

Stakeholder Report

PPTA held a Stakeholder Meeting on December 16 in Rockville, MD in connection with the two-day meeting of the Advisory Committee on Blood Safety and Availability to discuss federal affairs, communications, and the safety of therapies. After welcoming participants from national consumer organizations, the U.S. Food and Drug Administration, consultants working on behalf of consumer groups and PPTA members, and several industry representatives, Julie Birkofer, PPTA Vice President, North America, previewed the evening's program and expressed the collective desire to hold an interactive meeting where information is exchanged. She also welcomed several newcomers to the group, including Ed Burke from the Factor Foundation of America who described his organization's mission.

PPTA federal affairs staff presented an overview of health care reform and priorities of the 111th Congress including insurance reform, health information technology, chronic disease prevention, comparative effectiveness and follow-on biologics—the last two being particularly hot topics for the plasma protein therapeutics industry and for consumers of these therapies and the subject of robust discussion during the meeting.

The PPTA Director of Federal Affairs explained further the definition of comparative effectiveness as a comparison of clinical outcomes of

different treatments for the same medical condition to determine the most effective treatment option. Discussion continued regarding the three different pieces of legislation that have been introduced (S. 334 (Wyden-Bennett), H.R. 2184 (Allen), S. 3408 (Baucus-Conrad)), the potential and likelihood that data would be used for cost containment measures among federal payers, and the precedent set by the UK model. Additionally, staff presented the industry perspective in terms of risks to the small, fragile patient populations treated with plasma protein therapies, the need to ensure that any measures are patient-centered, and the barrier to physician prescribing power.

Comparative effectiveness was a subject of much debate. It was noted that creating an independent institute to conduct these studies has been a priority for many in the Senate, including Senate Finance Chairman Baucus (D-MT) and President-elect Obama. Because negotiations are ongoing, it was suggested that if the stakeholders wish to affect the lead piece of legislation, they must act quickly. One attendee stressed that none of the bills expressly authorizes the Centers for Medicare and Medicaid Services (CMS) to use the data gleaned at the institute to make coverage decisions and that providing physicians with proper information to make treatment decisions should be the priority. The point that this type of data is being used in several

European Union countries already for coverage determination was raised, and the issue was discussed as a potential caution for the users of plasma protein therapies. The group appeared to agree that there is no opposition to the concept of comparative effectiveness and evidence-based medicine in its most helpful interpretation. There is, however, a fear that this data could ultimately limit patient access.

In conclusion to this portion of the conversation, PPTA will develop a principles document on comparative effectiveness that will be circulated to the stakeholder group for input.

Another heavily discussed topic during the meeting was follow-on biologics. PPTA federal affairs staff explained the term as those biological products that would qualify for an abbreviated pathway for approval. Staff suggested that there could be safety issues for plasma protein therapies because the complexity of manufacturing does not guarantee that they can be copied, thus ensuring equivalency and ultimately interchangeability with the original therapy. Moreover, the lack of a requirement for clinical trials in an abbreviated process could be problematic, especially without a robust post-market surveillance program. PPTA staff shared the position of the U.S. Department of Health and Human Services that the agency does not believe the science currently exists to ensure the safety of follow-on versions of many biologics, including plasma protein therapies. Additionally, PPTA staff explained that while European law allows biosimilars (their term for follow-ons), the European Medicines Agency issued guidance strongly discouraging

submissions of abbreviated applications for plasma-derived therapies or recombinant blood clotting factors.

Political considerations for the legislative prospects were also addressed. Although the Senate Bill, S. 1695, which the Senate Committee on Health, Education, Labor, and Pensions reported out in July 2007, has the most traction, incoming House Committee on Energy and Commerce Chairman Henry Waxman will clearly play a pivotal role in shaping what ultimately passes. PPTA staff reported that Waxman has previously indicated he will work off the Senate bill as the House puts forth legislation. There is, however, also a good chance that his version, H.R. 1082, could be the leading House bill because his former chief of staff will be running the White House legislative affairs operation, and Representative Rahm Emanuel, President-elect Obama's incoming chief of staff, supported the Waxman bill over H.R. 1956 and H.R. 5629, which were the competing House bills.

A stakeholder expressed his support for follow-on biologics legislation because even small discounts on expensive therapies like blood clotting factors are critical for their affordability. That stakeholder also suggested that an abbreviated approval pathway and a designation of interchangeability are two separate issues. Another stakeholder suggested that PPTA's position should reflect the current science and be flexible if there are advances in technology. The need for post-market surveillance was stressed by another stakeholder. PPTA agreed to take a look at its position statement on follow-on

biologics and keep the dialogue open on this subject.

PPTA communications staff presented information on leveraging communications in support of advocacy by introducing a webinar series for the coming year. PPTA would develop the content of the webinars in collaboration with national consumer groups, and they would be deployed live, posted on the PPTA website after the live session, or provided to the consumer organization to use with grassroots members. PPTA will use the content of the fact sheets to deliver information to consumer volunteers about plasma protein therapies, how they are made, plasma collection, safety and standards programs, and their differences with other biologics and chemical pharmaceuticals. It was discussed and agreed that national consumer organizations would participate in the content development and assist with informing their grassroots of the webinar programs and their availability and/or posting the session on their websites. It was further discussed that later in 2009 and only if there was an agreed upon set of talking points on a given issue would there be “an ask” of the webinar participants. It was stressed that this is a collaborative effort and that consumer groups will be engaged in the process. Staff also thanked national consumer groups that are participating in the PPTA advertorial that will appear in Politico newspaper in March.

The PPTA Senior Director of Global Access presented information regarding “Two Decades of Safety Measures” for the plasma protein therapeutics industry providing detailed slides and an explanation of innovations in donor

screening, testing, and viral inactivation and removal over the past 20-plus years. He explained the industry standards programs, the International Quality Plasma Program (IQPP), which applies to source plasma for fractionation and the Quality Standards for Excellence, Assurance and Leadership (QSEAL), which applies to manufacturers. Staff discussed the rise in life expectancy with the advent of therapies, how manufactures have invested research into testing and use model viruses to protect against the emergence of new pathogens, and provided details on how some of the voluntary industry standards, which go beyond and complement regulatory requirements, add to the safety of products. It was noted that industry, government and consumers have worked together to make plasma protein therapies among the safest medicines, that those safety measures are proven against known and unknown viruses, and the need for constant vigilance was stressed.

National Hemophilia Foundation (NHF) CEO Val Bias took a few moments to let stakeholders know that the NHF is reviving the Plasma Users Coalition to provide a “clear patient voice and perspective” to the new Obama administration, and that he plans to engage other groups representing rare coagulation disorders and sickle cell anemia. This group would only comprise consumers and will be welcome to report at future Stakeholder meetings.

In closing, PPTA offered thanks to all of the consumer organization representatives and members who were able to participate in the meeting. Given the complexity of the issues facing

health care and the discussion regarding follow-on biologics and comparative effectiveness it was decided that the group will need to meet again in late January or early February of 2009.

[Update: Please hold the date of February 10, 2009 for the next meeting of the Stakeholders.]

Attachments:

1. Powerpoint Presentations:
Stakeholder Meeting December 16th