Plasma Protein Therapeutics Association

Date: March 8, 2018
Reference No.: FDAA18001 **VIA EMAIL**

Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, rm. 1061
Rockville, MD 20852

SUBJECT: Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products; Draft Guidance for Industry; Availability; Request for Comments [Docket No. FDA–2017–D–6530]

Dear Sir or Madam:

The Plasma Protein Therapeutics Association (PPTA) thanks FDA for the opportunity to participate in the guidance development process and is pleased to provide these comments on the Agency's draft guidance entitled "Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products" ("Draft Guidance").[1] PPTA understands that the Draft Guidance replaces the Agency's previous guidance for industry "Formal Meetings Between the FDA and Sponsors or Applicants" published May 19, 2009, and the draft guidance for industry "Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products" published March 11, 2015, which have been withdrawn.

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. PPTA members are committed to assuring the safety and availability of these medically needed, life-sustaining therapies.

Comments

III. A Lines 86-88: Inclusion of meetings requested in response to refuse to file (RTF) letters as requests for formal meetings contradicts 21 CFR 314.101(a)(3), which describes such meetings as "an informal conference with the Agency." Additionally, it contradicts guidance for industry *Refuse to File: NDA and BLA Submissions to CDER* (Draft issued December 2017), which also

[1] https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM590547.pdf, accessed February 14, 2018

describes meetings in response to RTF letters as informal conferences: lines 191 – 193 "Within 30 days of the date of the review division's RTF notification, the applicant may request in writing an informal conference with the FDA to discuss whether the FDA should file the application." PPTA requests that FDA review guidance documents to ensure language is harmonized and aligned with the language and intent of the CFR.

VI. B. Line 302: PPTA requests that FDA confirm that it will be providing conferencing information (telephone dial-ins and/or video conference information) as stated in this draft. PPTA member companies have experienced recently that FDA has required the Sponsor to provide these services.

VII. B. Lines 357-359: PPTA requests that FDA confirm that "CBER neither requests nor accepts paper copies (desk copies). . . ." This contradicts recent experiences in which CBER has requested paper desk copies.

VII. C. 15. Lines 425-428: PPTA requests that FDA provide guidance on the level of CMC-related data recommended and to discuss the introduction of new information within the overall application through use of a meeting request, e.g., what is the recommended sequence for filing a CMC IND amendment with detailed reports prior to submitting a meeting request to discuss implications/topics arising from these data or following such a meeting?

VIII. Lines 457-463: PPTA applauds FDA for including the intent to provide preliminary responses to the requester prior to the meeting. Companies appreciate receiving such feedback prior to the meeting. Providing this information should facilitate both more productive meetings and potentially could increase the acceptance of these preliminary responses as final, pre-empting the need for a meeting.

References. Related Guidances. Line 614: PPTA requests that FDA review whether two additional guidances could be cross-referenced or superseded by the content of this Draft Guidance. Theses guidance documents are: guidance for industry *End-of-Phase 2A Meetings* (September 2009) and guidance for industry *IND Meetings for Human Drugs and Biologics Chemistry, Manufacturing, and Controls Information* (May 2001).

Conclusion

PPTA appreciates the opportunity to comment on the Draft Guidance and welcomes any questions regarding these comments.

Respectfully submitted,

Mary Gustafson
Vice President, Global Regulatory Policy
Plasma Protein Therapeutics Association