PPTA Stakeholder Report

March 2011
Vol. 9, Issue 1

PPTA would like to recognize and thank all of the consumer organization representatives who attended this first Stakeholder meeting of 2011 held on February 15 in Washington, D.C. Following precedent, this “In take” meeting gives the patient communities, manufacturer members and PPTA the opportunity to learn more about the advocacy and organizational priorities of each of the groups for the coming year, facilitates greater understanding of key concerns and goals across the community of consumers, and encourages a healthy exchange about how we can work together in furtherance of patient access. Stakeholders universally expressed that they appreciate PPTA’s role as an information resource, as well as the access to and working relationships with Association staff. Stakeholder representatives from the primary immune deficiency, alpha-1 antitrypsin deficiency, Guillian-Barre/chronic inflammatory demyelinating polyneuropathy, platelet disorders and hemophilia communities were present, as was a representative of the National Organization for Rare Disorders (NORD) as an invited guest.

Several themes were identified in each of the presentations from the consumer community including concerns over cuts in President Obama’s 2012 proposed budget; concerns over partisanship with respect to repealing or reforming the health care law; the need to ensure that access to specialty care and the full range of plasma protein therapies is reflected in Essential Benefits packages; uncertainty over the implementation of state exchanges and how that process will impact patient access; the growing need and creativity with which states are seeking to reduce costs associated with health care; threats of “fail first” and preferred drug list policies for the use and reimbursement of biologics; constant vigilance of blood components and plasma products; the need for more data on patients and their treatment; and the need and opportunity to inform and educate federal and state lawmakers about specifics of their respective diseases, required therapies and need for specialized care.

Additionally, the Immune Deficiency Foundation (IDF), which is getting ready to celebrate its 30th anniversary, informed the group that they are working on a program for Newborn Screening Initiative for severe combined immune deficiency (SCID) and continue to advocate for the IVIG Access Medicare demonstration program and are working toward collecting additional data on the gaps that exist with coverage of ancillary services associated with home infusion of IVIG. IDF also is preparing for its lobby day on May 26. In 2011, IDF will continue to strengthen its grassroots advocacy network and focus the PID community on protecting their access to immune globulin therapies. View slides.

The GBS/CIDP Foundation International is working toward establishing more Centers of Excellence – six such centers already are in existence for treating these extremely rare and neurological disorders. Further, it was reported that the Foundation conducted a second survey of CIDP patients, receiving about 1,300 responses. Temple University is currently looking at compiling result, working with the Foundation’s medical director. In 2011, GBS/CIDP will focus on patient education, developing state level advocates and prioritizing early diagnosis and treatment. View slides.

The Alpha-1 Foundation and Alpha-1 Association representative related that

they are working on a genetics project with respect to alpha 1 and two other conditions. The Association continues to work on issues concerning respiratory therapists and is getting ready to join that group for its lobby day on March 8. Additionally, it was reported that the Alpha-1/FDA liaison group has been reinstituted and has met once thus far. The Alpha-1 community is also concerned with proposed budget cuts at NIH and participates in the "Friends of the NHLBI" group. It was also noted that the Foundation & Association are also supportive of risk tolerance initiatives and are participating in discussion groups.

The Committee of Ten Thousand (COTT) expressed concerns about blood safety and the need to carve blood products out of the biosimilars process. COTT continues to be very active on Capitol Hill and is mindful of CDC's new approach to hemophilia care. COTT is concerned about the impact of state exchanges on hemophilia care. The federal role in setting up the structure of state exchanges was discussed, particularly federal involvement in setting essential benefits. COTT continues to support screenings of the "Bad Blood" documentary film. It was mentioned that at this time a June PBS screening of the film appears likely.

The National Hemophilia Foundation (NHF) reported that it is focusing on payer education this year. NHF expressed concern over cuts in the President's proposed 2012 budget that would affect hemophilia care and also mentioned that another well-attended lobby day will be taking place later in the week. NHF also expressed the need for additional patient data to assist with their advocacy any to help further explain the specialized treatment required for hemophilia care, and referenced a white paper in development on the treatment of hemophilia and why it is vastly different from patient to patient and a survey of patients conducted in partnership with the Hemophilia Federation of America. The NHF also focused this week on during its "Washington Days" on providing training to advocates on how to reach out to state Medicaid and Pharmacy Directors and insurance commissioners to provide information on perspective on treating the disease. NHF also shared their plans to conduct a second survey of their community in April.

The Jeffrey Model Foundation (JMF) also is working on raising awareness for the need for newborn screening for SCID, reporting that eight states have adopted measures to screen newborns thus far, resulting in 1.5 million of 4 million annual births in the U.S. being screened this year. The Foundation also is concerned with potential cuts to CDC that will eliminate federal funding for its public awareness campaign for the signs of PID. The JMF is preparing for its 25th anniversary and the World Immunology Conference this summer in New York. The Foundation continues to focus on education and awareness. They also are looking at software tied to ICD-9 codes that will trigger a hospital that based on a patient's frequency of visits and diagnosis of certain illnesses; he or she should be tested for a PID.

The American Plasma Users Coalition (A-PLUS) continues to leverage political resources to advocate on issues that affect the community of plasma protein therapies users. The group is monitoring implementation of the new health care law and was one of the few consumer groups to submit comments on the Essential Benefits Packages in development. The group also reported that it submitted comments to the FDA on its concerns for biosimilars and continues to monitor safety concerns including the MSM deferral issue with respect to blood and plasma donation. View slides.

