PPTA
Plasma Protein Therapeutics Association

Statement on Proposed Recommendations for the Reauthorization of the Prescription Drug User Fee Act

The Plasma Protein Therapeutics Association (PPTA) is pleased to provide these comments on the Food and Drug Administration's (FDA) public meeting regarding the proposed recommendations for the reauthorization of the Prescription Drug User Fee Act (PDUFA) for the process of human drug application review for fiscal years 2008 - 2012. PPTA is the international trade association and standards setting organization for the world's major producers of plasma-derived and recombinant analog therapies. Our members provide 60 percent of the world's needs for Source Plasma and protein therapies. These include clotting therapies for individuals with bleeding disorders, immunoglobulins to treat a complex of diseases in persons with immune deficiencies, therapies for individuals who have alpha-1 anti-trypsin deficiency which typically manifests as adult onset emphysema and substantially limits life expectancy, and albumin which is used in emergency room settings to treat individuals with shock, trauma, burns, and other conditions. PPTA members are committed to assuring the safety and availability of these medically needed life-sustaining therapies.

PPTA appreciates the opportunity to comment on FDA's proposed recommendations. PPTA presented at the previous PDUFA public meeting held in November 2005, submitted written comments to the docket in December 2005, and met with FDA representatives in May 2006 to discuss the reauthorization of PDUFA. PPTA member companies, including those not subject to user fees, are regulated by the Center for Biologics and Evaluation and Research (CBER). The products produced by our member companies are reviewed by the Office of Blood Research and Review. In these previous communications, PPTA stated that overall the companies are pleased with CBER's performance regarding PDUFA. Without the development of this user fee program, many life-saving therapies would not have come to fruition. Because of the success of the user fee program, PPTA supports reauthorization of PDUFA. At the same time, PPTA is interested in containing costs of the program while ensuring that FDA's important programs, whether user fee funded or not, remain viable.

To improve this essential program, PPTA communicated initiatives that should be implemented. We are pleased to see that some of the proposed recommendations included in PDUFA IV are priorities PPTA presented. For instance: (1) the withdrawal of programs, such as the Continuous Market Application (CMA) pilot programs, if the overall benefits of the programs did not justify their costs to FDA; (2) revising the timeline during the drug review process to include earlier discussion of labeling and negotiation of post-market (Phase IV) commitments to alleviate some of the detrimental last minute decisions that can occur right before a drug is approved; and (3) improvements to the IT infrastructure that would help improve transparency and predictability of the human drug review process. Additionally, PPTA is supportive of fund allocation for the development of guidance documents, particularly for clinical trial design expectations. PPTA member companies produce therapies that are often

147 Old Solomons Island Road · Suite 100 · Annapolis, MD 21401 USA
tel: 202.789.3100 · 410.263.8296 · fax: 410.263.2298 · e-mail: ppta@pptaglobal.org · www.pptaglobal.org
PPTA Offices in Washington · Annapolis · Brussels · Tokyo

indicated for small patient populations. Allocation of PDUFA funds to develop guidance under "Good Guidance Practices" allows industry input into licensing criteria standards, including clinical trial design, rather than having those standards developed on an ad hoc basis during the application review process.

Despite the positive proposed recommendations included in PDUFA IV, PPTA remains concerned about the increase in user fees towards post-market surveillance and the possible expansion beyond the periapproval period included under PDUFA III. PPTA previously advocated that if PDUFA IV included an expanded post-market surveillance program, the program should be developed on an interactive basis between FDA, industry, consumers, and other interested parties. PPTA is pleased that FDA's recommendations include plans for transparency in the process and opportunities for input via participating in public workshops and commenting on published documents. PPTA's unease with the expansion of post-market surveillance programs supported by user fees is that it presents certain conflicts that may be perceived in a public health drug safety monitoring program that is underwritten by industry. PPTA members support the use of PDUFA user fees for post-market studies and monitoring during the periapproval period as included in PDUFA III. However, FDA should receive public funds through Congressional appropriations to undertake a comprehensive effort to modernize and transform its post-market safety system.

Finally, PPTA members remain alarmed by the erosion of FDA's, and particularly CBER's, funding outside of the user fee programs. CBER operates uniquely under both PDUFA and the Medical Device User Fee Program (MDUFMA). In addition, CBER maintains a significant number of non-user fee programs. Therefore, it is of great importance to our member companies that user fees, in both programs, be used appropriately (including tracking and accountability), while non-user fee programs continue to receive adequate funds. CBER cannot perform all essential regulatory functions without sufficient funding. CBER plays an important role in the lives of many individuals who rely on plasma protein therapies manufactured by PPTA members. CBER must be provided the necessary funding to administer programs appropriately to assure the availability of these life-saving therapies to those who desperately need them.

In summary, PPTA thanks FDA for holding this public meeting and allowing us the opportunity to speak on behalf of members. PPTA member companies have supported PDUFA since its inception. The open process during PDUFA IV gave PPTA and its member companies a voice in shaping the FDA recommendations that will directly affect our industry. We believe the inclusion of all affected industry, as well as patients and other stakeholders, is vital to the user fee program and commend FDA for this open process. Should you have any questions regarding these comments or would like additional information, please contact PPTA.