

INSIDE PPTA

PPTA NEWS FROM
AROUND THE GLOBE

Save the Date!

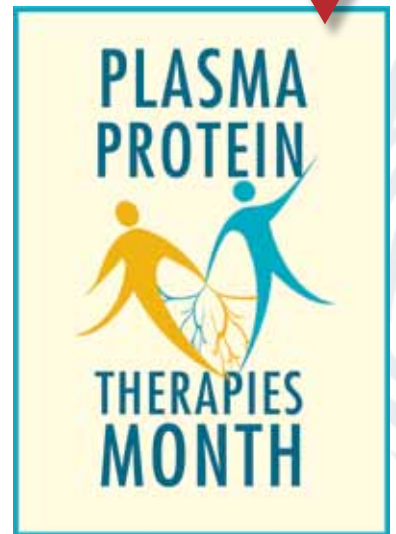
PPTA CELEBRATES 20TH ANNIVERSARY WITH EVENT IN WASHINGTON, D.C.



PPTA is planning a celebration of the Association's 20th anniversary recognizing decades of saving and improving lives on Wednesday, June 20, 2012 at The Pavilion at the Ronald Reagan Building in Washington, D.C. During the gala, a silent auction, dinner and special recognitions will be held. The event venue will be adjacent to the Plasma Protein Forum meeting site at the J.W. Marriott Hotel and will be held the evening prior to the Forum. Reservations, program sponsorships and more information will be available in early 2012 or contact PPTA to learn how you can make the event spectacular!

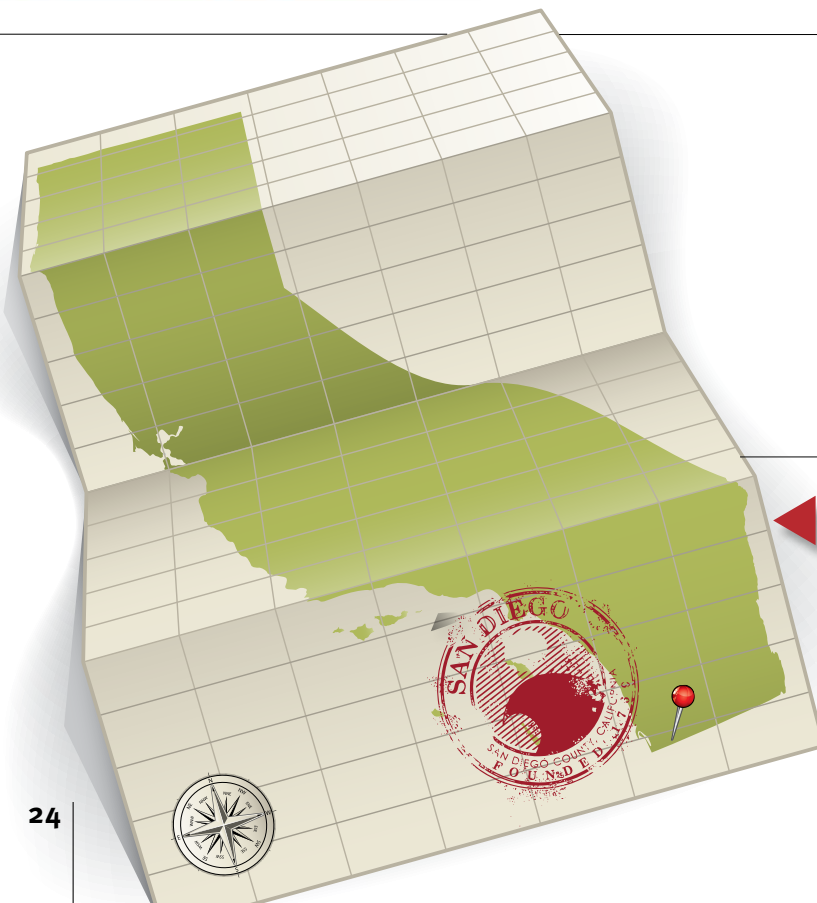
MEMBERS PARTICIPATE IN PLASMA PROTEIN THERAPIES MONTH EVENTS IN CALIFORNIA AND FLORIDA

Two PPTA members hosted activities at plasma collection centers in California and Florida as part of the Plasma Protein Therapies Month recognition, which raised awareness for the importance of plasma donation and for the therapies that treat rare, chronic conditions. Advanced BioSciences held an event at its Reseda, California center that recognized donors and patients, and the contribution the center makes to the community and lifesaving therapies. Invited guests included local officials and the event was covered by community newspapers. Grifols hosted three open houses at its centers in Atlantic Beach, Pensacola, and Tallahassee that included ribbon cutting ceremonies, facility tours and the opportunity to meet with patients and donors. Plasma Protein Therapies Month is a joint initiative of the State Affairs Steering Committee and the Source Industry Image and Credibility Campaign.



PPTA HOSTS SUCCESSFUL SOURCE BUSINESS FORUM IN SAN DIEGO, CALIFORNIA

PPTA held a well-attended and highly successful 2011 Source Business Forum in conjunction with the annual meeting of AABB, in San Diego, California, on Sunday, October 23, 2011. See page 6 for the Forum Report.



PPTA COMMENTS ON PDUFA REAUTHORIZATION

In response to the reopening of the comment period for the **Prescription Drug User Fee Act (PDUFA)**, PPTA submitted comments to the **U.S. Food and Drug Administration (FDA)** on October 28, 2011, that reiterated the Association's April 2010 public meeting testimony and its May 2010 written comments. PPTA also urged FDA to allow products to be orphan designated without a showing of clinical superiority in circumstances where the first to market product's orphan exclusivity has expired or was never granted. Eliminating the clinical superiority requirement in these limited circumstances would provide an opportunity for orphan designation for plasma protein therapies, which, as a result of the unique pharmacokinetics and pharmacodynamics exhibited per patient, are often challenged to show clinical superiority on a population-wide basis, and thus face a high, frequently insurmountable, barrier to orphan designation.

