Plasma Protein Therapeutics Association

November 22, 2013

Marilyn Tavenner
Administrator
Chief Operating Officer
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Room 445-G
Hubert H. Humphrey Building
200 Independence Avenue, S.W.
Washington, D.C. 20201

ELECTRONIC DELIVERY

Re: CMS-2380-P (Basic Health Program Proposed Rule)

Dear Acting Administrator Tavenner,

The Plasma Protein Therapeutics Association (PPTA) appreciates this opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS) proposed rule establishing the Basic Health Program (BHP) as required by section 1331 of the Affordable Care Act (Proposed Rule).[1] Our comments on the Proposed Rule are intended to ensure that all beneficiaries requiring plasma protein therapies who enroll in standard health plans established pursuant to the BHP have access to the full range of therapies they may need. To that end, PPTA respectfully requests that CMS take the following actions:

- Provide greater clarity on coverage of plasma protein therapies under BHP standard health plans;
- Take steps to ensure that patients enrolled in BHP standard health plans have access to specialty services, drugs and physicians;
- Ensure that a timely and transparent appeals process exists, so that patients enrolled in standard health plans can obtain medically necessary drugs that may not be on a plan's formulary;
- Ensure continuity of care for patients who fluctuate between standard health plans and other coverage.

BACKGROUND

PPTA represents human plasma collection centers and the manufacturers of lifesaving medicinal therapies, including albumin, alpha$_1$-proteinase inhibitor, antithrombin III, blood clotting factors, C1 esterase inhibitor, fibrin sealant, immune globulin, hyperimmune immune globulin, prothrombin complex concentrate and protein

[1] 78 Fed. Reg. 59121 (Sept. 25, 2013).

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C concentrate, from this human plasma.[2] Some PPTA 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." The manufacturer membership of PPTA in the United States (U.S.) currently includes Baxter BioScience, Biotest, CSL Behring, Grifols, and Kedrion.

Because plasma protein therapies are unique, non-interchangeable therapies, patients require unencumbered access to the same therapy to ensure the best possible clinical outcome. Patients receiving plasma protein therapies generally receive regular infusions or injections for the duration of their lives. Any interruption in a patient's administration schedule can result in severe or potentially life-threatening consequences. It is therefore of paramount importance that patients receiving plasma protein therapies have consistent access to the drugs needed for their particular treatment regimens.

Additionally, the majority of plasma protein therapies are approved for marketing in the U.S. by the Food and Drug Administration (FDA) solely for the treatment of one or more rare disease, disorder, or condition. In the U.S., a "rare disease or condition" is generally defined as a disease or condition that affects less than 200,000 people.[3] Most of the rare conditions that require treatment with plasma protein therapies are genetic, chronic, and life-threatening.[4] As representatives of a segment of the biopharmaceutical industry with considerable experience in treating rare diseases, PPTA recognizes that coverage of a wide range of therapies is necessary to ensure that patients receive the best possible treatment. Because of the rare, chronic, life-threatening nature of rare diseases like primary immunodeficiency disease, alpha-1 antitrypsin deficiency and hemophilia and other bleeding disorders, any impediment to treatment is particularly dangerous. Given that the Proposed Rule will affect coverage of plasma protein therapies for low-income, and therefore particularly vulnerable, beneficiaries, PPTA urges CMS to take steps to ensure that these individuals have consistent access to the therapies they need.

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[2] Human plasma is the clear liquid portion of blood that remains after the red cells, leukocytes, and platelets are removed. Due to its human origin, complexity, and richness in therapeutically useful proteins, human plasma is a unique biological material. *See* Thierry Burnouf, *Plasma Proteins: Unique Biopharmaceuticals – Unique Economics*, *in* 7 PHARMACEUTICALS POLICY AND LAW, BLOOD, PLASMA AND PLASMA PROTEINS: A UNIQUE CONTRIBUTION TO MODERN HEALTHCARE 209 (2005, 2006).

[3] *See* 21 U.S.C. § 360bb(a)(2) (2006).

