

May 26, 2009

Reference No.: FASC09029

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

The Honorable Charles Grassley  
Ranking Member, Committee on Finance  
United States Senate  
Washington, DC 20510

**Re: Financing Comprehensive Health Care Reform: Proposed Health System Savings and Policy Options**

Dear Chairman Baucus and Ranking Member Grassley,

The Plasma Protein Therapeutics Association (“PPTA”) appreciates this opportunity to submit comments to the Senate Committee on Finance on its outline for financing health care reform in the 111<sup>th</sup> Congress. As the committee drafts its health care reform legislation, PPTA respectfully requests that the committee:

1. Include comparative clinical effectiveness research language similar to S. 3408 from the 110<sup>th</sup> Congress, but with a few specific changes that would recognize the critical importance of access to all therapies in a product class that is the accepted standard of care in treating a rare disease;
2. Promote prevention and wellness through the improvement of patient access to plasma protein therapies by including not only S. 701, the Medicare Patient IVIG Access Act of 2009, but also legislation that would restore the payment level in the hospital outpatient prospective payment system for all separately payable non-pass-through drugs and biologicals to a minimum of average sales price +6%
3. Increase the private health insurance lifetime cap as proposed in S. 442, the Health Insurance Coverage Protection Act; and
4. **Maintain the 15.1% Medicaid outpatient drug rebate percentage for plasma protein therapies, while also excluding these therapies from the expansion of such rebate liability to drugs provided through managed care organizations (“MCOs”) – The Moran Company has estimated that these two policies will only cost \$323 million over ten years if the Medicaid outpatient drug rebate percentage is increased from 15.1% to 22.1% for brand drugs [Appendix A: Medicaid Outpatient Drug Rebate Expansion].**

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.” This niche biologicals industry represents only about 1.6% of the overall drug industry in global sales volume.<sup>1</sup>

Plasma protein therapies are used to treat rare and debilitating diseases, disorders, and medical conditions, such as afibrinogenemia, alpha<sub>1</sub>-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.<sup>2</sup> Many of these diseases are chronic, genetic conditions that require, as part of the standard of care, patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives. Very often, plasma protein therapies are the only viable treatment option for these patients.

PPTA is committed to ensuring that patients who require such infusions or injections of lifesaving plasma protein therapies as part of their treatment plan are able to obtain the therapy best suited for their individual needs without impediment. We recognize the importance of health care reform in achieving this goal and support concepts currently under consideration, including comparative clinical effectiveness research. PPTA is, however, deeply troubled about the potential expansion of entitlement programs as a means to cover many of the 46 million uninsured, and the 25 million underinsured.

Before engaging in health care reform, one must understand that not all “drug manufacturers” are the same. The branded pharmaceuticals, the generics, the biologicals, the vaccines, and the plasma protein therapeutics industries not only offer different benefits to the patient, but also require considerably different business models and cost structures that Congress must appreciate and consider when making such dramatic changes, as the Finance Committee has proposed, to our health care system. For example, therapies derived from human plasma cost nearly four times more to manufacture than traditional pharmaceuticals.<sup>3</sup> In addition, unlike the

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<sup>1</sup> Compare PHILIP FLOOD, ET AL., COMMONWEALTH OF AUSTRALIA, REVIEW OF AUSTRALIA’S PLASMA FRACTIONATION ARRANGEMENTS 53 (2006) (showing the plasma protein therapeutics industry is a \$10.5 billion global market in 2006) with IMS Health Inc., Global Pharmaceutical Sales 2001-2008. (describing global sales for the entire pharmaceutical industry in 2006 to be about \$648 billion).

<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).

<sup>3</sup> See Charles Waller, *Historical Perspective on Blood and Plasma Products*, in 7 PHARMACEUTICALS POLICY AND LAW, BLOOD, PLASMA AND PLASMA PROTEINS: A UNIQUE CONTRIBUTION TO MODERN HEALTHCARE 17, fig. 2 (J.L.

broader pharmaceuticals industry, the plasma protein therapeutics industry does not use direct to consumer advertising because our therapies are critical to the quality of life, and in many cases, the survival of the patient.

Nearly all plasma protein therapies<sup>4</sup> rely on the donation of human plasma for the source material. In 2007, more than 85% of human plasma collected for use in the U.S. was source plasma,<sup>5</sup> which cost about \$150 per liter in 2008.<sup>6</sup> The cost of nucleic acid amplification technology testing for HIV and hepatitis A and B is included in this price.<sup>7</sup> Threats of emerging pathogens will also increase the overall manufacturing costs of plasma protein therapies because manufacturers may have to develop new tests and viral inactivation and viral reduction procedures.

