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Executive Summary Advocacy Priorities

Eight patient organizations, industry representatives including North America Board members and PPTA staff met in Washington, DC on February 7, for the first Stakeholder meeting of 2012. The meeting was well attended with representatives from:

- World Federation of Hemophilia
- GBS/CIDP Foundation International
- Alpha-1 Association Foundation
- National Hemophilia Foundation
- Immune Deficiency Foundation
- Hemophilia Federation of America
- Jeffrey Modell Foundation
- Committee of Ten Thousand
- Platelet Disorder Support Association
- A-PLUS

Attendees shared priorities ranging from health care reform implementation to products and sites of care. Much remains unknown with state health exchanges and essential benefits and there is much work to be done.

The purpose of this meeting was to:

- Share advocacy and organizational priorities
- Facilitate greater understanding of key concerns and goals
- Identify opportunities for alignment and collaboration on key patient access issues

Participants engaged in lively discussions centered on shared priorities:

- Increased awareness of rare diseases and coverage issues
- Formulary restrictions that limit product choice
- Health plans that attempt to implement "fail first" therapeutic protocols
- Medicare/Medicaid cuts to include physician reimbursement
 - Eliminating or further reducing Average Sales Price (ASP) of drugs, making it less attractive for physicians to want to treat patients
- Increased R&D
- Biosimilars
- Ending first dollar Medigap coverage for Medicare Part B
- Health outcomes research
 - Patient Stakeholders in Research Design Decision-Making
- Medicare moving Part B drugs to Part D (stiff co-insurance payments/plasma products specialty tier)
- Protecting patient access during health care reform implementation: affordable care, and access to all therapies, providers and sites of service
- Restoration of Centers for Disease Control and Prevention (CDC)

- funding for public awareness
- Augmenting National Institutes of Health (NIH) funding for rare disease research
- Early diagnosis and treatment
- Medicaid managed care expansion
- Newborn screening for severe combined immune deficiency (SCID)
- Educating and training grassroots advocates

In addition, the group identified several opportunities for collaboration:

- Threats from Medicaid managed care expansion, particularly with the bleeding disorders community
- Health Care Reform Implementation
 - Essential Health Benefits
 - State Health Exchanges
 - CER/PCORI—opportunity to inform and influence
- Specialty Tiers
- Consumer led legislative initiatives
- Meeting with National Association of Medicaid Directors (NAMD)
- State insurance exchanges, essential health benefits, comparative effectiveness of treatment requirements, allowance for biosimilar biologics and more

PPTA

In addition, PPTA staff highlighted North America programs including the Patient Notification System and the data program. The federal and state affairs 2012 advocacy agenda was also noted.

Patient Notification System (PNS)

In 1998, in collaboration with consumer groups, PPTA developed a system to provide patients with confidence in plasma protein therapies. Initially, funded by industry, the PNS has been adopted in Canada and boasts a long standing record of safety and is considered by many to be a model public health system. The Patient Notification Working Group (PNWG) is composed of industry and patient representatives who guide direction. The system allows for recalls and other manufacturer notices to be distributed via phone, fax and express mail. The system is outsourced to Stericycle to ensure patient confidentiality. Stakeholders were asked to help increase the number of registrants.

North America Data Program

The North America data program was launched in 1998 in response to requests from the federal Advisory Committee on Blood Safety and Availability (ACBSA). Since 1998, patients, their family members and treaters have relied on the information available on the PPTA website to give them confidence and peace of mind that their therapy is available. Stakeholders were reminded that the temporary suspension of data reporting has ended, and that data for October 2011 is now on the PPTA website. One modification mentioned to the group was that the data now reflects a three month lag. Copies of the February 3, letter to Stakeholders were distributed, in addition to copies of PPTA's comments on FDA's October 2011 drug shortage report, which described over a decade of success with the data program.

Capitol Hill Fly-In

PPTA invited stakeholders to participate in the Annual Capitol Hill Fly-in on Wednesday, May 9. The Fly-in provides an opportunity to inform and educate legislators and their staffs and ensure patient access during health reform legislation. A dinner will be held on May 8.

National Association of Medicaid Directors

PPTA's recently met with the NAMD in January to discuss increased reliance upon managed care, mandatory managed care and opportunities for dialogue. The NAMD staff is interested in meeting with Stakeholders to better understand the unique patient access needs of their communities.

Patient-Centered Outcomes Research Institute (PCORI)

PCORI recently released its research agenda and is open to public comment and feedback on the direction of its research priorities. The agenda is quite

broad and does not discuss rare diseases nor how the Rare Disease Advisory Panels will be constructed or operate. This may be a collaborative opportunity moving forward. The group discussed the possibility of a letter to the PCORI board to discuss the unique nature of the diseases treated with plasma protein therapies and the importance of patient access. The possibility of a joint meeting was also discussed.

Conclusion

PPTA sincerely appreciates the participation and attendance of industry and patient organizations. If you have any questions, or require additional information please contact Association staff.

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We hope to see you at our next Stakeholder Meeting which will be in late spring. An announcement and invitation will be sent as soon as a date is confirmed.

[Stakeholder Presentations](#)

If you have any queries or would like to add a recipient to our electronic mailing list, please contact the association office.

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