[4] Diseases treated with plasma protein therapies include alpha-1 antitrypsin deficiency, chronic B-cell lymphocytic leukemia, chronic inflammatory demyelinating polyneuropathy (CIDP), hereditary angioedema, hereditary antithrombin III deficiency, protein C deficiency, primary immune deficiency diseases (PIDDs), such as common variable immunodeficiency, X-linked agammaglobulinemia (Bruton's disease), DiGeorge syndrome, Wiskott-Aldrich syndrome, Nezelof's syndrome, severe combined immunodeficiency, graft-versus-host diseases, and bleeding disorders, such as hemophilia A, hemophilia B, congenital fibrinogen deficiency, von Willebrand disease, and factor XIII deficiency. Cytomegalovirus disease associated with transplant patients, hepatitis B reinfection in liver transplant patients, idiopathic thrombocytopenic purpura (ITP), infant botulism, and Kawasaki's disease. Rabies, rhesus incompatible pregnancies, and tetanus are examples of acute rare conditions that are treated with plasma protein therapies.

DISCUSSION

I. PPTA Urges CMS to Provide Greater Clarity on Coverage of Plasma Protein Therapies under BHP Standard Health Plans

Adequate coverage of plasma protein therapies is vital to ensure patient access to therapeutic interventions for rare diseases and conditions. CMS has proposed to adopt existing Medicaid or Exchange standards in specifying coverage requirements for standard health plans offered pursuant to the BHP.[5] Specially, CMS has proposed to require standard health plans to include, at a minimum, the Essential Health Benefits (EHBs) as defined by regulations applicable to plans offered on Exchanges. As a result, BHP standard health plans will need to offer at least the greatest of: (i) one drug in every United States Pharmacopeia (USP) category and class; or (ii) the same number of prescription drugs in in each category and class as the EHB "benchmark plan" under a plan's prescription drug benefit.[6]

While we appreciate CMS's desire to ensure consistency between the BHP and other insurance affordability programs, we are concerned that importing the Exchange's minimum coverage standards for formularies into the BHP program would result in restricted access to the unique plasma protein therapies that these vulnerable beneficiaries may need. Specifically, we are concerned that because the USP categories and classes are defined in a broad manner, a requirement that a standard health plan cover one drug per category and class would fail to ensure adequate coverage to the range of plasma protein therapies that patients need.

For example, there are multiple brands of products in each therapeutic class of plasma protein therapies, including alpha-1 protein inhibitors; blood clotting factors; and immune globulin. Plasma protein therapies are biologics with unique pharmacokinetic and pharmacodynamic properties and thus are not interchangeable. This means that the absorption and metabolization of a plasma protein therapy, as well as the clinical effect of a particular therapy, can vary significantly between products within the same USP class. Additionally, patients on plasma protein therapies have unique and complex health needs, such that even patients with the same condition may respond differently to the same treatment.[7]

Unfortunately, the USP classification system does not capture clinically significant distinctions between plasma protein therapies currently placed in the same class. Therefore, by only requiring standard health plans to cover one drug in each therapeutic class, patients and their health care providers may be forced to use a product that is not the best choice for the patients' needs or to switch to a less appropriate therapy.

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[5] 78 Fed. Reg. at 59147 (proposed 45 C.F.R. § 600.405(a)).
[6] 45 C.F.R. § 156.122.
[7] See "IVIG Medicare Safety: A stepwise Guide to Product Selection and Use," Jerry Siegel, PharmD, FASHP, Pharmacy Practice News, September 2010, p.2

Because the USP classification system was originally designed for use with the Medicare Part D population,[8] it may not adequately reflect the needs of the younger population that will be enrolling in BHP standard health plans. Specifically, the categories and classes of drugs described in the USP classification system may not adequately describe or categorize the drugs, including plasma protein therapies, needed by this demographically different population. Therefore, the needs of these patients may not be sufficiently captured in the USP system.

In addition, there is currently a lack of clarity regarding what therapies may or may not be covered by the medical benefit under a BHP. BHPs should provide a clear understanding of plan benefits and the associated costs. Patients should have access to information regarding how the therapies they are taking are covered and reimbursed under BHP standard health plans. Patients should never be disadvantaged, relative to the cost for lifesaving treatments, such that it would impede access to plasma protein therapies. We therefore have deep concerns regarding a lack of information on sufficient coverage of physician-administered plasma protein therapies for the financially vulnerable population that will be enrolling in BHP standard health plans. We suggest that CMS should not rely on the USP categories and classes when determining coverage for physician-administered therapies under the medical benefit under the BHP

For these reasons, we respectfully urge CMS to require that BHP standard health plans offer coverage of a wide-range of physician-administered drugs as part of the medical benefit in addition to a comprehensive drug benefit. It is vital that patients know which plasma protein therapies are covered and that they will have access to a wide range of therapies to address their unique health needs.

