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## Overview

Representatives from U.S. based consumer organizations, industry, including North America Board members and PPTA staff met in Washington, DC on January 22, for the first Stakeholder meeting of 2013. The meeting was well attended by the following stakeholders:

- Alpha-1 Association/Alpha-1 Foundation
- Committee of Ten Thousand
- GBS/CIDP Foundation International
- Immune Deficiency Foundation
- National Hemophilia Foundation
- Hemophilia Federation of America
- Jeffrey Modell Foundation
- Patient Services, Inc.
- A-Plus

PPTA's Senior Director, Legal Affairs referenced the PPTA Meeting Guidelines and reviewed the antitrust compliance rules for meetings.

PPTA's Senior Vice President, North America welcomed participants to the annual Intake meeting which provides the Association and industry with an opportunity to listen to stakeholder advocacy priorities. Access to therapies in all sites of service frames the Association's advocacy priorities and PPTA looks for alignment on issues and opportunities for collaboration.

Several common concerns emerged:

- Participation in the Food and Drug Administration's (FDA) Patient-Focused Drug Development
- Outreach to new Members of Congress
- Educating and training grassroots advocates
- Implementation of the Affordable Care Act (ACA)
- Focus on the need for data to support patient access advocacy
  - Evidence based guidelines ([www.guidelines.gov](http://www.guidelines.gov))
- Medicaid Expansion
  - State Health Exchanges
  - Essential Health Benefits – plan management
  - Coverage & formularies
- Medicaid Managed Care
- Newborn screening (SCID)

## Stakeholder Plans and Priorities

In addition, stakeholders articulated specific plans and priorities:

### **Alpha-1 Association/Alpha-1 Foundation**

- FDA Patient-Focused Drug Development
- Monitor Patient-Centered Outcomes Research Institute (PCORI) with regard to rare disease panels
- Leverage evidence-based augmentation therapy literature

#### ***A-Plus***

- Joint comments on ACA implementation, Essential Health Benefits, Biosimilars, State Health Exchanges, and Independent Payment Advisory Board (IPAB)
- Educational webinars in conjunction with Georgetown Health Policy Institute to train grassroots advocates
- Meeting with (PCORI) and National Organization on Rare Disease (NORD) to discuss expert rare disease advisory panels and comparative effectiveness research

#### ***Committee of Ten Thousand***

- U.S. prevalence of Hepatitis E virus, susceptibility thereto among immune suppressed populations, and lack of clarity concerning the ability of current purification techniques to eliminate the virus

#### ***GBS/CIDP Foundation International***

- Advocate for increased federal research funding from the National Institutes of Health (NIH) and expand the portfolio to include the National Institutes of Allergies and Infectious Diseases (NIAID), the National Institute of Neurological Disorders and Stroke (NINDS) and NIH Office of Rare Disease Research (ORDR)
- Create a national network of local advocacy volunteers and increase public and professional awareness leveraging network and resources of the Centers for Disease Control (CDC)
- Expand footprint on Capitol Hill and reach out to key legislators with annual advocacy day to commence this March

#### ***Hemophilia Federation of America***

- Increase community engagement at the federal and state level
- Consumer education on key policy issues for bleeding disorders community via redesigned website, resource toolkits, webinars, social media video, a Capitol Hill advocacy day in March and Congressional Awards Reception
- Improve the awareness of HFA among state-based hemophilia organizations, related industry and other patient advocacy organizations

#### ***Immune Deficiency Foundation***

- Implementation of the Medicare IVIG Access Demonstration Project
- IDF Payer Policy Task Force comprised of health care providers, payers and stakeholders to build consensus on safe, cost efficient acceptable standard of care. Results to be published in white paper and other publications
- Newborn SCID Screening—in effect in 13 states, Navajo Nation and Puerto Rico, 16 others have voted to authorize
- Contingency planning to assure on-going Ig supply for patients with primary immunodeficiency diseases in the event of an Ig shortage

#### ***Jeffrey Modell Foundation***

- Oppose discretionary budget cuts and support for continued funding and expansion of the PI education and awareness program
- Implementation and expansion of newborn SCID screening

- throughout the states
- Partnership with Gates Foundation to eradicate polio worldwide utilizing the Jeffrey Modell Centers Network

### ***National Hemophilia Foundation***

- Education of grassroots advocates, payers, local pharmacy boards and others with specific focus on MASAC 188 recommendations
- Protect federal program funding which faces a “triple threat” from the debt ceiling bill, sequestration and major changes to HRSA’s Maternal Child Health Bureau
- Support reintroduction of Representative David McKinley(R-VA) bill H.R. 209 on specialty tiers and Representative Carolyn McCarthy (D-NY) legislation on Von Willebrand disease

### ***Patient Services, Inc.***

- Medicaid expansion in Virginia
- Move patients into private insurance with premium assistance
- Partnering with state level organization on legislative and payer issues

## **PPTA Presentations**

### ***Patient Notification System (PNS)***

Developed in concert with patient organizations in 1998 and managed by an independent third party vendor, this model system provides FDA approved language and notification of therapy withdrawals or recalls. It is considered a superior system that includes all manufacturers, not just PPTA members. It provides email, phone and fax notifications of its 6500 enrolled users and every effort is made to provide notification within 24 hours. There was one recall in March 2012 and there have been none since.