The National Organization of Rare Disorders (NORD) attended the meeting as an invited guest to share information about the group's 2011 priorities. Key issues include: educating members of Congress the access needs of rare disease populations; participation in a Risk Working Group to address the need to balance risk with bringing drugs that treat rare conditions to market more quickly; the need to reimburse medical foods, which often are required to treat rare diseases; addressing conflict of interest when conducting clinical trials and the need to treat this differently for rare disease treatment development; and ways to provide incentives to pharmaceutical companies to continue the research and development needed to bring drugs to market to treat rare diseases.

The Hemophilia Federation of America (HFA) highlighted the importance of

access to safe and effective blood clotting factors to their community. HFA stressed the need for data collection spearheaded by patient-based organization. HFA will continue to focus in 2011 on empowering the bleeding disorders community to have the necessary tools to advocate for access to the full range of therapies available. The Administration's proposed 2012 budget is of concern to HFA, particularly the proposed CDC reductions that would negatively impact surveillance programs. With regard to state issues, HFA will be monitoring cuts to state Medicaid budgets and their impact on access to therapies and access to care. It was noted that since states are struggling under spiraling Medicaid costs, increased reliance on tiering of drugs needs to be watched as it often is an obstacle to access. HFA is monitoring the evolving state exchanges and noted the importance of influencing the essential benefits package early on in the process.

The World Federation of America (WFH) reported on its 2011 global advocacy agenda and highlighted its World Hemophilia Day (April 17) theme of "Be inspired, get involved in Treatment for All." It was mentioned that the WFH takes a different view of issues because of the global nature of the organization and the importance to take into account global blood safety and availability issues. An example of this was offered relating to biosimilars—one country was mentioned that allows a blood clotting factor therapy on the market without requiring clinical trials. The European requirements for pediatric licensing were mentioned and the opinion was offered that this could result in a slowing down of pediatric trials. Comparative effectiveness research in Europe and the use of health technology assessments was also discussed. WFH is considering the "science side of advocacy" and stressed the importance of meaningful data.

The Platelet Disorders Support Association (PDSA) will focus in 2011 on strengthening its web site content by adding more patient stories to its "Expressions of ITP—Inside Stories" page. This patient based information is very motivational to the community and informative to new patients and their families. In 2011, PDSA will also be branching out to leverage social media outlets such as facebook and YouTube. The reoccurring theme of the importance of early diagnosis will continue to be a priority of PDSA.

Additional discussion by Stakeholder groups surrounded issues including safety and efficacy of biosimilars and comparative effectiveness research and health technology assessments, which continue to be identified by consumer organizations as issues to monitor.

PPTA staff then discussed federal and state advocacy priorities and issues with the group. Jay Greissing, Senior Director, Federal Affairs, described plans for an upcoming Congressional staff briefing to provide information and education about the special nature of plasma protein therapies, and the need to preserve access to therapies. The staff briefing is an opportunity for PPTA and consumer groups to partner by providing compelling, personal stories about the diseases they have (or their children have), how they are treated and how access to plasma protein therapies affects their health. PPTA will also describe the vein-to-vein story of plasma protein therapies and discuss the uniqueness of the therapies and the patients they treat. Stakeholders expressed strong support for the briefing and for the opportunity to participate. The tentative date for the lunch briefing is March 31, 2011. More information will be provided to Stakeholders as further details become available. Mr. Greissing also let consumers know about the Association's annual Fly-in, which will take place on May 11. As always, consumer representatives are encouraged and welcomed to attend. Additional details will be forthcoming. View slides.

PPTA's State Affairs Director Bill Speir discussed some of the most pressing developments with the implementation of the health reform law, the serious budget crisis that many states are facing, proposed changes in Medicaid programs to contain costs that may negatively affect access to plasma protein therapies. Mr. Speir discussed several states in particular including the Arizona Medicaid waiver that would would eliminate Medicaid eligibility for optional adults. He spoke as well about the proposal to move to 100 percent managed care in Florida and proposed elimination of the medically needy. Mr. Speir reiteratied that many states will be holding hearings on health care proposals and that it presents consumer advocates and medical professionals with an opportunity to inform lawmakers and other key opinion leaders about

the need to preserve patient access to plasma protein therapies. Further, PPTA will work with the A-PLUS on the issue of state exchanges to develop a strategy to ensure patient access to plasma protein therapies. View slides.

After discussion of consumer group advocacy priorities and potential threats to access to therapies, PPTA President & CEO Jan M. Bult discussed the value and importance of the industry's voluntary standards programs for the collection and manufacturing of source. He explained how the two standards programs (IQPP and QSEAL) work in concert with one another and with established federal and international regulatory guidelines to promote the highest levels of safety and efficacy in plasma protein therapies and how the standards programs offer another level of confidence to consumers. Mr. Bult also discussed the North America data collection program describing its history from its inception in 1998 to the present. The Stakeholders noted their appreciation of the data collection program particularly because it is relied upon as a source of information on the availability of plasma protein therapies. The data program and standards were described as "exceptional programs" and again the "value of the data collection program" to patient groups was stressed. There was discussion regarding the status of the QSEAL recovered plasma specification. Mr. Bult explained that the specification will be put out for another round of public comment and he encouraged Stakeholders to once again feel free to submit comments during this open process.

As always, PPTA appreciates the participation of the consumer organizations and members companies in these valuable Stakeholder meetings and will provide information about the next meeting once the Advisory Committee for Blood Safety and Availability calendar becomes available.

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

Forward to a Colleague

Annapolis Office
147 Old Solomons Island Road
Suite 100
Annapolis MD 21401 USA
tel: +1 202 789 3100
fax: +1 410 263 2298
ppta@pptaglobal.org

Washington Office
Washington Harbor
3050 K Street NW Suite 400
Washington DC 20007 USA
tel: +1 202 789 3100