

September 23, 2011  
Reference No. FASC 11074

The Honorable Fred Upton  
United States House of Representatives  
Washington, DC 20515

The Honorable Henry Waxman  
United States House of Representatives  
Washington, DC 20515

Dear Chairman Upton, and Ranking Member Waxman,

On behalf of The Plasma Protein Therapeutics Association (“PPTA”), I am writing to express the Association’s strong support for H.R. 2672, the Preserving Access to Orphan Drugs Act of 2011. H.R. 2672 amends section 9008(e) of the Patient Protection and Affordable Care Act to modify the definition of “orphan drug” for the purpose of the annual pharmaceutical fee to include all drugs solely approved for marketing in the United States (“U.S.”) by the Food and Drug Administration (“FDA”) for one or more rare diseases or conditions. This important budget-neutral legislation will ensure sustained therapeutic access for rare disease patients, and will continue the success of the Orphan Drug Act (“ODA”) by preserving incentives for the development and innovation of therapies and drugs used to treat rare diseases.

PPTA represents human plasma collection centers and the manufacturers of medicinal therapies derived from this human plasma including albumin, alpha<sub>1</sub>-proteinase inhibitor, antithrombin III, blood clotting factors, C1 esterase inhibitor, fibrin sealant, immune globulin, hyperimmune immune globulin, and protein C concentrate. Some of our members also use recombinant DNA technology to produce blood clotting factors. Collectively, these therapies—plasma-derived and recombinant—are known as “plasma protein therapies.” The manufacturer membership of PPTA in the United States currently includes Baxter BioScience, Biotest, Cangene, CSL Behring, Grifols, and Kedrion.

Excluding albumin and fibrin sealant, plasma protein therapies are exclusively indicated for the treatment of complex rare diseases, disorders, and conditions.<sup>1</sup> Most of these disorders are genetic, chronic, life threatening conditions that require patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives. Due to the rare nature of these diseases, plasma protein therapies are quite often the only viable treatment option for these patients.

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<sup>1</sup> In the U.S., a “rare disease or condition” is generally defined as a disease or condition that affects less than 200,000 people. *See* 21 U.S.C. § 360bb(a)(2) (2006).

Beginning in September, 2011, the Internal Revenue Service (“IRS”) will collect an excise tax known as the “annual pharmaceutical fee,” calculated based on the sales volume of government purchased branded pharmaceuticals.<sup>2</sup> In calculating the market share for each manufacturer for the purpose of assessing the annual fee, the agency is to exclude sales of “orphan drugs.”<sup>3</sup> The new law narrowly defines “orphan drugs” as it pertains to the annual pharmaceutical fee as drugs for which the manufacturer or sponsor received orphan designation and claimed the ODA tax credit.<sup>4</sup> The result of such a narrow definition is that several drugs and biologics approved for marketing by FDA solely for one or more rare disease remain included in the annual pharmaceutical fee calculation for several manufacturers.

The existing FDA regulatory framework makes it nearly impossible for most plasma protein therapies to receive the orphan designation required to claim the ODA tax credit because of the presence of multiple brands of unique, non-interchangeable biological products in most therapeutic classes (i.e., there are nine brands of immune globulin, eight brands of factor VIII, etc.). Many classes of plasma protein therapies are well established to meet patient needs and satisfy the unique economics of the industry. Without the modification of the definition of “orphan drug” in the annual pharmaceutical fee statute provided in H.R. 2672, future research and development investments will be compromised, affecting the ability of innovators of plasma protein therapies to: (1) produce existing rare disease therapies that would otherwise be cost-prohibitive; (2) bring to market therapies that treat bleeding disorders that have largely been untreated; (3) improve formulations and route of administration for existing therapies; and (4) develop technology to increase the protein yield from each liter of plasma to address growing demand, especially for immune globulin. The Preserving Access to Orphan Drugs Act of 2011 will appropriately apply the orphan drug exclusion equitably to all rare disease therapies. PPTA asks that you support this important legislation that will benefit the rare disease patient communities that require plasma protein therapies as part of their treatment regimen.

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<sup>2</sup> See Patient Protection and Affordable Care Act (“PPACA”) § 9008, Pub. L. No. 111-148, 124 Stat. 119, 859-862, *amended by* Health Care and Education Reconciliation Act of 2010 § 1404, Pub. L. No. 111-152, 124 Stat. 1029, 1064 (codified as amended at 26 U.S.C.S. prec. § 4001).

<sup>3</sup> See PPACA § 9008(e)(3).

<sup>4</sup> *Id.*; Branded Prescription Drug Fee, 76 Fed. Reg. 51245, 51246-51247 (Aug. 18, 2011).

Sincerely,



Julie Birkofer

cc: House Committee on Energy and Commerce, Members and Staff.