November 12, 2010

For Immediate Release

Contact: Kym H. Kilbourne
443-458-4682 (o)
443-995-2102 (c)
kkilbourne@pptaglobal.org

PLASMA PROTEIN THERAPIES NOT SUITABLE FOR BIOSIMILARS

ANNAPOLIS, MD (November 12, 2010)—The current science and experience does not support the safety and efficaciousness of a biosimilar pathway for plasma protein therapies. At a public hearing last week, the Plasma Protein Therapeutics Association (PPTA) urged the United States Food and Drug Administration (FDA) to make patient safety its top priority by adopting a global approach in its evaluation of plasma protein therapies for the biosimilars process. The hearing sought to obtain input from stakeholders prior to FDA's implementation of the Biologics Price Competition and Innovation Act of 2009, which was part of the new health care reform law.

Plasma-derived therapies and their recombinant DNA technology analogs, collectively known as plasma protein therapies, treat extremely rare, chronic and life-threatening diseases and disorders, including alpha-1 proteinase inhibitor deficiency, hemophilia and primary immune deficiency diseases. At the FDA hearing, PPTA called for harmonization with European Medicines Agency (EMA) guidelines with regard to therapeutic class exceptions for plasma protein therapies.

Under new U.S. federal law, FDA is empowered to exclude a specific product or an entire therapeutic class from the biosimilars process based on the current science and experience. The new law, however, expressly prohibits FDA from using that same rationale to exempt recombinant proteins, including blood clotting factors used to treat hemophilia and other bleeding disorders. This recombinant protein provision runs counter to the precedent established by the EMA in its 2005 guideline that exempts certain plasma protein therapies, including recombinant blood clotting factors, from the biosimilar process in the European Union. Specifically, if a manufacturer of a biological product is seeking approval as a biosimilar by referencing a brand of immune globulin or blood clotting factor (either plasma-derived or recombinant), the EMA guidelines prohibit the manufacturer from submitting an abbreviated application, as it must instead satisfy the agency's safety and efficacy requirements for a new product.

Plasma protein therapies are complex, large molecule biological therapies that replace missing or deficient proteins in an individual's blood. Each therapy has a specific, multi-step manufacturing process that defines the product. For plasma protein therapies, no range of structural differences between a proposed biosimilar product and its reference product is consistent with the "highly similar" standard that is required for an application for a biosimilar. Even small differences in manufacturing methods can produce changes in the final product that can interact with patients and are impossible to detect without data from human clinical trials.

Most therapeutic classes of plasma protein therapies include multiple brands, yet none of the brands in any class are interchangeable. Because each brand in a class of plasma protein therapies is a unique therapy that is often part of a lifelong treatment regimen in patients with serious, chronic disorders, there is a significant risk associated with alternating or switching brands. "It is well understood that changing brands of plasma protein therapies in a given therapeutic class puts patients at increased risk for adverse events and other complications

including the body's rejection of the replacement protein through an immune system response," said Mary Gustafson, Vice President, Global Regulatory Policy, PPTA.

The new law provides FDA with the authority to deem a biosimilar as interchangeable with its reference product, meaning it can be substituted for the innovator product without consultation with the prescribing physician or the patient, if it meets a certain threshold. Since existing brands of plasma protein therapies fail to qualify as interchangeable, an innovator plasma protein therapy should never be considered interchangeable with a biosimilar version of the therapy.

PPTA is a global trade association that represents source plasma collectors and manufacturers of plasma protein therapies including blood clotting factors that treat bleeding disorders; immune globulins that bolster or supplant a failed immune system; alpha-1 proteinase inhibitors, which treat genetic chronic obstructive pulmonary disease (COPD); and albumin, which is used in critical care settings to treat severe trauma and burns and during surgery.

While Congress debated the federal health reform legislation, the Association commissioned a white paper to evaluate the impact biosimilars would have on patient care, concluding it would put the health of fragile patient populations at increased risk of developing complications. "The bottom line is that science and precedent do not support a biosimilar approach for plasma protein therapies, and protecting access to safe and effective treatment should remain the first priority," said Julie Birkofer, Senior Vice President, PPTA North America.

###

The Plasma Protein Therapeutics Association (PPTA) represents the world's leading manufacturers of plasma-derived and recombinant biological therapies, collectively known as plasma protein therapies and the collectors of source plasma. These critical therapies are infused or injected by more than 1 million people worldwide to treat a variety of rare, life threatening diseases and serious medical conditions including hemophilia, primary immunodeficiency diseases and alpha-1 antitrypsin deficiency. PPTA members produce in excess of 80 percent of the plasma protein therapies used in the United States today and more than 60 percent worldwide. PPTA is a global trade association that administers international, voluntary standards programs to help ensure the highest quality and safety of plasma protein therapies and the plasma collected to manufacture them. Additionally, PPTA works in partnership with the patient community and consumer advocates to help ensure continued access to lifesaving plasma protein therapies. Visit www.pptaglobal.org.