROM
AROUND THE GLOBE

PPTA HEALTH REFORM WEBINAR SERIES ►

The U.S. State Affairs staff and members of the U.S. State Affairs Steering Committee have collaborated to produce a series of webinars on how the health reform law will affect people with rare, chronic diseases that require plasma protein therapies. The first webinar was held to great success on August 19 and featured changes to private insurance and the implementation and use of high risk pools. The free webinar reached capacity of 125 consumers; however a recording of the program is available for 30 days. Future topics include medical loss ratios, state exchanges and individual mandates. The next webinar is tentatively scheduled for September 23.



GLOSSARY OF TERMS

AAT	Alpha-1 Antitrypsin Deficiency	IQWIG	German Institute for Quality and Efficiency in Health Care
CCC	Comprehensive Care Centers	MAA	Marketing Authorization Application
CDER	Center of Drug Research and Evaluation	MEP	Member of the European Parliament
DHG	German Hemophilia Society	NORD	National Organization for Rare Disorders
EDQM	European Directorate for the Quality of Medicines	PDUFA	Prescription Drug User Fee Act
EMA	European Medicines Agency	PID	Primary Immunodeficiency Disease
EU	European Union	RCT	Randomized Clinical Trial
FDA	U.S. Food and Drug Administration	SHI	Statutory Health Insurance
HTA	Health Technology Application	STOA	Scientific Technological Options Assessment Panel
IPOPI	International Patient Organization for Primary Immunodeficiency	WFH	World Federation of Hemophilia



◀ NEW VIDEOS PROMOTE PLASMA DONATION EXPERIENCE

PPTA is developing videos as part of the Source Industry Image and Credibility Campaign that will be used to “demystify” the plasma donation process. The videos will show viewers the step-by-step process of donating plasma in the U.S. and Europe. Beginning with the arrival of a donor in a plasma center, the video will show the various steps that an individual must go through in order to become a plasma donor. From the greeting of the donor when they enter a plasma collection center, to filling out the donor history questionnaire to the physical examination and collection process, the videos will inform and educate individuals interested in donating plasma about what they can expect. The videos ultimately will be available on PPTA’s global website, as well as both the German (dieplasmaspende.de/at—in development) and English language versions of donatingplasma.org. In addition, there will be a 1:30 minute version and an 8-10 minute version of each of the videos, which will give viewers the opportunity to learn a little bit, or a lot about the plasma donation process.

PHARMA TAX MAJOR REVENUE RAISER FOR HEALTH REFORM ▼

In order to help fund health care reform, Congress and the Administration implemented a fee on drug manufacturers and several other industries that they perceived as “benefitting” from the new laws. Beginning in 2011, drug manufacturers must pay an annual fee, which is to be treated as an excise tax, based on their sales volume of branded prescription drugs sold into most government channels. In calculating the market share to determine the fee, manufacturers are to exclude sales of any branded drugs that qualified for the Orphan Drug Act tax credit. Orphan drugs that later become “blockbuster drugs” do not qualify for the exclusion. The annual revenue raised by the fee—initially \$2.5 billion, escalating each year to 2018, when it reaches \$4.1 billion, before reverting to \$2.8 billion in 2019—will be used to help fund the trust fund used to pay for items and services covered under Medicare Part B.

PPTA believes the exclusion for orphan drugs is too narrow to the detriment of patient access, because of the difficulties many plasma protein therapies have in obtaining the orphan tax credit. In crafting such a narrow carve out, Congress intended to limit “me-too” drug products from benefitting from the efforts of the

therapeutic class’ innovator, since a subsequent brand in the same therapeutic class approved for the same rare disease or condition must demonstrate it is “clinically superior” to the original brand or makes “a major contribution to patient care,” in order to receive “orphan designation” by the U.S. Food and Drug Administration (FDA). “Orphan designation” is required for the tax credit. Additionally, some plasma protein therapies, even brands that are the only brand in a particular therapeutic class, cannot qualify for the orphan drug tax credit because they were initially developed abroad.

