

May 12, 2010

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Dockets Management Branch, HFA-305  
Food and Drug Administration  
5630 Fishers Lane, Room 1061  
Rockville, MD 20852

**VIA WEB**

**SUBJECT:** Prescription Drug User Fee Act (PDUFA); Public Meeting  
[Docket No. 2010-N-0128]

Dear Sir or Madam:

The Plasma Protein Therapeutics Association (PPTA) would like to thank the Food and Drug Administration (FDA) for the opportunity to participate in the Prescription Drug User Fee Act (PDUFA) V Public Meeting on April 12 and is pleased to provide these written comments. PPTA is the international trade association and standards-setting organization for the world's major producers of plasma-derived and recombinant analog therapies, collectively referred to as plasma protein therapies. PPTA represent 97 percent of the Source Plasma collection centers and eight manufacturers of plasma protein therapies in the U.S. Plasma protein therapies are used in the treatment of a number of rare diseases. These diseases are often genetic, chronic, life threatening conditions that require patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives. These therapies 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 participated in the PDUFA IV reauthorization process and appreciated the agency's effort to reach out to stakeholders who had not previously been included in PDUFA reauthorizations. The openness and transparency of the PDUFA IV process provided PPTA a voice to help shape PDUFA IV performance goals that directly affect our industry and ultimately the patients we serve. PPTA believes the inclusion of all affected industry, as well as patients and stakeholders, is vital to the user fee program and commends FDA for continuing this during the PDUFA V reauthorization process.

Plasma protein therapies are regulated by the Center for Biologics Evaluation and Research (CBER) and reviewed within the Office of Blood Research and Review (OBRR). PPTA understands that PPTA members are only a small portion of the pharmaceutical industry that pay user fees but these companies play an essential role

in the patient community they serve. PPTA and its members are committed to assuring the safety and availability of these medically needed life-sustaining therapies.

Overall, PPTA members are pleased with CBER's performance under PDUFA IV. CBER consistently has met PDUFA IV timeframes. PPTA members support the original intent of PDUFA, which was to provide additional resources to the FDA to establish a better managed human drug review process. PPTA recognizes that 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 in 2012.

Despite the strengths of the PDUFA user fee program, PPTA members would like to express their concerns regarding a few areas under PDUFA IV. First, the timeliness of data requests during the review process. Data requests that occur at the end of the review process can be costly and difficult to manage. Often, these requests are poorly coordinated (i.e., several requests for the same data that have already been submitted) and occur right before a FDA action date. Also, there are often requests for significant labeling changes at the end of a review. PPTA believes better coordination and earlier communication of requests to the sponsor during the review process is needed. PPTA supported previous efforts by CBER to utilize mid-cycle reviews under Good Review Management Principles (GRMPs). However, due to other statutory PDUFA IV obligations it does not appear that mid-cycle reviews are being used consistently. Use of mid-cycle reviews would allow FDA to communicate expectations for the remainder of a review process and provide a sponsor the opportunity to clarify the scope of any requests that have been made. The consistent use of mid-cycle reviews would allow for better coordination and communication of data requests and FDA expectations providing a more predictable and transparent review process as intended by PDUFA.

Another area of concern is the use of user fees to fund post market surveillance programs. PPTA recognizes that safety needs to be an imperative part of the FDA review process. PPTA appreciates that FDA spends half of its effort and resources on drug safety activities. However, all of these activities cannot and should not be funded with PDUFA user fees. PPTA supported the use of user fees for post market surveillance programs during the peri-approval phase under PDUFA III but did not support the expansion of post market surveillance programs with PDUFA fees under PDUFA IV. PPTA believes that these programs should be funded with congressional appropriations.

PDUFA user fees should be considered a supplement to the FDA budget. As FDA moves forward with the PDUFA V reauthorization process, PPTA would like to stress the importance of containing costs of the PDUFA program. Current fees are significant. PPTA would like FDA to understand that the continued expansion of the PDUFA program will continue to drive up the fees to the detriment of companies that develop therapies for small, rare patient populations. For that reason, PPTA does not believe

there should be an expansion of any PDUFA programs. PPTA knows that there have been considerable cost increases for the agency and PPTA will continue to encourage Congress to provide FDA with adequate appropriated funding to ensure that both user fee and non-user fee programs remain viable.

Again, PPTA appreciates the opportunity to comment on the PDUFA IV program. PPTA member companies support PDUFA and look forward to working with FDA during the PDUFA V reauthorization process. Should you have any questions regarding these comments or would like additional information, please contact PPTA.

Respectfully submitted,



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