August 31, 2009
Reference No.: FASC09043

Charlene Frizzera
Acting Administrator, Centers for Medicare and Medicaid Services
Department of Health and Human Services
Room 445-G
Hubert H. Humphrey Building
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: CMS–1413–P (Medicare Program; Payment Policies Under the Physician Fee Schedule and Other Revisions to Part B for CY 2010)

Dear Acting Administrator Frizzera:

The Alpha-1 Association, Alpha-1 Foundation, Guillain-Barre Syndrome (GBS)/ Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) Foundation International, Hemophilia Federation of America and Immune Deficiency Foundation as 'stakeholders' within the community of patients who rely upon lifesaving plasma derived and recombinant analog therapies and ASD Healthcare and the Plasma Protein Therapeutics Association ("PPTA") appreciate this opportunity to comment on the proposed rule regarding revisions to payment policies under the Medicare physician fee schedule, published in the *Federal Register* on July 13, 2009 (Proposed Rule).[1] As stakeholders deeply committed to the health and safety of the patients it serves, these comments on the Proposed Rule are intended to ensure that Medicare beneficiaries have full access to the complete range of life-saving, Food and Drug Administration (FDA) approved, plasma-based and their recombinant analog therapies (plasma protein therapies) in the physician office setting. Plasma protein therapies treat serious medical conditions for a very small patient fragile patient population in the United States. These therapies include blood clotting factor therapies for individuals with bleeding disorders such as hemophilia; alpha-1 proteinase inhibitors (A1PI) for treating genetic emphysema clinically known as alpha-1 antitrypsin deficiency disorder; and intravenous immunoglobulin (IVIG) therapies used in the treatment of primary and secondary immune deficiencies as well as autoimmune diseases including Guillain-Barre Syndrome and Chronic Inflammatory Demyelinating Polyneuropathy (CIDP).

Patient access to plasma protein therapies depends on adequate physician reimbursement for the acquisition and administration of these biologicals, as well as the availability of all plasma protein therapies to Medicare beneficiaries. With the reemergence of the competitive acquisition program (CAP), we are concerned that

1 74 Fed. Reg. 33520 (Jul. 13, 2009).

inclusion of plasma protein therapies in CAP would curtail patient access to the specific therapy that would best suit the needs of beneficiaries. As a result, consistent with the Proposed Rule, CMS should not include plasma protein therapies in CAP. We are concerned about the proposed reduction in the payments for drug administration services that are billed when furnishing plasma protein therapies that stems from the use of a new survey. As the agency has done with other practice expense changes, CMS should establish a transition period to implement the changes resulting from the survey to avoid payment reductions that could affect the availability of plasma protein therapies and other products.

DISCUSSION

I. PLASMA PROTEIN THERAPIES SHOULD NOT BE INCLUDED IN CAP

The Proposed Rule signals the agency's intention to reestablish CAP. In doing so, the agency proposes changes to the CAP drug list to make it a narrower list of drugs. Specifically, the agency proposes to work from the prior drug list and "filter" the list by specialties that most frequently prescribe drugs under CAP and the highest dollar volume CAP drugs. In addition, CMS proposes to "fill in" groups of drugs with related products not on the list. The Proposed Rule identifies the products that would be on the next CAP list, none of which are plasma protein therapies.[2] As discussed in more detail below, whether CMS finalizes the proposed changes to the CAP drug list or uses other criteria to populate the list, plasma protein therapies should not be included in the next CAP drug list because this program cannot ensure that beneficiaries have full access to these therapies.

For many plasma protein therapies (e.g., blood clotting factors and A1PI), there are multiple brand name products under a single Healthcare Common Procedure Coding System (HCPCS) code. These brand name products are not interchangeable. The different brand name products have clinical differences such that a particular product may be more beneficial for certain patients. Under CAP, a contractor would only be required to offer one product within a HCPCS code. As such, the danger of subjecting blood clotting factors and A1PI to competitive acquisition is that the participating vendor might not provide each of the therapies within the HCPCS code, but offer only the therapy for which they are able to negotiate the lowest acquisition price. The problematic nature of this, and thus the need for exclusion from the CAP drug list, is illustrated well by the discussion below of the unique aspects of blood clotting factors and A1PI.

Individuals with hemophilia and persons with other bleeding disorders are dependent upon lifesaving blood clotting factors and must have unfettered access to sustain life. For persons with hemophilia who infuse blood clotting factor on a regular

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2 74 Fed. Reg. at 33625-33627.

basis to replace absent proteins, the ability to choose which therapy works best for them is an important component of their treatment regimen. Blood clotting factors are not interchangeable, some are derived from human plasma, while others are recombinant, created from single cells. It is crucial that the consumer and physician have the freedom to jointly determine the most efficacious therapy. Without treatment, individuals with hemophilia bleed internally, causing severe joint damage and potentially fatal outcomes.

Alpha-1-Antitrypsin Deficiency (A1AD) is one of the most prevalent, potentially lethal hereditary disorders. A1AD is the leading cause of pediatric liver transplants and causes chronic obstructive pulmonary disease with a high frequency of panacinar emphysema in adults. The only known treatment to stop disease progression in patients with A1AD is the weekly infusion of plasma derived A1PI. Weekly infusions help maintain a protective level of alpha-1 protein in the blood stream. Without adequate plasma protein therapy, patients suffer from repeated infections resulting in reduced lung function, hospitalization and reduced quality of life. Others develop relentless progressive pulmonary emphysema often leading to premature respiratory death.