In order to recover these significant, unavoidable costs, manufacturers of plasma protein therapies must produce brands in multiple therapeutic classes from each liter of plasma that it fractionates. This economic necessity also provides incentives for plasma fractionators to invest in the research and development of therapies for treating diseases with extraordinarily low prevalence. For example, the Food and Drug Administration recently approved a biologics license application for a plasma-derived coagulant used to treat Factor I protein deficiency, which afflicts only 300 people in the U.S.<sup>8</sup> Some policies outlined in the committee draft will, however, provide disincentives for such innovation in the future.

PPTA member companies are committed to providing treatment for patients. Unfortunately, the added cost of an increased Medicaid outpatient drug rebate percentage and expanded rebate liability will lead to an unsustainable business model that will impede patient access by curtailing the ability of manufacturers of plasma protein therapies to continue the research and development of critical new therapies for rare diseases that are untreated or ineffectively treated, improve formulations and modes of delivery for existing therapies, and develop technology to increase the protein yield from each liter of plasma to address growing demand, especially for immune globulin. PPTA, therefore, opposes the committee's proposal to increase the Medicaid outpatient drug rebate percentage by 53% for branded pharmaceuticals and biologicals as well as its proposal to expand such rebate liability to drugs purchased by MCOs for Medicaid beneficiaries. Increasing what is essentially a tax on innovation is

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Valverde ed., 2005) (providing a comparison of the plasma industry with the pharmaceutical industry through the analysis of Smith Barney estimates from December 2003 and the 2004 Annual Reports of major pharmaceutical companies).

<sup>4</sup> Of the six brands of recombinant blood clotting factors available for consumption in the U.S., four brands contain traces of human plasma or a derivative (albumin).

<sup>5</sup> See THE MARKETING RESEARCH BUREAU, INC., THE PLASMA FRACTIONS MARKET IN THE UNITED STATES 2007 14 (2008).

<sup>6</sup> *Id.* at 40.

<sup>7</sup> *Id.*

<sup>8</sup> See Press Release, CSL Behring, CSL Behring Receives FDA Approval of RiaSTAP(TM), First and Only Approved Treatment of Acute Bleeding Episodes in Patients with Congenital Fibrinogen Deficiency (Jan 16, 2009) available at <http://news.prnewswire.com/ViewContent.aspx?ACCT=109&STORY=/www/story/01-16-2009/0004956268&EDATE> (last visited May 26, 2009).

counterintuitive when the economy is struggling, unemployment is growing, and the hopes for future cures are fading. These Medicaid proposals are particularly arbitrary and unjustified when considering the disparate impact they will have on the plasma protein therapeutics industry, if enacted. Using this same rationale, PPTA does not support expansion of the Medicaid program to cover individuals at 133% of the federal poverty level, as the Pharmaceutical Research and Manufacturers of America recently proposed.<sup>9</sup>

Plasma protein therapies are most generally administered incident to a physician office visit rather than fulfilled in a retail pharmacy, making Medicaid drug rebate liability much more significant for the plasma protein therapeutics industry. According to preliminary plasma protein therapeutics industry survey data collected and analyzed by Georgetown Economic Services, the aggregate unit sales of blood clotting factors, which physicians use to treat patients suffering from hemophilia and other bleeding disorders, in Medicaid fee-for-service and to 340B Drug Pricing Program covered entities amounted to nearly 50% of the total of all blood clotting factors sales in the U.S. in 2008. The aggregate volume of sales to these two federal programs is critical to note not only because of the large discounts manufacturers must provide on such sales, but also because the two programs are statutorily linked, creating a situation where an increase to the Medicaid drug rebate percentage will simultaneously lower the 340B ceiling price at or below which manufacturers must sell product to 340B covered entities.<sup>10</sup>

With nearly five times the exposure to deep, mandatory federal discounts compared to the drug industry as a whole,<sup>11</sup> the Finance Committee's proposals for the Medicaid drug rebate would affect the niche plasma protein therapeutics industry much more acutely than the broader branded pharmaceuticals and biologicals industries; thus, further expansion of the Medicaid population would prove to be unsustainable for this smaller industry. This is especially true considering the rapid growth of the 340B Program. Over the last decade, the number of the covered entities participating in the 340B Drug Pricing Program has increased by nearly 1030% from 1,223 to 13,818.<sup>12</sup>

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<sup>9</sup> See Jennifer Lubell, *Families USA, PhRMA Join Forces, Push Medicaid Expansion*, MODERN HEALTHCARE (April 21, 2009) available at <http://www.modernhealthcare.com/article/20090421/REG/304219976> (last visited May 26, 2009).

<sup>10</sup> See 42 U.S.C. § 256b(a) (2006).

<sup>11</sup> Georgetown Economic Services has indicated that prescription drugs purchased collectively in Medicaid fee-for-service and the 340B Drug Pricing Program amount to approximately 11% of the total of prescription drugs purchased in the U.S. in 2009.