II. PPTA Requests that CMS Take Steps to Ensure Patient Access to Specialty Services and Physicians

We are also concerned that CMS has not taken adequate steps to ensure that BHP standard health plans do not implement benefit designs that could restrict access to medically appropriate care. In particular, we are concerned that the Proposed Rule does not go far enough to protect patients from potentially discriminatory benefit designs. For example, the Proposed Rule does not specifically address any limitations on the use of Specialty Tiers by BHP standard health plans. While we are grateful that the Proposed Rule includes provisions on cost sharing protections for enrollees in BHP standard health plans,[9] we have grave concerns that despite these protections, specialty tiers could have a serious impact on the vulnerable patients who rely on plasma protein therapies as excessive cost-sharing has a disparate impact on chronically ill patients. We remain concerned that the use of high cost-sharing on specialty tiers can result in patients not filling prescriptions, delaying treatment, or opting for less effective therapies.

[8] Medicare Prescription Drug Improvement and Modernization Act of 2003, Pub. L. No. 108-173, 117 Stat. 2085.
[9] 78 Fed. Reg. at 59149 (proposed 45 C.F.R. § 600.520).

We are also concerned that the Proposed Rule does not allow for sufficient oversight of network adequacy to ensure that patients have access to the specialized services that they need. Because many plasma protein therapies are administered by a physician, it is extremely important that patients have access to physicians with the expertise required to effectively treat these complex and rare conditions. The Proposed Rule provides that a state contracting with standard health plan offerers must include provisions in the contracts addressing network adequacy.[10] It also requires that only plans with a network of health care providers sufficient to provide required benefits are qualified to operate as a BHP standard health plan.[11] While we appreciate the desire for CMS to grant states flexibility in running their BHPs, we are concerned that without greater specificity on standards for network adequacy and sufficient federal oversight to ensure that these standards are met, provider networks in BHP standard health plans may not be large enough to guarantee that patients with rare diseases have access to the specialists they need. This could lead to the patient going out-of-network where there is the potential for high cost-sharing or even no coverage at all.

Furthermore, although we appreciate that CMS has proposed to apply the Exchange provision regarding non-discrimination in benefit design,[12] we are concerned that the existence of specialty tiers and inadequate provider networks would result in the violation of this non-discrimination provision. Pursuant to this provision, an issuer cannot adopt a benefit design or implement a benefit design in such a way that "discriminates based on an individual's age, expected length of life, present or predicted disability, degree of medical dependency, quality of life, or other health conditions."[13] Because patients using plasma protein therapies have particularly serious and rare conditions, we believe that a benefit design that limits access to the treatments and the specialists they need would violate this regulatory provision.

We therefore respectfully urge CMS to prohibit the use of specialty drug tiers in BHP standard health plans and to impose specific network adequacy standards to ensure that patients have access to necessary treatments.

III. CMS Should Enact Regulations to Ensure a Timely and Transparent Appeals Process

CMS has proposed that states use the Medicaid appeals process for individuals wishing to challenge BHP eligibility determinations. Although CMS recognizes that eligibility for enrollment in a BHP standard health plan is largely based on eligibility for participation in the Exchange, it argues in the preamble to the Proposed Rule that the BHP eligibility appeals process cannot be modeled on the Exchange appeal process because there is no "independent authority" for a federal level appeal of an adverse eligibility determination, as there is in the Exchanges. CMS also recognizes that some Medicaid programs may delegate Medicaid appeals to the Exchange, and in these

[10] 78 Fed. Reg. at 59148 (proposed 45 C.F.R. § 600.415(b)(1)).
[11] 78 Fed. Reg. at 59148 (proposed 45 C.F.R. § 600.415(a)(3)).
[12] 78 Fed. Reg. at 59147 (proposed 45 C.F.R. § 600.405(d)).
[13] 45 C.F.R. § 156.125.

cases, there will not be overlap between the Medicaid appeals process and the BHP process, because the BHP appeal will not include a federal level appeal. CMS invited comment on this proposal.[14]

We are concerned that if the proposed eligibility appeals process is adopted, there will be a great deal of variation in such processes throughout the country, resulting in beneficiary confusion and a potential for beneficiaries receiving adverse eligibility determinations to fail to exercise their right to appeal such determinations. This confusion would be compounded in states where the Medicaid agency has delegated responsibility for addressing eligibility appeals to Exchanges, because there will not be a single, consistent appeals process for all individuals appealing their eligibility determinations for participation in various insurance affordability programs. We believe that because states will receive federal funds to run BHPs[15], enrollees should be eligible for the same federal-level appeal as all other enrollees in Exchange or Medicaid plans.

