Plasma Protein Therapeutics Association

November 1, 2012
Reference No.: FDAA12019 **VIA WEB**

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

SUBJECT: Prescription Drug User Fee Act Patient-Focused Drug Development; Public Meeting and Request for Comments [Docket No. FDA-2012-N-0967]

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 public comments related to FDA's patient-focused drug development initiative 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. 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 applauds FDA's listing of disease areas as potential candidates for the focus of public input. PPTA is pleased that the list includes the rare diseases for which PPTA member companies provide life-saving and life-supporting therapies. We view these as priority areas for consideration. We have one suggestion that the examples for "neurologic disorders treated with immune globulins" be expanded to include other specific neurologic disorders treated with immune globulins, e.g., Guillain-Barre Syndrome (GBS) and others.

Again, PPTA appreciates the opportunity to comment on this important initiative. PPTA member companies look forward to participating in the applicable public meetings. Should you have any questions regarding these comments or would like additional information, please contact PPTA.

Respectfully submitted,

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