Plasma Protein Therapeutics Association

December 5, 2014
Reference No.: FDAA14010 **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; 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 continued opportunity to participate in the patient-focused drug development initiative and is pleased to provide these comments on the preliminary list of nominated disease areas for consideration in meetings during fiscal years (FYs) 2016-2017.[1]

About PPTA

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.

Background

In 2012 comments, PPTA applauded FDA's listing of disease areas as potential candidates for the focus of one of 20 future public meetings and was pleased that the list included rare diseases for which Association member companies provide life-saving and life-supporting therapies. PPTA views these diseases as priority areas for FDA consideration. In its comments, PPTA suggested that the examples for "neurologic disorders treated with immune globulins" be expanded to include other neurologic disorders treated with immune globulins, e.g. Guillain-Barre Syndrome (GBS) and others. PPTA appreciates that in 2013, FDA selected Alpha-1 antitrypsin deficiency and hemophilia A, hemophilia B, von Willebrand disease, and other heritable bleeding disorders as two of the 16 disease areas to be addressed in meetings during FYs 2013-2015.[2] PPTA attended the September 22, 2014, hemophilia meeting and looks forward to FDA sharing outcomes from the meeting. PPTA also looks forward to FDA scheduling the Alpha-1 meeting.

[1] *See* FR Notice, 79 Fed. Reg. 60857 (October 8, 2014)

[2] *See* FR Notice, 78 Fed. Reg. 21613 (April 11, 2013)

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General comments

As in 2012, PPTA applauds FDA's listing of disease areas as potential candidates for the focus of the remaining public meetings in FYs 2016-2017 and is pleased that the list included rare diseases for which Association member companies provide life-saving and life-supporting therapies. PPTA reiterates that the Association views these diseases as priority areas for FDA consideration.

Specific comments

PPTA notes that chronic inflammatory demyelinating polyneuropathy (CIDP) was deleted as an example of "neurologic disorders treated with immune globulins" and other examples were not added. PPTA interprets this change as an adoption of its 2012 comment, i.e. an expansion of the disease area to include not only CIDP but also other neurologic disorders treated with IG, e.g. GBS and others; however, PPTA asks that FDA confirm this interpretation. PPTA also asks that FDA confirm that the deletions of examples of "primary humoral immune deficiencies" and "thrombotic disorders" similarly expand these disease areas to include all possible examples.

Conclusion

Again, PPTA appreciates the continued opportunity to comment on this important initiative and looks forward to continued participation in 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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Plasma Protein Therapeutics Association