Plasma Protein Therapeutics Association

Internal Revenue Service

Public Hearing on Proposed Regulations
26 CFR Part 51
"Branded Prescription Drug Fee"
[REG-112805-10]
1545-BJ39

IRS Auditorium
Washington, D.C.
November 9, 2012

Comments on behalf of
Plasma Protein Therapeutics Association

Good morning. I am Kym Kilbourne, director of Federal Affairs for the Plasma Protein Therapeutics Association, and I am speaking today on behalf of PPTA. We would like to thank the IRS for the opportunity to offer our comments on the important issues raised by these proposed and temporary rules. We greatly appreciate the IRS's careful efforts thus far to implement the annual branded prescription drug fee in a manner that is fair and consistent with the statute, however we remain concerned about the unintended effects that certain provisions of the rules will have on our members and on the rare disease patients whose lives depend on access to the innovative, life-saving and life-sustaining treatments PPTA members produce.

After providing you with a brief background on the association and the plasma protein therapeutics industry, our comments will detail how the interpretation by IRS of the orphan drug exclusion from the annual branded prescription drug fee negatively and disproportionately affects the manufacturers of plasma protein therapies because of characteristics unique to this segment of the drug industry.

In our comments today, we urge the IRS to clarify that it will exclude sales of all drugs and biologicals approved by the Food and Drug Administration solely to treat one or more rare disease from the calculation of the branded prescription drug fee.

Secondly, though PPTA recognizes the difficulty of accurately calculating Part B sales where multiple drugs share a HCPCS code, we urge the IRS to revise the proposed method to ensure that the calculation of each manufacturer's share of the fee is fair, predictable, and consistent with the requirements of the statute.

PPTA is a global trade association that represents human plasma collection centers and manufacturers of plasma protein therapies. Our members' products include medicinal therapies derived from human plasma and their recombinant analogs. Collectively, these therapies—both plasma-derived and recombinant—are known as "plasma protein

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therapies." The manufacturer membership of PPTA in the U.S. currently includes Baxter, Biotest, CSL Behring, Grifols and Kedrion.

Because plasma protein therapies predominantly treat patients with rare diseases, disorders and conditions, PPTA is particularly sensitive to policies that affect the rare disease community in general and that specifically may hinder or support open access to the plasma protein therapy best suited for the patient's individual clinical needs.

The vast majority of our members' therapies are approved for marketing in the United States by the FDA solely for the treatment of diseases effecting far fewer than 200,000 patients, which is the patient population threshold for designation by the FDA as an orphan drug. The rare, chronic, genetic diseases that physicians treat with plasma protein therapies include primary immune deficiencies, which prevent the body from fighting infection; bleeding disorders including hemophilia, which can cause painful and life-threatening internal bleeding; and alpha-1 antitrypsin deficiency, a genetic form of COPD that can severely diminish lung capacity.

Most therapeutic classes of plasma protein therapies include multiple, unique, non-interchangeable biologicals that have the same active ingredient – for example, the IgG protein in immune globulin therapies and the factor VIII protein in blood clotting factor VIII therapies.

It is typical for the complex and step-wise manufacturing process of plasma protein therapies to take between seven to nine months from the donation of plasma to final lot release of the therapy. Each manufacturer's distinct approach to viral removal, viral inactivation, protein purification, and use of excipient ingredients as stabilizers results in a unique, non-interchangeable brand of therapy despite having the same active ingredient. Consequently, plasma protein therapies are not one-size-fit-all treatments and may give rise to different outcomes in the patient based on his or her biology, medical history, and the specific characteristic of the brand. The patients suffering from the rare diseases that require plasma protein therapies therefore must have open access to each brand in a specific class.

As our comments will explain, having multiple, unique brands with the same active ingredient approved for the same rare condition is significant because of existing FDA regulations that have made it impossible for most plasma protein therapies to obtain orphan drug designation and thus claim the Orphan Drug Act tax credit. Claiming this tax credit is required to exclude the sales of the product from the annual branded prescription drug fee.

Orphan Drug Act regulations require a product that is otherwise the same as an already approved drug to demonstrate a plausible hypothesis of "clinically superior" to that already approved drug in order to be orphan drug designated. Again, for this purpose, the pertinent FDA regulations specify that two products are considered "the same" if they have the same active ingredient and are seeking orphan drug designation for the same orphan indication. The result is that there are existing therapies approved solely to treat one or more rare disease, but that were nevertheless ineligible to have claimed

the Orphan Drug Act tax credit because they were unable to qualify for orphan drug designation.

As such, we urge the IRS use its discretion under the law to clarify that it will exclude sales of all drugs and biologicals approved by the FDA solely to treat one or more rare disease from the calculation of the branded prescription drug fee. The rules, which would define an "orphan drug" by reference to the Orphan Drug Act tax credit under the Internal Revenue Code, risk stifling the innovation and development of critical treatments for rare disease patients.

In contrast, the IRS exercising this discretion will effectively carry out the longstanding policies that Congress has established to encourage the development of rare disease therapies, and should have continued in this instance had they been more sensitive to the nuances of the FDA regulations for orphan drug designation. By protecting manufacturers in the rare disease space from the additional financial burden of being unnecessarily and inappropriately subject to the annual branded prescription drug fee for drugs exclusively treating an orphan population, these manufacturers will be better able to invest in research and development to produce for rare diseases that would otherwise be cost-prohibitive.

Lastly, another effect of multiple brands of biologicals in the same class that were approved prior to October 1, 2003 and are included in the same Health Care Common Procedures Code (HCPCS) is that these therapies have remained in that same HCPCS code and therefore it is impossible for an accurate calculation of the fee liability for those therapies because of the inability to have precise Medicare Part B sales data.

PPTA is specifically concerned about the impact on manufacturers of factor VIII therapy because of the multiple brands in a HCPCS code for plasma-derived factor VIII and multiple brands in a HCPCS code for recombinant factor VIII. The temporary and proposed rules propose to estimate Part B sales for multiple-drug HCPCS codes using the data used to calculate average sales price.

As you know, the ASP data includes all commercial sales, not only sales under Medicare Part B. Thus any manufacturer whose shares of commercial sales as a whole is greater than its share of Medicare Part B sales will pay a larger share of the annual fee than the statute requires. This potential for miscalculation is of particular concern to PPTA members.

Although we recognize the difficulty of accurately calculating Part B sales where multiple drugs share a HCPCS code, nevertheless we urge the IRS to revise the proposed method to ensure that the calculation of each manufacturer's share of the fee is fair, predictable, and consistent with the requirements of the statute. This issue, however, would be eliminated for blood clotting factors and some other plasma protein therapies if the IRS uses its discretion to exclude sales of all drugs and biologicals approved by the FDA solely to treat a rare disease from the calculation of the branded prescription drug fee.

Thank you for your consideration of our industry's concerns. PPTA appreciates the opportunity to present these comments and looks forward to continuing to work with the IRS toward the equitable and accurate implementation of the annual branded prescription drug fee.