► **European Parliament program** PPTA European Health Policy Steering Committee (HIPSC) met with several Members of the



European Parliament and health attachés from the Permanent Representation of Germany, Italy and Spain. The objective of these meetings was to establish a first contact with key stakeholders for the upcoming revision of several health-related European legislations. In fact, legislations such as the Clinical Trials Directive, the Pharmacovigilance Directive and the Transparency Directive, setting the framework for the reimbursement of pharmaceuticals in Europe, will be revised in the next 12 months by both the European Parliament and Member States.

► PPTA attended the 62nd meeting of the **World Health Organization (WHO)** Expert Committee on Biological Standardization (ECBS). The WHO Blood Regulators Network presented the draft "Assessment Criteria for National Blood Regulatory Systems" which was developed upon request from WHO and the International Conference of Drug Regulatory Authorities (ICDRA). The document aims at the development of an assessment tool to assist capacity building of national regulatory authorities for blood and blood products and to sustain development of the World Health Assembly (WHA) Resolution 63.12 on availability, quality and safety of blood products. The document should aid convergence of blood standards on a global level, however it is not meant as a harmonization tool. During the meeting the ECBS endorsed the proposals to establish the 1st WHO international standard for Hepatitis E Virus (HEV) Ribonucleic Acid (RNA) and to establish the 1st WHO international reference panel for Hepatitis E Virus genotypes. It was noted that in developing countries, HEV is the major cause of acute hepatitis, transmitted by the faecal oral route or contaminated drinking water.

► **PPTA Deutschland**, for the first time since its founding was requested to nominate a representative and a substitute for the National Advisory Committee "Blood". The PPTA Deutschland Board of Directors nominated Dr. Albrecht Gröner, CSL Behring, to be the



main representative and Dr. Matthias Germer, Biotest, to be the substitute. The committee has a long standing history and its votes have influenced the landscape for blood and plasma products and all related aspects, including voluntary and unpaid donations and look back procedures just to name two.

► **PPTA Netherlands met with the Ministry of Health** to share PPTA's view on the recently published ConQuaestor report.



The Ministry confirmed that the ConQuaestor report was the final step to prepare a response from the Dutch Minister of Health to the Parliament. According to the Ministry it is obvious that the current outcomes regarding the position of the local manufacturer needs to be evaluated and are subject of discussion in the Parliament. The impact of the report is not yet clear, though it is clear that changes in the current Law on Blood Supply may be the result of the ConQuaestor report.

► **The FIND-ID network**, supported by PPTA, hosted its first symposium at the 45th Workshop for Ear Nose Throat (ENT) specialists in Mannheim, Germany. 140 ENT experts attended the symposium entitled "Sinusitis - yet again? News about Immunodeficiencies!" The four presentations addressed one or more key symptoms that an ENT expert would frequently see in his daily life and which is a typical hint towards immunodeficiency. The feedback was enthusiastic and many physicians took the opportunity to visit the FIND-ID booth afterwards to get more information about



Primary Immunodeficiencies (PID) and FIND-ID itself. They confirmed what PID experts and industry have been stating for a long time, i.e. that a lot of patients are sent to ENT experts only after general practitioners and pediatricians are at the end of their wisdom with patients who have an above-average number of sinusitis or otitis each year, hence fulfilling one or more of the classical warning signs for PID. Encouraged by this success, FIND-ID will continue to focus on the education of experts in medical areas where traditionally un- or misdiagnosed patients are found.

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PPTA RUNS ADS SUPPORTING LEGISLATION

During the first week in November, PPTA ran online ads on Politico.com and Roll Call.com in support of House and Senate legislation that would modify the orphan drug exclusion from the annual pharmaceutical fee

enacted as part of the Affordable Care Act last year. H.R. 2672 and S.1423 would exempt all therapies that are solely indicated by the Food and Drug Administration to treat one or more rare disease or condition. The ads urged members to cosponsor the bipartisan legislation to protect access to rare disease therapies.

PROTECT ACCESS TO RARE DISEASE THERAPIES

Congress must ensure that the needs of rare disease patient populations continue to be met and that incentives for orphan drug development continue to support bringing life-saving medicines to patients.

The Plasma Protein Therapeutics Association and its members urge you to co-sponsor the bipartisan H.R. 2672 and S. 1423, the "Preserving Access to Orphan Drugs Act of 2011"

Learn more at www.pptaglobal.org/rarediseasetherapies



GLOSSARY OF TERMS

ABRA American Blood Resources Association

ALBIOS Albumin Italian Outcome Sepsis Study

CBER Center for Biologics Evaluation and Research

CDRA Conference of Drug Regulatory Authorities

EMA European Medicines Agency

ENT Ear, Nose and Throat

FDA U.S. Food and Drug Administration

GFC Global Financial Crisis

HEV Hepatitis E Virus

HRSA Health Resources and Services Administration

HTA Health Technology Assessment

MBLD Mannan Binding Lectin Deficiency

MRSA Methicillin-resistant Staphylococcus aureus

PDUFA Prescription Drug User Fee Act

PID Primary Immune Deficiency

QALY Quality-adjusted life year

RNA Ribonucleic Acid

SAFE Saline versus Albumin Fluid Evaluation

SGR Sustainable growth rate

SPAC State Patient Access Coalition

WHO World Health Organization