

July 10, 2009

Reference No.: FASC09037

The Honorable Max Baucus  
Chairman, Committee on Finance  
United States Senate  
Washington, DC 20510

The Honorable Kent Conrad  
Chairman, Committee on the Budget  
United States Senate  
Washington, DC 20510

**RE: S. 1213, the Patient-Centered Outcomes Research Act of 2009**

Dear Chairman Baucus and Chairman Conrad:

On behalf of the Plasma Protein Therapeutics Association (“PPTA”),<sup>1</sup> I am writing today to express the association’s strong support for your legislation, S. 1213, the Patient-Centered Outcomes Research Act of 2009. PPTA is particularly pleased by the inclusion of the provision requiring that an expert advisory panel for rare disease be convened each time a rare disease is proposed as a comparative effectiveness research (“CER”) topic, as we had made such a recommendation to you in a March 2, 2009 letter. PPTA greatly appreciates your recognition that such a panel that is comprised of patient and physician experts on the unique disease or condition under consideration is best equipped to not only design a CER study for the rare disease, but also determine whether such a study is even appropriate.

Plasma protein therapies are used in the treatment of a number of rare diseases.<sup>2</sup> Most of these disorders are genetic, chronic, life threatening conditions that require, as part of the standard of care, patients to receive regular infusions or injections of plasma protein therapies for

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<sup>1</sup> PPTA is the association that represents human plasma collection centers and the manufacturers of medicinal therapies, including albumin, alpha<sub>1</sub>-proteinase inhibitor, blood clotting factors, and immune globulin from this human plasma. Some of our members also use recombinant DNA technology to produce blood clotting factors. Collectively, these therapies – both plasma-derived and recombinant – are known as “plasma protein therapies.”

<sup>2</sup> The National Institute of Health Office of Rare Diseases generally defines rare diseases as those having a “prevalence of fewer than 200,000 affected individuals in the United States.” See OFFICE OF RARE DISEASES, U.S. DEP’T OF HEALTH & HUMAN SERVS., RARE DISEASE AND RELATED TERMS, *at* <http://rarediseases.info.nih.gov/RareDiseaseList.aspx?PageID=1> (last visited May 12, 2009). Afibrinogenemia, alpha1-antitrypsin deficiency, B-cell chronic lymphocytic leukemia, chronic inflammatory demyelinating polyneuropathy, Guillain-Barre syndrome, hemophilia A, hemophilia B, hyperimmunoglobulinemia E syndrome, hypofibrinogenemia, idiopathic thrombocytopenic purpura, Kawasaki syndrome, Lambert-Eaton myasthenic syndrome, multifocal motor neuropathy, multiple sclerosis, myasthenia gravis, primary immune deficiency disease, staphylococcal toxic shock syndrome, and von Willebrand disease are among the diseases that satisfy this definition.

the duration of their lives. Very often, plasma protein therapies are the only viable treatment option for these patients.

The requirement that the Patient-Centered Outcomes Research Institute (“the Institute”) consult with an expert advisory panel for rare disease each time it proposes the conduct of a CER study on a rare disease is a critical safeguard for patients suffering from conditions like alpha1-antitrypsin deficiency, hemophilia, and primary immune deficiency diseases. Because such a panel must consist of physicians with the relevant clinical experience in the research topic and patients (or their representatives) suffering from the diseases for which the panel is established, patients can be assured that this legislation truly does put them first. PPTA commends you for taking such an initiative.

Consulting with an expert advisory panel for rare disease each time a rare disease is addressed as part of a proposed CER study topic is imperative to ensure the specific rare disease patient population directly affected by the proposed study is adequately represented. For example, patients with rare conditions may receive a range of treatment interventions in the absence of a consensus “standard of care” or any recent clinical guideline, so it can be difficult to establish a consistent comparator for CER. The patients and physicians on this panel will be able to clearly convey such types of treatment regimen nuances to the Institute. For these same reasons, the ability of an expert panel on rare disease to assist in the design of the CER study should the rare disease topic be approved is another vital feature of S. 1213 that PPTA strongly supports. PPTA is also extremely encouraged about the provision that would allow an expert panel on a rare disease to include “a representative of each manufacturer of each medical technology that is included under the relevant topic, project, or category for which the panel is established.” The inclusion of this expert panel for rare disease in S. 1213 is a critical step in protecting patient access to the full range of plasma protein therapies in each therapeutic class.

The protection of such access should also be enhanced by two new factors that S. 1213 requires the Institute to take into consideration as it sets its CER study priorities. Specifically, the Institute must consider “the effect or potential for an effect on patient needs, outcomes, and preferences, including quality of life,” and “the relevance to assisting patients and clinicians in making informed health decisions.” Both of these additional factors for consideration are consistent with PPTA’s previous recommendations that we specifically tailored to plasma protein therapies and rare diseases. For example, PPTA had recommended that CER not be conducted in instances where it is unlikely to yield information that will improve the treatment of patients suffering from the disease under consideration by the Institute. PPTA contended that it is likely that the individual patient reaction to the different brands in a therapeutic class of plasma protein therapies in the treatment of a rare disease will be determinative as to the choice of treatment; thus, such a CER study would be unlikely to provide relevant information to the patient or physician. In making these arguments, we largely focused on potential patient tolerability issues when using IVIG and the risk of inhibitor development when using blood clotting factors. PPTA strongly supports the emphasis that these two new factors for consideration in setting CER study priorities put on patients and their unique characteristics because saving and improving lives is the priority for PPTA and its member companies.

Finally, PPTA also supports the new requirement that CER studies be designed to take into account the differences in “genetic and molecular subtypes” and “quality of life preferences.” Consistent with the focus of S. 1213 on improving personalized medicine and its interest in protecting the rare disease community, the additional language serves an important purpose. This is also true of the requirement that the methodology committee that would be created by S. 1213 to “provide specific criteria for...feasibility” and “standards...by which patient subpopulations can be accounted for and evaluated in different types of research.” Furthermore, PPTA supports the requirement that the methodology committee examine methods to assess quality of life “in a scientifically valid and standardized way.”

With regard to coverage determinations, PPTA supports the language requiring an “iterative and transparent process” for the use of CER by the Secretary in making coverage determinations. This language makes clear that the purpose of CER is not to develop coverage mandates, but to yield information that can be utilized within an iterative and transparent coverage process that considers all available evidence. The language serves as a crucial reminder that, for example, the Centers for Medicare and Medicaid Services must involve stakeholders in its development of a national coverage determination. Requiring such a process and that all relevant evidence in addition to CER be considered should help preserve patient access to the complete range of plasma protein therapies in each therapeutic class.

We hope this important piece of legislation will be included in the Committee on Finance’s broader health care reform legislation. While we appreciate the efforts of many Members of the Senate Committee on Health, Education, Labor, and Pensions during its mark up of the Affordable Health Choices Act, S. 1213 is the far superior CER legislation in the 111<sup>th</sup> Congress and must be the starting point in the health care reform debate. We applaud you and your staff for not only producing a fine piece of legislation, but also your willingness to seriously consider comments from stakeholders, such as PPTA, throughout the process of crafting a policy that will have the most significant long term impact on improving patient care than any other policy in the health care reform debate.

If you have any questions, please contact Jay Greissing ([jgreissing@pptaglobal.org](mailto:jgreissing@pptaglobal.org)) or Jon McKnight ([jmcknight@pptaglobal.org](mailto:jmcknight@pptaglobal.org)) in our office at 202-789-3100.

Sincerely,



Julie Birkofer  
Vice President, PPTA North America

CC: Members of the Senate Committee on Finance, and Senators Dodd, Mikulski, and Coburn