<sup>12</sup> Compare OIG, HHS, AUDIT OF THE UTILIZATION OF THE PUBLIC HEALTH SERVICE 340B DRUG PRICING PROGRAM 3 (1998) with OFFICE OF PHARMACY AFFAIRS, HHS, LIST OF COVERED ENTITIES, <http://openet.hrsa.gov/opa/CE/CEExtract.aspx> (last visited May 12, 2009) [hereinafter "OPA List"]. Legislation currently before the United States House of Representatives would expand the program even further if enacted. See H.R. 444, 111<sup>th</sup> Cong. H.R. 444 would not only expand the program to cover drugs purchased for use in the hospital inpatient setting, but also expand the type of covered entities eligible for the program to include children's hospitals, critical access hospitals, rural referral centers, and sole community hospitals that meet the DSH requirements. Additionally, this bill would also expand the program to cover mental health facilities, substance abuse centers, Medicare-dependent small rural hospitals, and facilities, in addition to hemophilia treatment centers, that receive grants from the Maternal and Child Health Bureau.

The plasma protein therapeutics industry will neither be able to cross-subsidize the 53% increase to the Medicaid outpatient drug rebate percentage in the same manner as the rest of the drug industry because of its limited pricing flexibility due to the generic drug-like competition in each therapeutic class, nor will it be able to offset these costs through an increase in patient volume. It would be incorrect to assume that health care reform will create a windfall for the plasma protein therapeutics industry. Indeed, because plasma protein therapies are primarily used to treat patients suffering from life threatening, chronic diseases, such patients that are uninsured and underinsured are generally already receiving treatment through various patient assistance programs; thus, the plasma protein therapeutics industry will see few if any of the estimated 71 million potentially new patients resulting from universal health care coverage. In contrast, the generics industry, which offers little beyond cost containment, will stand to benefit the most from health care reform, especially if Congress passes biosimilars legislation as part of the health care reform package. As a matter of equity, the plasma protein therapeutics industry should not be subject to a 53% increase when the committee has proposed a mere 18% increase for the generics industry.

In conclusion, PPTA urges the committee to create a special class of products – “plasma protein therapies” – for the purpose of the Medicaid outpatient drug rebate program. Allowing this class to maintain its current minimum rebate percentage of 15.1% will preserve this niche industry’s health, which will help ensure continued patient access to these therapies. Thank you for your consideration. If you would like to discuss these comments further, 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

Attachments

**APPENDIX A: MEDICAID OUTPATIENT DRUG REBATE EXPANSION – PPTA URGES CONGRESS TO REFRAIN FROM ENACTING A 53% INCREASE TO THE MEDICAID OUTPATIENT DRUG REBATE APPLIED TO PLASMA PROTEIN THERAPIES**

- PPTA urges Congress to create a special class of products – “plasma protein therapies” – for the purpose of the Medicaid outpatient drug rebate program. This class should maintain their current minimum rebate percentage of 15.1%.
- The plasma protein therapeutics industry differs considerably from the broader branded pharmaceuticals and biologicals industries; thus, increasing the Medicaid outpatient drug rebate percentage from 15.1% to 23.1% – an overall increase of more than 53% – would have a much different impact on patient access to plasma protein therapies.
  - ◆ Plasma protein therapies are complex biologicals, which are most generally administered incident to a physician office visit rather than fulfilled in a retail pharmacy, making Medicaid drug rebate liability much more significant for the plasma protein therapeutics industry – for example, Medicaid fee-for-service and 340B Drug Pricing program covered entities account for nearly 50% of the aggregate unit sales of blood clotting factors sold in the U.S., giving this industry nearly five times more exposure to deep federal discounts than the rest of the drug industry.
  - ◆ Plasma protein therapies, which are often the only treatment option for some rare, chronic, and debilitating diseases, disorders, and medical conditions, must generally be infused or injected regularly for the duration of a patient’s life.
  - ◆ Plasma protein therapies, including most recombinant blood clotting factors, rely on the donation of human plasma for the source material, which at \$150 per liter,<sup>1</sup> creates a very unique manufacturing cost structure for the industry – approximately four times greater than for the pharmaceuticals industry.
  - ◆ The plasma protein therapeutics industry has had previous patient access issues resulting from closures of plasma collection centers due to market forces.
- A 53% increase to the Medicaid drug rebate results in a simultaneous 53% decrease in the 340B Drug Pricing Program ceiling price, which is critical to note because a large amount of plasma protein therapies are also subject to the 340B discount.
- The overall impact of increasing the rebate percentage by 53% on both the Medicaid Outpatient Drug Rebate Program and the 340B Drug Pricing Program is the type of policy change that could have far reaching consequences for the plasma protein industry.
- Because a 53% increase to the minimum rebate percentage level is unprecedented, anticipating the market response to the Finance Committee’s proposal is difficult. Congress has, however, recognized that the initial 12.5% rebate level in 1991 led to unsustainable price increases by drug manufacturers on drugs sold to the Department of Veterans Affairs, federally funded clinics, public hospitals, HMOs, and GPOs.