Additionally, we are concerned that the Proposed Rule does not include adequate detail on the appeals process for adverse coverage determinations. As previously discussed, BHP standard health plans are required to provide EHBs as defined by reference to Exchange regulations. Pursuant to the prescription drug benefit EHB standard, a health plan must have procedures in place that allow a beneficiary to request and gain access to drugs not covered by the health plan.[16] While we appreciate that this protection will also apply to BHP standard health plans, we do not believe it goes far enough to ensure that patients can receive access to treatments not on the plan's formulary. We urge CMS to adopt more specific guidance applicable to BHP standard health plans to ensure a streamlined, easily navigable and timely appeals process and that patients have access to their medically appropriate treatment during the appeals process. An effective and efficient appeals process is especially important to patients who need consistent and timely access to the specific plasma protein therapy they need. We also request that CMS clarify that enrollees in BHP standard health plans also have the right to challenge adverse coverage determinations for drugs included as part of the plan's medical benefit.

IV. CMS Should Adopt Regulations to Ensure Continuity of Care

Throughout the preamble to the Proposed Rule, CMS emphasizes the way in which the proposed regulations will ensure coordination between the BHP and Medicaid, the Exchange, CHIP and other state-administered health plans. We appreciate and agree with CMS's desire to create as much consistency as possible between these insurance affordability programs. In particular, we appreciate CMS's proposed regulation that will require states to include in their BHP Blueprints how they will ensure coordination of the provision of health care services to ensure continuity of care as enrollees transition in and out of these various programs.[17]

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[14] 78 Fed. Reg. at 59128.
[15] 78 Fed. Reg. at 59133.
[16] 45 C.F.R. § 156.122.
[17] 78 Fed. Reg. at 59148 (proposed 45 C.F.R. § 600.425).

Continuity of care is of critical importance to patients receiving plasma protein therapies. As we previously mentioned, patients receiving these therapies generally receive fixed infusions or injections for the length of their lives, and any interruption in a patient's administration schedule can result in severe or potentially life-threatening consequences. Additionally, due to variations in plan formularies, a patient switching between different plans may need to change the products they use in order to retain coverage of the therapy. Because many plasma protein therapies are not interchangeable, switching between therapies may result in less than optimal clinical outcomes. It is therefore of paramount importance that ensuring continuity of care remain of primary importance.

To that end, we suggest that CMS incorporate more detailed continuity of care standards in the final regulations. In the preamble to the Proposed Rule, CMS describes a number of steps that states can take to ensure continuity of care, including:

- Ensuring that individuals who are undergoing an ongoing course of treatment can continue receiving such treatment and have access to their providers through the duration of their treatment;
- Promote the sharing of data through the use of health information technology;
- Propose access to the same providers and services through BHP available through other insurance affordability programs;
- Use auto-enrollment protocols in BHP, Medicaid and CHIP to maximize continuity with a provider.[18]

PPTA supports these suggestions and requests that CMS incorporate them into the regulations. We believe that imposing more specific requirements on states to ensure continuity of care as individuals transition between insurance affordability programs will benefit all enrollees in BHP standard health plans, and in particular those who need to continue an existing plasma protein therapy regimen.

V. Conclusion

PPTA greatly appreciates the opportunity to provide comments to CMS on its proposed rule implementing the BHP. While we appreciate CMS's desire to create as much consistency as possible between the BHP and other insurance affordability programs, we believe that more specific beneficiary protections should be incorporated into the BHP regulations to ensure patient access to plasma protein therapies. Specifically, we request that CMS take steps to ensure patient access to a wide range of therapies and the health care specialists that these patients need. We also urge CMS to ensure that enrollees in BHP standard health plans have access to a robust and timely appeals process for adverse eligibility and coverage determinations. Finally, we ask that CMS impose more specific requirements on states to ensure continuity of care as patients transition between insurance affordability programs.

[18] 78 Fed. Reg. at 59131.

Please do not hesitate to contact Carrie Fiarman at 443-433-1116 or by email (cfiarman@pptgaglobal.org) if you have any questions regarding these comments.

Sincerely,

Julie Birkofer
Senior Vice President, North America