Subsequent plasma protein therapies entering a particular

therapeutic class are unique drug products, rather than “me-too” drug products, and have only received FDA approval to treat rare diseases and conditions. Therefore PPTA is advocating that Congress amend the annual fee’s orphan exclusion to also protect drugs that treat only rare diseases or conditions. Over the last three decades, the federal government has gone to significant lengths to facilitate the availability of treatments for rare diseases or conditions. This exclusion language must be modified to continue this vital policy goal. ◀



STAFF

SYBILLE BECK

MY NAME IS SYBILLE BECK. I have been working at PPTA since April 2007. I am PPTA's Senior Manager, Source Europe. At PPTA I have three responsibilities: representing the interests of the European plasma collector's – as my title indicates - within the association and towards national and European organizations. The European plasma collection centers are mainly located in Germany and Austria and so it appears to be helpful to be German, which I am, actually even more helpful to come from the South of Germany which has usually a close connection to its Austrian neighbors. I am also responsible for PPTA's relatively new entity "PPTA Deutschland" and, hence, for all issues concerning the German plasma fractionators. This part of my job covers such issues as political outreach and reimbursement to working with German patient organizations. Another central piece of my work is the European project FIND-ID. With this initiative we strive to increase awareness for primary immunodeficiencies and to build a network between general practitioners and specialized treatment centers for an earlier diagnosis of this disease and adequate treatment.

Tell us about your background.

I was born in Munich, where I went to school and to university. Several years ago I decided to move to Berlin, which is still my elected German hometown. Before Berlin I worked for a headhunting agency, where my main responsibility consisted of evaluating resumes. In Berlin I joined the copyright and business affairs depart-

ment of Sony/ATV Music Publishing. Over time my major projects developed to be all and any issues with regard to music broadcasted live, on radio, online and most importantly on television as compared to the other part of the business which is record sales. The years in the beginning of this century have been tumultuous for the music industry and it has been interesting to witness how a whole industry redefined itself. After five years in this very demanding and intriguing environment, I decided to finally finish my degree in law and philosophy and gain at the same time work experience in a European setting. Since I also have a weak point for Art Deco, it was obvious: Brussels it is! And this is how I came to work at PPTA.

What is your proudest professional achievement at PPTA?

There are many moments when I am proud of what I do at PPTA, but one of the highlights for me was this year's Satellite Symposium at the 31st Congress of the International Society for Blood Transfusion, which was very successful and the first PPTA event in that kind of setting.

What is most rewarding about working in this industry?

In a nutshell: The people! It is naturally rewarding to work in an industry which has a direct impact on helping other people, i.e. the patients. I also work with patient organizations on a national and European level and to see the dedication and commitment of these people is very gratifying. But I would like to take the opportunity to thank the people in the industry

I work with: my talented and wonderful colleagues in all our offices and first and foremost the individuals of the member companies I work with. Regardless of the fact, that I have neither a scientific background nor any industry experience, they never let me down, but continue to be most generous with their time, their advice and their support. A big thank you! I do not take that for granted. 🙏



EVENTS

UPCOMING CONFERENCES & SYMPOSIUMS

2010

September 22 – 23	VI Baltic Transfusion Practice Conference <i>Riga, Latvia</i>
October 6 – 9	XIVth Meeting of the European Society for Immunodeficiency (ESID) <i>Istanbul, Turkey</i>
October 9 – 12	AABB Annual Meeting <i>Baltimore, Maryland, United States</i>
October 10	Source Business Forum <i>Baltimore, Maryland, United States</i> PPTA Members Only
October 17 – 20	Annual Scientific Meeting of the Haematology Society of Australia and New Zealand, the Australian and New Zealand Society of Blood Transfusion and the Australasian Society of Thrombosis and Haemostasis <i>Auckland, New Zealand</i>
October 21 – 24	XI European Symposium on Platelet and Granulocyte Immunobiology <i>Beaune, France</i>
October 22 – 24	European Haemophilia Consortium Annual General Meeting <i>Lisbon, Portugal</i>
November 24 – 26	6 th Red Cross and Red Crescent Symposium <i>Tokyo, Japan</i>

2011

February 2 – 4	4 th Annual Congress of the European Association for Haemophilia and Allied Disorders <i>Geneva, Switzerland</i>
March 13 – 17	6th World Congress on Paediatric Critical Care <i>Sydney, Australia</i>
March 15 – 16	International Plasma Protein Congress <i>Lisbon, Portugal</i>
March 22 – 25	31th Symposium on Intensive Care and Emergency Medicine <i>Brussels, Belgium</i>
June 9 – 12	16 th Congress of the European Hematology Association <i>London, United Kingdom</i>
June 14–15	Plasma Protein Forum <i>Reston, Virginia, United States</i>
June 18 – 22	XXIst International Congress of the International Society of Blood Transfusion (ISBT) <i>Lisbon, Portugal</i>
October 22 – 25	AABB Annual Meeting <i>San Diego, United States</i>



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