### ***North America Data Program***

Developed in 1998 as an drug shortage preparedness mechanism, the Data Program provides intelligence and data in times of real shortage and allows PPTA member companies to respond to product availability issues. FDA receives both company and aggregate data and which is published with a three-month lag on the Association’s website.

### ***International Quality Plasma Program (IQPP)***

IQPP is the cornerstone of industry’s commitment to safety and quality. Nine core standards work in conjunction with regulatory frameworks in both the U.S. and Europe. Most importantly, the [National Donor Deferral Registry](#) (NDDR), the Qualified Donor Standard and the Viral Marker Standard add measurable safety to plasma. The standards enhance regulatory requirements of the Food and Drug Administration (FDA) and European Medicines Agency (EMA), as well as regulations by the Occupational Safety and Health Administration, the Department of Labor and state agencies. Certification is achieved and maintained by rigorous independent audits. Over 400 centers in the U.S. and over 50 in Germany, Austria and the Czech Republic are IQPP certified. In addition, PPTA is working to define global applicability and identify alternatives with equivalent outcomes where national laws may require modification.

### ***International Plasma Awareness Week***

PPTA, together with member companies will launch **International Plasma Awareness Week** (IPAW), October 13-20 to raise global awareness about source plasma collection, to celebrate and recognize plasma donors and raise awareness about plasma protein therapies. Stakeholder groups are invited to participate. Interested organizations should contact [Lisa LoVullo](#), 443.458.4669.

### ***Quality Standards of Excellence Assurance and Leadership (QSEAL)***

QSEAL voluntary standards add measurable safety and quality at the fractionator level. Proposed changes to QSEAL include: a new specification for recovered plasma, a new standard on controls on

specification for recovered plasma, a new standard on controls on incoming plasma, and revisions to standards for NAT testing and intermediates, which were posted for public comment. These will become effective pending final Board of Directors approval and auditor education.

### **Federal Affairs Priorities**

- Patient access, Federal reimbursement specifically Medicare Part B
  - Leverage the Moran Company analysis
- Annual Pharmaceutical Fee
- Support Consumer Led Initiatives
- Comparative Effectiveness Research
- Essential Health Benefits Implementation

PPTA invited stakeholders to participate in the Annual Capitol Hill Fly-In on Wednesday, May 8. The Fly-In provides an opportunity to inform legislators and their staffs regarding the importance of patient access to plasma protein therapies for rare disease patients. A Capitol Hill reception will be held on May 7.

### **State Affairs Priorities**

- Open access to plasma protein therapies
- Assist patient organizations with state level advocacy efforts
- Medicaid expansion
- Specialty Tiers
- SCID Newborn Screening
- Standards of Service/Advisory Boards
- Medicaid MCOs Preferred Drug Lists

The [State Patient Access Coalition](#) (SPAC), a coalition of manufacturers and specialty pharmacies is working to address threats to blood clotting factor access. The coalition supports patient access to all blood clotting factors, from all qualified specialty pharmacies, and in all sites of service.

### **Health Technology Assessments**

Professor Albert Farrugia made a compelling case for engaging with patient groups to generate data when health technology assessments (HTAs) are used to inform decision makers on therapeutic choice. HTAs are being used to justify barriers to access, but can support rare disease treatments if careful attention and commitment is given to using all the evidence including patients views. This is particularly crucial in the assessment of health related quality of life, which is used to generate the key outcomes in HTAs. All HTA systems have the same goal—to compare, assess and recommend policy about choice and payment of therapies. HTAs are used widely in taxpayer funded systems in Europe, Australia and Canada and particular aspects such as cost-effectiveness analysis are also being used by private payers. In the USA the predominant form of government-sponsored HTA is Comparative Effectiveness Research (CER), which through the Patient Centered Outcomes Research Institute (PCORI) is funding many projects comparing health interventions. It is important that the rare diseases community engages with this process through inclusion in PCORI's panels to ensure that the particular needs of rare disease patients are not overlooked.

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