**PPTA Position:** In order to preserve patient access to plasma protein therapies, PPTA urges Congress to create a new class of products – “plasma protein therapies” – for the purpose of the Medicaid Outpatient Drug Rebate Program. Under this proposal, “plasma protein therapies” would continue to be subject to the minimum rebate percentage of 15.1% and be limited to alpha-1-proteinase inhibitor, blood clotting factors, and immune globulin.

**Issue:** Currently, manufacturers of plasma protein therapies generally pay each state Medicaid program a rebate of 15.1% of a therapy’s reported average manufacturer’s price (“AMP”) under the Medicaid Outpatient Drug Rebate Program. The Obama Administration’s FY 2010 budget proposes to increase this minimum rebate percentage from 15.1% to 22.1% – an overall increase of more than 46% – for covered outpatient brand name drugs, such as plasma protein therapies. The Senate Committee on Finance has proposed to increase the minimum rebate percentage to as much as 23.1% – an overall increase of more than 53%. Additionally, both the President and the Finance Committee have proposed to expand the Medicaid drug rebate to sales to Medicaid managed care organizations (“MCOs”) and to apply the “additional rebate amount” to new formulations of existing drugs. The proposed 46% to 53% increase to the minimum rebate percentage and the overall expansion of the Medicaid outpatient drug rebate program would particularly penalize the plasma protein therapeutics industry and consequently impede access for those patients who require these lifesaving therapies.

**Current Law:** Section 1927(a) of the Social Security Act compels a manufacturer seeking federal reimbursement for its covered outpatient drugs from both Medicaid and Medicare Part B to participate in both the 340B Drug Pricing Program and the Medicaid Outpatient Drug Rebate Program. The rebate program generally requires manufacturers to provide to each state Medicaid program a rebate on the manufacturer’s covered outpatient drugs that the state has reimbursed.<sup>ii</sup> The 340B Program requires manufacturers that sell drugs to certain federally funded grantees and other safety net health providers to do so at or below the 340B ceiling price, which is calculated in the same manner as the Medicaid rebate amount.<sup>iii</sup>

- ***Basic rebate amount:*** The Centers for Medicare and Medicaid Services (“CMS”) calculates the rebate amount for most drugs and biologicals as the greater of the minimum rebate percentage (15.1% for brands) of the AMP reported for the product or the difference between the AMP and the “best price” reported, plus any “additional rebate amount.”<sup>iv</sup>
  - ***Additional rebate amount:*** CMS applies an extra “penalty” rebate to brand name drugs when the AMP for a product increases faster than a specified inflation factor.<sup>v</sup> This added rebate unfairly subjects plasma protein therapies to a much higher rebate amount and a lower 340B ceiling price as compared to many other drugs or biologicals in the marketplace because price increases for plasma protein therapies will often outpace inflation. This is a result of significant increases in manufacturing expenditures – including the costs of human plasma, emerging technologies, and safety requirements – that are unique to producing these therapies and cannot be avoided.
  - ***Exceptions:*** Vaccines are statutorily exempt from Medicaid drug rebate liability.<sup>vi</sup>

**Previous Statutory Protections for Plasma Protein Therapies:** Because preserving patient access to plasma protein therapies has historically been a priority for Congress, precedent exists for providing them with special consideration in developing legislative solutions. For example, in 2003, the Medicare Modernization Act:

1. exempted intravenous immune globulin (“IVIG”) from the competitive acquisition program that it established;<sup>vii</sup>
2. created an add-on payment for blood clotting factors after recognizing the additional costs associated with furnishing the therapy;<sup>viii</sup> and
3. maintained the *status quo* for Medicare reimbursement of blood clotting factors as Medicare began transitioning toward a new reimbursement methodology.<sup>ix</sup>

**Plasma Protein Therapies Treat Rare Diseases:** Plasma protein therapies are used to treat rare and debilitating diseases, disorders, and medical conditions, such as 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.<sup>x</sup> Many of these diseases are chronic, genetic conditions that require, as part of the standard of care, patients to receive regular infusions or injections of plasma protein therapies for the duration of their lives. Very often, plasma protein therapies are the only viable treatment option for these patients.

**Continued Patient Access to Plasma Protein Therapies Requires a Healthy Industry:** The plasma protein therapeutics industry continues to grow. In 2007, plasma collection increased by 17% with approximately 14.6 million liters of human plasma available for fractionation in the United States.<sup>xi</sup> Aided by the rise in raw material, U.S. distribution of plasma protein therapies has also expanded. For example, in 2008, immune globulin distribution rose by nearly 8% from the previous year to 36,788 kg. Several variables unique to the plasma protein therapeutics industry are determinative of a healthy industry and may be adversely affected by unsuitable public policies. PPTA member companies are committed to providing treatment for patients, but the added cost of an increased Medicaid rebate percentage will lead to an unsustainable business model that will impede patient access and curtail development of new manufacturing processes and therapies.

