



STAKEHOLDER REPORT

PPTA held a stakeholder meeting in Washington, D.C., on June 11, 2018. The following stakeholders participated in the meeting, along with PPTA members and staff:

- Alpha-1 Foundation
- GBS|CIDP Foundation International
- Immune Deficiency Foundation
- Cavarocchi-Ruscio-Dennis Associates (on behalf of Alpha-1, Jeffrey Modell Foundation, and National Hemophilia Foundation)

Guests included:

- Richard Manning, Ph.D., Bates White
- Paul Melmeyer, National Organization for Rare Diseases (NORD)

The antitrust statement was acknowledged by all.

PPTA's Senior Vice President, North America & Global Health Policy welcomed participants to the Stakeholder meeting which provides the opportunity for the Association to interact with stakeholder groups and better understand their advocacy priorities and collaborate on patient access to care advocacy.

Access to orphan drugs: A priority for rare disease patients

Paul Melmeyer, NORD's Director of Federal Policy, provided an overview of the Orphan Drug Act (ODA), including incentives at designation and approval, as well as the timeline for approval, noting the steady increase in indications and approvals. Although there is still support for the ODA, it has been gaining attention and receiving more scrutiny among policymakers and the general public. As there are some in Congress who are questioning the ODA, there is a need to continue to educate and raise awareness about rare diseases and how the ODA may impact patients. Therefore, NORD is prioritizing the ODA—this includes organizing an ODA task force, focusing on data collection and dissemination, as well as developing champions to support the ODA. Additionally, Medicare part-B and part-D are being analyzed to determine any potential impact on patient access. NORD welcomes collaboration with PPTA and encourages feedback on materials and advocacy efforts.

Congress and the Administration: Where do we go from here?

Lyle Dennis, Partner, Cavarocchi-Ruscio-Dennis Associates, LLC. shared an outlook on the current landscape and discussed the uncertainty surrounding the Affordable Care Act, noting some Association health plans don't necessarily include essential health benefits; to lose these benefits could be problematic for patients. There has not been an overhaul to drug pricing, but only minor tweaks at this time. In Congress, spending bills are being brought to the floor in one package in order to help the bill pass; bundled bills are harder to veto compared to individual bills. Looking ahead at potential impacts on patient

access, there are mid-term elections in November and a number of republican members not running for re-election, which may change the landscape.

Positioning Bates White economic analysis with policymakers

In order to highlight the value of plasma protein therapies (PPT) and differentiate from traditional pharmaceuticals, PPTA retained Bates White consulting firm to conduct an economic analysis of the PPT sector. Bates White developed a White Paper, "[Key Economic and Value Considerations in the US Market for Plasma Protein Therapies.](#)" Richard Manning, Ph.D., commented that the PPT market is not well known, especially how it differs from traditional pharmaceuticals, which includes the small patient populations, rare medical conditions, and high value that they provide to patients. There is a growing use of therapies, particularly IVIG, albumin, factor VIII, and with more diagnosis and indications, consumption will continue to grow. To perform the analysis, Bates White reviewed evidence on the cost effectiveness of PPTs and the value they've provided for patients and the impact therapies have had on their lives. It was expressed that sharing true stories of patients who use PPTs with policymakers is very compelling. The white paper also highlights the overall savings to the health care system, the added economic value by creating manufacturing and collection center jobs as well as the economic contributions from donor compensation. Additionally, the cost structure differs tremendously between the manufacturing of pharmaceuticals and PPTs. While the White Paper provided data points for policymakers to better understand the uniqueness of the PPT sector, reimbursement challenges remain. PPTA will continue to leverage the White Paper by circulating to key thought leaders and referencing in relevant documents.

PPTA Patient Access Advocacy Priorities Overview

Federal activities

PPTA shared an overview of the May Capitol Hill Fly-In which included participation from patient advocates and member companies. The Fly-In provides an opportunity to educate decision-makers on the value of PPTs and how they differ from other medicines. In addition to supporting policies that provide access to PPTs, the key messages focused on the "four pillars":

- No exclusion for pre-existing conditions
- No lifetime or annual caps
- Ability for dependents to remain on parents plans until age 26
- Keeping out of pocket expenses manageable

State activities

At the state level, there are efforts to improve the plasma donation process, particularly in New York and Washington. Using more sophisticated and illustrative materials help explain the nuances of the complex PPT sector when meeting with policymakers.

Communications

Social

Last year PPTA launched three social media channels providing an opportunity for direct engagement and communication with congressional staffers. PPTA encourages members and patient organizations to follow, like, and share our posts to help messages reach a wider audience. Learn more on [LinkedIn](#), [Facebook](#), and [Twitter](#).

Media

"How Is Your Day?"
Following the heightened number of negative news coverage around the plasma industry, particularly focusing on the collection of plasma, PPTA launched its newest initiative—"How Is Your Day?"—which strives to raise awareness about the value provided by PPTs and highlight the differences between PPTs and traditional pharmaceuticals. For this initiative, PPTA features the personal stories and images of plasma donors and patients who use PPTs in order to help policymakers better understand the uniqueness of the PPT

sector. Donors and patients may submit their stories via the “How Is Your Day?” [website](#). PPTA invites you and your colleagues to become a part of the initiative by liking, following, and sharing our messages on [Facebook](#) and [Twitter](#).

Other business

The American Plasma Users Coalition (A-PLUS) representatives shared that the commitment from patient organizations remains strong. The A-PLUS is establishing its key priorities. A-PLUS plans to hold a consensus meeting in October to continue discussing priorities and reflecting on areas of concentration, such as expanding their focus to include plasma collection issues. A-PLUS will continue to keep an open dialogue with the Association.

Stakeholder meeting presentations

- [Access to Orphan Drugs: A Priority for Rare Disease Patients](#)
- [Key economic and value considerations in the US market for plasma protein therapies](#)

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