PPTA Statement on the Rare Bleeding Disorders Workshop

FDA Blood Products Advisory Committee
July 21, 2005

The Plasma Protein Therapeutics Association (PPTA) appreciates actions taken by the FDA and the Department of Health and Human Services in holding a workshop to discuss very rare blood disorders. Many of the patient populations served by PPTA member companies have "orphan" diseases; our member companies therefore have experience in dealing with development of therapies for rare diseases and have secured licensure of a number of products. PPTA member companies hold great enthusiasm for further investigation of the possibilities for producing therapies addressing the conditions.

Workshop participants brought a variety of issues and perspectives to the discussion of rare bleeding disorders. Many critical distinctions were made, ranging from encouragement of non-conventional thinking with respect to clinical trial design, to the delineation of a "normal" orphan population, as opposed to a much smaller patient population, with appellations such as "hyper-orphan" or "super-orphan" signifying the extraordinary rarity of some of the conditions described. In fact, this distinction, when coupled with an understanding of the costs involved in product development, highlights areas of policy improvement that would aid in the development of products for very rare conditions. While some Workshop presenters discussed incentive programs, especially for smaller businesses, PPTA notes that such direct aid may not have a commensurate benefit for a larger organization. A number of mechanisms may be used to support innovation, and PPTA member companies would encourage FDA to study other jurisdictions' that have had a measure of success with licensing hyper-orphan or super-orphan products. These other jurisdictions may have policy regimes that can serve as a benchmark for a regulatory or statutory structure in the US that would further encourage innovative research on very rare disorders.

The FDA has promulgated a number of programs over the past three years that may also supply synergy in developing a paradigm for licensure of therapies. Initiatives such as the Critical Path supply a possible policy direction that can be leveraged into a regulatory arrangement that can foster and sustain development of therapies targeted at the very rare disease. The Critical Path focuses on bridging the gaps between the research-intensive scientific discovery paradigm and the transition toward application of tangible, medicinal product advances. The Critical Path Whitepaper mentions numerous tools for this transition, such as biomarker development, predictive models, and clinical evaluation techniques, and may be an effective blueprint for CBER to use in channeling areas of research and development incentives.

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PPTA and its member companies commend FDA for organizing and holding this important Workshop, which as an important first step in creating a framework addressing the need for therapies treating very rare diseases. With this discussion, an effective conceptual foundation has been constructed upon which many benefits may be built. Many more years of hard work, clear thinking, and enthusiastic discussions may lie ahead, but the results will be well worth the effort.

The Plasma Protein Therapeutics Association (PPTA) is the international trade association and standards-setting organization for the world's major producers of plasma-derived and recombinant analog therapies. Our members provide 60 percent of the world's needs for Source Plasma and protein therapies. These 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.