- ***Unique manufacturing cost structure of the plasma protein therapeutics industry:*** Plasma-derived therapies cost nearly four times more to manufacture than traditional pharmaceuticals.<sup>xii</sup> In 2007, more than 85% of human plasma collected for use in the U.S. was source plasma,<sup>xiii</sup> which cost about \$150 per liter in 2008.<sup>xiv</sup> The cost of nucleic acid amplification technology testing for HIV and hepatitis A and B is included in this price.<sup>xv</sup> Threats of emerging pathogens will also increase the overall manufacturing costs of plasma protein therapies because manufacturers may have to develop new tests and viral inactivation and viral reduction procedures. In order to recover these significant, unavoidable costs, manufacturers of plasma protein therapies must produce brands in multiple therapeutic

classes from each liter of plasma that it fractionates. It should be noted that these costs increase the risk for plasma protein therapies to be subject to the “additional rebate amount.”

- ***Unique marketplace volatility:*** For more than a decade, the plasma protein industry has been characterized by its volatility. Similar to the volatility of the vaccines industry, there are few market participants and a history of supply challenges. While the volatility of the two industries has been driven by different factors, Congress must recognize the similar link – it is critical to the public health for Congress to protect the viability of both the vaccines and the plasma protein therapeutics industries. Like the liability exposure that nearly decimated the vaccines industry in the 1980s, a higher Medicaid rebate percentage and an increased volume of sales at or below an even lower 340B ceiling price could have a similar impact on the plasma protein therapeutics industry.
  - ***IVIG shortage in 1997 and the resulting allocation system:*** In 1998, Congress and HHS pressured the industry to allocate IVIG based on historical utilization of its authorized distributors and group purchasing organizations because of a 20% shortage of IVIG in 1997.<sup>xvi</sup> HHS has recognized that although manufacturers of plasma protein therapies are developing new technologies to handle increasing demand for these critical therapies, these manufacturers are unable to increase allocation for certain therapies to existing customers or for the purpose of accommodating new customers, including some 340B covered entities.
  - ***Structural changes from 2002 through 2004:*** Within this two-year period, market forces resulted in the closures of nearly 100 plasma collection centers. Because the plasma protein therapeutics industry relies on human plasma collection to meet therapy demand, further closures could have a detrimental impact on patient access to therapy.
  - ***Patient access difficulties for IVIG since 2005:*** In 2005, a shift to a new Medicare reimbursement methodology resulted in significant payment cut for IVIG. Because of this reduction, some physicians discontinued providing this lifesaving therapy, thereby driving many patients to hospital outpatient departments, which were initially unprepared for the surge of new patients. Allocation of IVIG based on historical utilization contributed to some of the delays faced by patients who shifted their site of service.
- ***Unique effect of the 340B Drug Pricing Program:*** In creating the 340B Program, it was the intent of Congress to “establish price controls to limit the cost of drugs to Federal purchasers and to certain grantees of Federal agencies.”<sup>xvii</sup> Congress believed such protection against excessive pharmaceutical prices would “enable these entities to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services.”<sup>xviii</sup> Over the last decade, the number of the covered entities participating in the 340B Drug Pricing Program has increased by nearly 1030% from 1,223 to 13,818.<sup>xix</sup> As a result, a rapidly growing portion of plasma protein therapies are sold at or below the 340B ceiling price.

- According to 2007 Medicare hospital outpatient prospective payment system claims data, a large volume of plasma protein therapies – especially blood clotting factors – dispensed or administered in the hospital outpatient department are done so from the 2,332 disproportionate share hospital sites<sup>xx</sup> that have purchased these therapies at or below the 340B ceiling price:
  - 27% of alpha<sub>1</sub>-proteinase inhibitor
  - 32% of immune globulin (defined as IVIG and SCIG)
  - 64% of blood clotting factors (defined as von Willebrand factor, plasma-derived and recombinant factor VIII; plasma-derived and recombinant factor IX; prothrombin complex concentrate; and activated prothrombin complex concentrate);
- According to consumer organizations, approximately 80% of the approximately 20,000 hemophilia patients (Hemophilia A and Hemophilia B) in the U.S. receive care at the 147 federally funded comprehensive hemophilia treatment centers (“HTCs”) throughout the U.S.;
- 3,363 of those hemophilia patients that receive care at these HTCs (nearly 21%) are Medicaid fee-for-service beneficiaries, while approximately 840 additional hemophilia patients are Medicaid fee-for-service beneficiaries that receive treatment from sites of service other than an HTC;
- 94 of these HTCs (nearly 64%) are enrolled in the 340B Drug Pricing Program and, thus, qualify for the 340B ceiling price;<sup>xxi</sup>
- The Congressional Budget Office has estimated that the Medicaid program will account for 9.1% of the estimated drug expenditures in the U.S. in 2009;<sup>xxii</sup>
- The 340B Program accounts for 1.9% of total U.S. drug expenditures;<sup>xxiii</sup>
- According to analysis by Georgetown Economic Services, in 2008, the aggregate unit sales of blood clotting factors in Medicaid fee-for-service and to 340B covered entities amounted to 46% of the total U.S. sales by manufacturers of these lifesaving therapies, giving this niche industry **nearly five times the exposure to deep, mandatory federal discounts as the rest of the drug industry.**