A1PI acts to inhibit destructive enzymes, or protease, that causes damage to the liver (*e.g.*, fibrosis and cirrhosis) and to the lungs (*e.g.*, emphysema). A1PI requires a series of complex manufacturing steps, validation criteria, and constantly evolving viral inactivation processes, all intended to ensure that the product is safe and effective. In addition, the product is subject to extremely extensive regulatory oversight from the FDA, which significantly adds to manufacturing costs. There are three Alpha-1 proteinase inhibitors on the market, all of which are billed under the same HCPCS code. As a result, under CAP, vendors would only have to offer one A1PI product.

This would be problematic because no one manufacturer can support the entire U.S. market on its own given the current manufacturing capacity. Moreover, there are occasions in which a manufacturer may have to stop production of A1PI (*e.g.*, to perform a routine cleaning or in the case of a natural disaster). In such circumstances, having multiple products allows patients to continue to have access to the therapy they need. Further, since each patient is different and each responds differently to the therapies, having more options is better clinically. Thus, including A1PI on the CAP drug list with vendors only having to offer one product could impede beneficiary access to this critical treatment.

Products like blood clotting factors and A1PI are precisely why Congress authorized CMS to exclude products from CAP if the program (i) is not likely to result in savings for the products or (ii) is likely to have an adverse impact on access to the products.[3] For the reasons noted above, inclusion of these products, as well as other

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[3] SSA § 1847B(a)(1)(D).

plasma protein therapies, would have an adverse impact on access. Therefore, these products should not be on any CAP drug list.[4]

II. CMS SHOULD ESTABLISH A TRANSITION MECHANISM FOR THE USE OF THE PHYSICIAN PRACTICE INFORMATION SURVEY

In the Proposed Rule, CMS indicates that the American Medical Association (AMA) has conducted a new survey (the Physician Practice Information Survey or PPIS) to update the specialty specific practice expense per hour data that is used to develop the practice expense relative value units (PE RVUs).[5] Apparently, the use of the PPIS lowers the PE RVUs for the drug administration service codes, including those most applicable to plasma protein therapies. We are concerned that the resulting payment rate decreases for administering plasma protein therapies will impede patient access to these products.

To address that concern, we ask CMS to establish a transition period for the use of the PPIS. The agency recently took this action when making another change to PE RVUs. When the agency moved from a top-down to a bottom-up approach to calculate direct PE RVUs effective January 1, 2007, the agency established a four year transition to the new PE RVUs resulting from this change because "the shifts in some of the PE RVUs resulting from the immediate implementation of our proposals could potentially cause some disruption for medical practices."[6] In our view, similar disruptions to those that concerned the agency prior to the practice expense methodology change in 2007 are of concern with the proposed changes to PE RVUs resulting from the proposed use of the PPIS. As such, a similar transition mechanism should be utilized if CMS finalizes the proposal to use PPIS in updating the practice expense per hour data.

III. STAKEHOLDERS SUPPORT CMS' CAUTION IN CONSIDERING WHETHER IT IS APPROPRIATE TO APPLY THE WAMP AND AMP THRESHOLD

Under the ASP statute, if the OIG finds that the ASP for a product exceeds the widely available market price (WAMP) or the Average Manufacturer Price (AMP) by a percentage threshold, the OIG informs CMS and the agency, in the next quarter, shall replace the ASP amount with the lesser of the WAMP or 103 percent of the AMP.[7] The

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4 In addition, we note that IVIG may not be included in the CAP since the statute states that payment for IVIG "in 2005 and subsequent years" is based on the average sales price (ASP) system. SSA § 1842(o)(1)(E)(ii). In other words, the buy and bill ASP scheme is the exclusive payment mechanism for IVIG. Moreover, Congress did not intend for IVIG and blood clotting factors to be in CAP. The Conference Report to the MMA states that "competitively biddable drugs and biologicals exclude IVIG products and blood products." Medicare Prescription Drug, Improvement, and Modernization Act of 2003 Conference H.R. Rep. No. 108-391, at 593.

5 74 Fed. Reg. at 33530.

6 71 Fed. Reg. 69624, 69638 (Dec. 1, 2006).

7 Social Security Act ("SSA") § 1847A(d)(3).

OIG must conduct studies, which can include surveys, to determine the WAMP.[8] In the Proposed Rule, CMS proposes to continue to set the WAMP and AMP threshold at 5 percent for CY 2010.[9]

Although we do not oppose this threshold generally, we caution CMS that any decision to apply this statutory provision to the reimbursement of IVIG could exacerbate existing difficulties a fragile patient population is experiencing in attempting to access these therapies in the physician office. We appreciate the statement in the Proposed Rule that the agency is cognizant of the complicated operational issues associated with payment substitutions and thus it "will continue to proceed cautiously in this area and provide stakeholders, including providers and manufacturers of drugs impacted by potential price substitutions with adequate notice of our intentions."[10] We believe that this is a sound approach for the agency to take in this area, and that it is especially appropriate for all plasma protein therapies, given their importance to the patients that take them.

IV. CONCLUSION

Again, we appreciate the opportunity to comment on the Proposed Rule. For the reasons discussed above, we believe that CMS should continue to exclude plasma protein therapies from CAP. Further, we ask CMS to create a transition mechanism for the incorporation of the PPIS data related to the payment for drug administration services. Thank you for your attention to this very important matter.

Respectfully submitted,

Alpha-1 Association
Alpha-1 Foundation
GBS/CIDP Foundation International
Hemophilia Federation of America
Immune Deficiency Foundation
ASD Healthcare
Plasma Protein Therapeutics Association

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8 Id. at § 1847A(d)(1).
9 74 Fed. Reg. at 33623.
10 Id.