Plasma Protein Therapeutics Association

December 18, 2012

The Honorable Max Baucus
Chairman
Committee on Finance
United States Senate
219 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Orrin G. Hatch
Ranking Member
Committee on Finance
United States Senate
219 Dirksen Senate Office Building
Washington, D.C. 20510

Re: S. 1423, The Preserving Access to Orphan Drugs Act

Dear Senator Baucus and Senator Hatch,

The Plasma Protein Therapeutics Association (PPTA) would like to recognize and thank you for your leadership and long history of support for orphan drug development and rare disease patients. PPTA urges you to include S. 1423, The Preserving Access to Orphan Drugs Act, in any legislative package to address the fiscal cliff. This critically important legislation clarifies that sales of therapies used solely for the treatment of rare diseases will be excluded from the annual pharmaceutical fee under the Affordable Care Act (ACA). This bi-partisan, budget neutral legislation will help ensure the continued development of treatments for rare disorders.

PPTA is a global trade association that represents human plasma collection centers and manufacturers of plasma protein therapies. The manufacturer membership of PPTA in the United States currently includes Baxter BioScience, Biotest, CSL Behring, Grifols and Kedrion.

Our members' products include medicinal therapies derived from human plasma and their recombinant analogs, and, collectively are known as "plasma protein therapies." PPTA members research, develop, and produce essential therapies to treat patients with life-threatening rare diseases including those suffering from hemophilia and other bleeding disorders, primary immune deficiency diseases, and alpha-1 antitrypsin deficiency, often referred to as genetic emphysema.

Plasma protein therapies derived from human plasma are defined by their complex manufacturing process, which gives rise to significant therapeutic variation between brands and makes them wholly non-interchangeable therapies that are infused or

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injected by patients often for the duration of their lives. Manufacturers of plasma protein therapies invest heavily in the manufacturing process, which includes acquisition of the essential starting material human plasma, for which there is no alternative source. The complicated process of fractionating that plasma into specific therapeutics, combined with the rigorous, step-wise and vigilant process of viral inactivation and removal techniques further defines the uniqueness of this niche industry's products and the challenges manufacturers face to innovate and develop therapies that treat extremely rare diseases.

PPTA supports Congress' leadership to enact policies that are helping to create a robust environment in which manufacturers can investigate bringing products to market that treat rare diseases. However, the narrow definition of an orphan drug used in the ACA for the purposes of determining eligibility for the exclusion from the annual pharmaceutical fee does not encompass all therapies developed exclusively to treat rare diseases and conditions.

The ACA created an annual branded prescription drug fee or "annual pharmaceutical fee" to be paid by all branded pharmaceutical and biologics manufacturers. Consistent with Congress' historic support for rare disease treatments, the provision contains an exclusion for sales of those drugs for which the manufacturer has received an Orphan Drug Act (ODA) tax credit. Unfortunately, the reliance on the ODA tax credit to define eligibility for the exclusion leaves a subset of therapies licensed and approved solely for the treatment of rare conditions subject to the annual pharmaceutical fee. For example:

- The ODA tax credit was not made permanent until June 1, 2007, and there exists a period of 18 months during 2005 and 2006 when it was not authorized, making it impossible for manufactures to have claimed it;

- Additionally, during the first 12 years of the ODA, new market entrants were unlikely to claim the ODA tax credit because manufacturers could not carry unused credits forward or backward. Manufacturers therefore may have elected to take a Research and Development (R &D) tax credit instead and are prevented from claiming both and R&D tax credit and the ODA tax credit for the same qualifying clinical testing expenses;

- In general, foreign testing expenses are not eligible for the ODA tax credit. Foreign clinical trials are common for our global industry that serves rare disease patients around the world; and

- Lastly, given the confines of the regulatory process that governs orphan drug designation, which is a prerequisite to claiming the ODA tax credit, it is extremely difficult for many plasma protein therapies to become orphan drug designated.

We believe that shifting the focus, as S. 1423 does, to products that are approved for marketing solely for rare diseases or conditions, properly aligns the exclusion from the annual fee with the goal of promoting the development of treatments for rare disorders.

Further, basing the exclusion from the annual fee on whether a product took the ODA tax credit disproportionately affects plasma protein therapies. Of the drug and biologic therapies that are exclusively indicated to treat a rare disease or condition but that do not qualify for the orphan drug exclusion from the annual fee, the vast majority are plasma protein therapies. A November 2010 study by health economics and policy consulting firm Dobson DaVanzo, identified 41 drugs and biologics that did not qualify for the orphan drug exclusion from the annual fee despite being solely indicated by the Food and Drug Administration (FDA) to treat one or more rare disease. Thirty-three of those 41 products are plasma protein therapies.

We believe S.1423 is consistent with longstanding Congressional support for policies that encourage the development of treatments for rare diseases. Incentives that help foster the development of orphan drugs have unquestionably been a success, encouraging manufacturers to innovate and market hundreds of therapies used to treat rare diseases and conditions. S. 1423 would help to continue that progress and preserve and promote the development of these vital therapies.

Ensuring that patients with rare diseases have access to effective treatment is a core priority of PPTA. We request that you work to ensure that the provisions of S. 1423 are passed before the end of the year to promote the continued development of these important treatments and to protect patients with rare diseases. Please contact Kym Kilbourne at kkilbourne@pptaglobal.org or 202-789-3100 with any questions.

Sincerely,

Julie Birkofer
Senior Vice President, North America