**Inherent Uncertainty of the Impact of the Rebate Increase on Patient Access:** Congress has only once increased the rebate percentage for branded pharmaceuticals, but not near the level the President and the Senate Finance Committee have proposed to increase it. Because an increase as large as 53% to the minimum rebate percentage level is unprecedented, except for the initial implementation of the Medicaid Outpatient Drug Rebate Program on January 1, 1991, when the minimum rebate percentage began at 12.5%,<sup>xxiv</sup> anticipating the market response to the committee’s proposal is difficult. It is, however, important to note that Congress recognized that this initial rebate level in 1991 led to unsustainable price increases by drug manufacturers on drugs sold to the Department of Veterans Affairs, federally funded clinics, and public hospitals.<sup>xxv</sup> These price increases resulted in the new

requirements for the Federal Supply Schedule and the creation of the 340B Drug Pricing Program.<sup>xxvi</sup> Moreover, within two years of the implementation of the Medicaid Outpatient Drug Rebate Program, prices for drugs sold to large private purchasers, such as HMOs and GPOs, increased substantially.<sup>xxvii</sup> As such, the Government Accountability Office has previously concluded that further expansion of federal prices for prescription drugs, which would occur by increasing the Medicaid drug rebate percentage beyond 15.1% for brand drugs, will likely lead to both federal and nonfederal purchasers paying more for their drugs “if drug manufacturers raise prices to them to offset revenue losses resulting from extending federal prices” beyond the current levels.<sup>xxviii</sup>

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<sup>i</sup> See THE MARKETING RESEARCH BUREAU, INC., THE PLASMA FRACTIONS MARKET IN THE UNITED STATES 2007 40 (2008) [hereinafter “MRB Report”].

<sup>ii</sup> See 42 U.S.C. § 1396r-8(b)(1)(A) (2006).

<sup>iii</sup> See 42 U.S.C. § 256b(a) (2006).

<sup>iv</sup> See 42 U.S.C. § 1396r-8(c).

<sup>v</sup> See 42 U.S.C. § 1396r-8(c)(2)(A).

<sup>vi</sup> See 42 U.S.C. § 1396r-8(k)(2)(B).

<sup>vii</sup> See 42 U.S.C. § 1395u(o)(1)(E)(ii) (2006); see also H.R. REP. NO. 108-391 at 593 (2003).

<sup>viii</sup> See 42 U.S.C. § 1395u(o)(5).

<sup>ix</sup> See 42 U.S.C. § 1395u(o)(1)(A)(ii).

<sup>x</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).

<sup>xi</sup> See MRB Report, *supra* note i at 14.

<sup>xii</sup> See Charles Waller, *Historical Perspective on Blood and Plasma Products*, in 7 PHARMACEUTICALS POLICY AND LAW, BLOOD, PLASMA AND PLASMA PROTEINS: A UNIQUE CONTRIBUTION TO MODERN HEALTHCARE 17, fig. 2 (J.L. Valverde ed., 2005) (providing a comparison of the plasma industry with the pharmaceutical industry through the analysis of Smith Barney estimates from December 2003 and the 2004 Annual Reports of major pharmaceutical companies).

<sup>xiii</sup> See MRB Report, *supra* note i, at 14.

<sup>xiv</sup> *Id.* at 40.

<sup>xv</sup> *Id.*

<sup>xvi</sup> See *Public Health 2000: Immune Globulin Shortages – Causes and Cures: Hearing Before the Subcomm. On Human Resource of the House Comm. on Government Reform and Oversight*, 105<sup>th</sup> Cong. 2 (1998) (statement of Rep. Shays, Chairman, Subcomm. on Human Resources of the House Comm. on Government Reform and Oversight).

<sup>xvii</sup> See Guidance Regarding Section 602 of the Veterans Health Care Act of 1992: Limitation on Prices of Drugs Purchased by Covered Entities, 58 Fed. Reg. 27289 (May 7, 1993).

<sup>xviii</sup> H.R. REP. NO. 102-384, PART 2 (1992).

