

PPTA Brings Value to Members Through **CRITICAL MEETINGS WITH FDA AND EMA** on Regulatory Issues

BY ALBERT FARRUGIA, ILKA VON HOEGEN, AND MARY GUSTAFSON

PPTA IS COMMITTED TO STRONG REGULATORY OVERSIGHT OF PLASMA PROTEIN THERAPIES.

The world's foremost regulatory agencies in the United States, Europe, Japan and Australia regulate PPTA member companies' therapies provided in those areas.

Therefore, the Association's interaction with these agencies is crucial to the industry.

The Association presents issues of common concern for the industry to these bodies and works to resolve problems and seek reasonable regulatory policies in the interests of patients, industry and agencies.

This work is accomplished in several ways:

1 PPTA has annual liaison meetings with both the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA). These meetings provide an opportunity for both the industry and the Agencies to discuss various topics of concern for the purpose of clarifying issues and seeking a common understanding. Meetings have also been held with the Japanese Ministry of Health Labour and Welfare and the Australian Therapeutic Goods Administration. These annual meetings are generally judged valuable by both the industry and the regulators; views have been exchanged and issues of common interest have advanced towards resolution.

Recent meetings have included the 2010 FDA/PPTA Liaison meeting in October 2010, where the FDA's draft Guidance on Process Validation was discussed. The industry elicited the Agency's agreement that timely release

of the final Guidance was essential to facilitate planning in manufacture, and this was reflected in the final Guidance's publication January 2011. Having the final guidance allows member companies to undertake new projects with increased predictability.

In Europe, the PPTA has engaged recently with the EMA on the proposed Annex on plasma-derived protein manufacture to the European code for Good Manufacturing Practice ("Annex 14"). This important Annex recognizes the unique nature of plasma protein therapies but, as initially proposed, would restrict considerably the capacity of the industry to perform contract manufacture for countries outside the European Union. This would impair access to therapy for patients in countries unable to manufacture plasma protein therapies, particularly emerging countries, an issue of interest to the World Health Organization (WHO) given the commitment of the 63rd World Health Assembly to making plasma for fractionation available for all countries. As a result of productive

discussions between the involved parties, it was decided in October 2010 that the draft Annex should progress without making any statement on import criteria for plasma fractionation programs for third countries. PPTA remains committed to introducing appropriate wording into the Annex 14, and continues its interactions with the European Commission to this end.

2 PPTA presents for the industry in regulatory agencies' meetings and workshops. Depending on the scenario, PPTA may provide a statement, an oral presentation on policy or a combined scientific presentation of data derived from member company studies. Some examples of past meeting participation include EMA workshops on epidemiology and FDA advisory committee meetings addressing concerns about variant Creutzfeldt Jakob disease and West Nile Virus, where the Association's collated data was pivotal in contributing to FDA's decision to exempt

source plasma for manufacture from the need to be screened for this agent.

3 PPTA writes letters to regulators presenting industry comments to proposed regulations and guidance documents and also to address specific regulatory concerns. Providing comment letters to agencies requires monitoring of Agency document releases and coordinating responses with the PPTA regulatory steering committees and task forces. In this context, interaction with PPTA's albumin task force in Europe has elicited from the EMA that the Agency considers that the basic volume expansion indication covers much of the usage relating to indications such as hepatology and severe sepsis. This is considered to be a very positive development by the Association as it widens the

scope of albumin usage considerably. PPTA has also engaged with a number of international regulators to heighten awareness regarding the risks posed by hydroxyethyl starches (HES) and the implications of recent findings of scientific fraud involving these products. This has been followed by the German Physician Association's withdrawal of its current albumin usage guidelines, admitting their lack of credibility as their author has been found to be guilty of scientific fraud. The Association committed to consulting the PPTA in a new iteration.

4 Sometimes PPTA will request an ad hoc meeting with regulators to resolve problems that are either too urgent or too complicated to address in correspondence. Recently, a proposed infectious disease standard for biological source materials was proposed by the

Australian TGA which, if implemented in its original state, would have severely limited access to plasma protein therapies in Australia. The Association's representatives held a meeting with the Administration that led to a considerable modification of the standard and adequate consideration of the special features of plasma for fractionation.

PPTA will continue its collegial and productive interaction with regulators. *"Let us never negotiate out of fear. But let us never fear to negotiate."*

— John F. Kennedy, 1961

ALBERT FARRUGIA is PPTA's Vice President, Global Access, ILKA VON HOEGEN is PPTA Europe's Senior Director, Quality and Safety and MARY GUSTAFSON is PPTA's Vice President, Regulatory Policy



“Let us never negotiate out of fear. But let us never fear to negotiate.”

—John F. Kennedy, 1961