<sup>xix</sup> Compare OIG, HHS, AUDIT OF THE UTILIZATION OF THE PUBLIC HEALTH SERVICE 340B DRUG PRICING PROGRAM 3 (1998) with OFFICE OF PHARMACY AFFAIRS, HHS, LIST OF COVERED ENTITIES, <http://openet.hrsa.gov/opa/CE/CEExtract.aspx> (last visited May 12, 2009) [hereinafter “OPA List”]. Legislation currently before the United States House of Representatives would expand the program even further if enacted. See H.R. 444, 111<sup>th</sup> Cong. H.R. 444 would not only expand the program to cover drugs purchased for use in the hospital inpatient setting, but also expand the type of covered entities eligible for the program to include children’s hospitals, critical access hospitals, rural referral centers, and sole community hospitals that meet the DSH requirements. Additionally, this bill would also expand the program to cover mental health facilities, substance abuse centers, Medicare-dependent small rural hospitals, and facilities, in addition to hemophilia treatment centers, that receive grants from the Maternal and Child Health Bureau.

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<sup>xx</sup> *Id.*

<sup>xxi</sup> *See* OPA List, *supra* note xix.

<sup>xxii</sup> *See* CONGRESSIONAL BUDGET OFFICE, KEY ISSUES IN ANALYZING MAJOR HEALTH INSURANCE PROPOSALS 19 (2008)

<sup>xxiii</sup> *See* Lisa Scholz, *The 340B Drug Pricing Program: State Optimization of 340B*, Presentation Before the National State Conference of State Legislatures' Legislative Summit, July 27, 2008 available at <http://www.ncsl.org/programs/health/drug340B.htm> (estimating that the 340B Program prescription drug expenditures in 2008 would be \$5 billion). Using this estimate of \$5 billion as an approximate measure of 2009 expenditures, one can attribute 1.9% of total U.S. drug sales to 340B.

<sup>xxiv</sup> *See* 42 U.S.C. § 1396r-8(c)(1)(B)(i)(I).

<sup>xxv</sup> *See* H.R. REP. NO. 102-384, PART 2 (1992).

<sup>xxvi</sup> *Id.*

<sup>xxvii</sup> *See* U.S. Gen. Accounting Office, Prescription Drugs: Expanding Access to Federal Prices Could Cause Other Price Changes 18-19 (Aug. 2000).

<sup>xxviii</sup> *Id.* at 19.

**Estimating the  
Budgetary Impact of  
Establishing an  
Alternative Medicaid  
Rebate Policy for  
Plasma Protein  
Products**

**May, 2009**

**THE MORAN COMPANY**

## **Estimating the Budgetary Impact of Establishing an Alternative Medicaid Rebate Policy for Plasma Protein Products**

Under the Medicaid program, plasma protein products are subject to the rebate requirements imposed on manufacturers of branded drugs. Manufacturers must report an Average Manufacturer Price (AMP) for each product to the Secretary of Health & Human Services, and pay both “basic” and “additional” rebates based on their reported AMP. The basic rebate requires manufacturers to pay the greater of (a) 15.1% of AMP, or (b) the amount of discount provided via the “best price” the manufacturer has charged to a non-Federal customer in the retail class of trade during the most recent reporting period. An additional rebate must also be paid if the reported AMP for a product exceeds the cumulative growth in the Consumer Price Index (CPI) since the introduction of that product. Under current law, drugs provided to Medicaid beneficiaries via the drug reimbursement mechanisms of managed care organizations are not subject to rebates.

The President’s budget proposal contains three proposed amendments to the Medicaid rebate requirements:

- The minimum percentage for the basic rebate would be increased to 22.1%.
- For new formulations of previously-marketed products, liability for additional rebates would be determined by applying the pricing history of the predecessor product to the new product.
- The exemption from rebates for drugs delivered via managed care organizations would be eliminated.

If these policies are actively considered in the Federal legislative process, the Plasma Protein Therapeutics Association (PPTA), a trade group of manufacturers of these products, is considering advocating for an amendment to establish a separate set of rebate requirements for plasma protein products. Requirements for that category would be identical to those now imposed on all branded drugs under current law.

The Moran Company was engaged by PPTA to evaluate the budgetary impact of establishing separate requirements for plasma protein products in the manner proposed. Specifically, we were asked to estimate how the Congressional Budget Office (CBO) might “score” such an amendment if offered to a bill containing the President’s budget policies for Medicaid rebates.

Our findings are as follows:

- Since plasma protein products are typically sold under multi-year contracts between manufacturers and distributors, so-called “best price” rebates are not a major factor in determining rebate obligations.
- Because plasma protein products are naturally-occurring proteins, the “new formulations” policy in the President’s budget would not meaningfully affect the determination of additional rebates.

- The combined effect of retaining the basic rebate percentage at 15.1%, and continuing the managed care exemption would increase mandatory Federal spending by \$123 million over 2010-2014 and by \$323 million over 2010-2019, relative to the President's proposed policies.

## Technical Approach

Since our assignment is to estimate how CBO might score such a policy, we relied, in developing our estimates, on budgetary analyses of comparable policies presented in CBO's recent Budget Options document.<sup>1</sup> In addition to estimating the budgetary effects of various changes to the basic rebate calculation, they also estimated two variations on eliminating the managed care exemption. As a baseline, we used the February, 2009 projections of Medicaid drug spending presented in the National Health Expenditures estimates prepared by the Office of the Actuary at the Centers for Medicare & Medicaid Services (CMS).

The impact of alternative rebate policies on plasma protein products is importantly affected by the distinctive character of the market for these products, which is dominated by cyclical swings in the availability of supply. In recent years, manufacturers and distributors have entered into long-term contracts that even out the pricing swings that would otherwise occur.<sup>2</sup> It is our understanding that essentially all of volume manufactured in the United States is delivered through these contracts. Hence we would not expect "best price" to be a major factor in determining manufacturers' rebate obligations.

We based our estimates of the volume of Medicaid spending on plasma protein products on historical Medicaid spending data for these products. In the last two years for which data are available (2007, and the first three quarters of 2008), Medicaid spending on plasma protein products declined from 2.0% to 1.6% of total Medicaid spending.<sup>3</sup> We generated estimates of baseline spending on plasma protein products by assuming cyclical variation between these extremes.<sup>4</sup> Using historical relationships between Medicaid pharmacy reimbursements and AMP for branded drugs, we estimated to the total volume in each year through 2019, with the volume priced at estimated AMP.

Using this baseline, we directly estimated the impact of reducing the minimum rebate from the assumed 22.1% to 15.1%. Based on our assumption that best price is negligible for these products in the retail class of trade, our estimates assume that rebates would decline by the full amount of the change in the minimum percentage (7.0% of AMP). Since this estimate reflects the combined state and federal rebate amount, we translated it into an estimate of the change in

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<sup>1</sup> Congressional Budget Office, *Budget Options Volume I: Health Care*, December, 2008, pp. 138-142

<sup>2</sup> While price swings are still observed in the spot market, these are ex-manufacturer sales and hence do not appear in the context of AMP and best price reporting.

<sup>3</sup> By contrast, in the 2006 data, spending on protein plasma products comprised 3.0% of total spending.

<sup>4</sup> While long term contracts make pharmacy acquisition costs stable for the volume guaranteed under those contracts, pharmacies experiencing demand in excess of their guaranteed quota must acquire products under prevailing market conditions.

mandatory federal spending based on an assumed federal matching percentage for drug spending of 56.0%.

To estimate the impact of maintaining the managed care exclusion, we started with CBO's estimate of the effect of requiring manufacturers to provide rebates to states for such volume. We translated their published score into a percentage change in the projected baseline for Medicaid drug spending, and applied that percentage change to our projected baseline of plasma protein product spending. Since CBO priced that policy assuming no other changes in rebate policy, we grossed the effect upward to reflect the fact that, under the President's policy, these rebates would be calculated using a 22.1% minimum basic rebate.<sup>5</sup>

Our evaluation of the effects of the President's proposal to modify the rules for calculating the additional rebates leads us to conclude that CBO would be unlikely to assume that that policy would have a material effect on plasma protein products. Unlike so-called small molecule drug products, which can be transformed into a new product through changes in formulation or dosage, plasma protein products are naturally-occurring components of human blood that are fractionated from plasma, rather than manufactured.<sup>6</sup> Hence we expect CBO to conclude that the President's proposed policy regarding pricing data used to determine the additional rebate would have no effect in this sector, and hence that exempting plasma protein products from these requirements would not affect the scoring of the policy under consideration.

Our estimates of the budgetary impact of the amendment under consideration are as follows:

	2010	2011	2012	2013	2014	2015	2016	2017	2018	2019	2010-2014	2010-2019
Change in Mandatory Spending (\$\$ M)												
Restore 15.1% Basic Rebate	\$ 8	\$ 9	\$ 11	\$ 12	\$ 14	\$ 14	\$ 14	\$ 14	\$ 14	\$ 16	\$ 54	\$ 126
Exempt from New Additional Rebate	---	---	---	---	---	---	---	---	---	---	\$ -	\$ -
Restore Managed Care Exception	\$ 3	\$ 9	\$ 16	\$ 19	\$ 22	\$ 23	\$ 23	\$ 26	\$ 26	\$ 31	\$ 69	\$ 197
Totals	\$ 11	\$ 18	\$ 27	\$ 31	\$ 36	\$ 37	\$ 37	\$ 40	\$ 40	\$ 47	\$ 123	\$ 323

<sup>5</sup> In its evaluation of alternative Medicaid rebate policies, CBO estimated revenue effects (and also analyzed potential changes in appropriated spending requirements) that would result from changes in manufacturer pricing behavior due to changes in the effects of "best price." Since we assume that best price is not material in determining rebate obligations for these products, we do not believe that the policy under consideration will generate revenue effects.

<sup>6</sup> While some plasma protein products, e.g., clotting factors are sometimes manufactured via recombinant DNA technology, these products are not candidates for "evergreening" via formulation changes.