

Stakeholder Report

On September 3, Stakeholders met in Washington, D.C. to discuss implications of federal health care reform legislation being hammered out in Congress. Representatives from national consumer organizations and PPTA member companies participated in the meeting. After a welcome by PPTA, including an acknowledgement of the accomplishments of the Plasma Users Coalition, which has been meeting regularly to discuss the impact of sweeping health care reform on their patient communities, guest speakers Diane Dorman, vice president of Public Policy, National Organization for Rare Diseases (NORD) and John Ford, Counsel, Sidley Austin LLP were introduced.

Diane Dorman began her overview of NORD's health reform priorities by indicating support for current health care reforms. She discussed follow-on biologics; supporting lifting lifetime insurance caps; monitoring the evolution of comparative effectiveness research; guaranteed issue and renewal of health insurance; and phasing out the donut hole in Medicare prescription drug plans. Ms. Dorman stated that NORD is taking a limited role when it comes to reimbursement issues. Ms. Dorman stated that she originally believed that CER would have little impact on orphan drugs, but upon further consideration realized that for diseases like hemophilia, alpha-1 antitrypsin deficiency and others, there may be an adverse impact on patient access to therapies and expressed her

support for establishing a rare disease advisory panel and an ombudsman housed most appropriately in the Office of Rare Disease Research at the National Institutes of Health. She commented that "CER could have an adverse impact on access should companies be required to change their labeling based on the findings of CER given the fact that 80 percent of rare disease patients are treated off-label and are already being denied the care they need based on current labeling."

Turing her attention to follow-on biologics, Ms. Dorman recalled that NORD was the first groups to hold a forum on the topic in 2003, and that NORD supports Chairman Waxman's direction of three-to-five years of data exclusivity believing this is an incentive to develop orphan products. Couching her presentation (see attached) with the *111th Congress: The Good, Bad and the Ugly* with health care reform, Ms. Dorman pointed to paving the way for greater Food and Drug Administration flexibility; decoupling of the biosimilars review and approval processes and patent litigation; timely resolution of patent disputes; and prohibiting frivolous lawsuits. According to Ms. Dorman, winding up with something is better than nothing in the way of health care reform.

Ms. Dorman pointed to several industry challenges, chiefly regulatory hurdles and adequate reimbursement, and burdens to patients such as length of time to diagnose, financial burdens, the use of QALYs (quality adjusted life years) in

insurance calculations and the introduction specialty tiers. Referring to a recent meeting she had with pharmacy benefit managers, Ms. Dorman paraphrased that they already are rationing high-cost products. She concluded that with all of the competing interests the challenge is to find balance to ensure patient access to lower cost, lifesaving therapies and to continue to foster industry innovation.

John Ford opened his remarks by asking the group to come up with a bill that can get 218 votes in the House and 60 in the Senate—with no other guidelines other than it must pass both Chambers—as if hired by President Obama, House Speaker Pelosi and Senate Majority Leader Reid to accomplish this singular goal. He continued, saying that 19 Democrats have indicated they would not sign any health care reform bill unless it included certain things that would never make it into a bill. He illustrated his point with two “reality checks.” One, if you can’t get people to realize that the concept of “death panels” was an outright lie, how can you get people to discuss the real truths and basic concepts? And, two, Rep. Dingell had a Town Hall meeting that drew 600 people, well over the expected 200, with the majority opposed to health care reform—prompting Mr. Ford to ask, “if a well-established and respected Member can’t sell reform, who can?”

Mr. Ford used the exercise as a springboard to discuss the state of the health care reform legislation, how public opinion and politics were at play throughout the August recess, the state of the public option and the possibility and consequences of a reconciliation bill.

Each national consumer representative in attendance discussed the six Principles for

Health Care Reform and the Rare Disease Community that were developed by the Plasma Users Coalition and sent to Senate Finance Committee Chairman Max Baucus in early August. The principles cover:

- 1) Private Insurance Market Reforms
- 2) Affordability
- 3) Access to Specialists
- 4) Access to Therapies
- 5) Comparative Effectiveness Research
- 6) General Recognition of Rare Diseases

PPTA’s Federal Affairs Director discussed top issues that the Association and members are focused on in health care reform legislation, explaining industry’s position on the proposed expansion of the 340B Drug Pricing Program; the proposed increase in the Medicaid Rebate percentage for biologics; follow-on biologics, including support for 12 years of data exclusivity proposed in the Eshoo bill and the Hatch-Enzi-Hagan amendment; and comparative effectiveness research including discussion of the Conrad-Baucus legislation introduced June 9 that includes a permanent advisory panel on rare diseases for which PPTA and members advocated.

The meeting concluded with a discussion of the advocacy and communications needs of the Plasma Users Coalition, with a request for the group to propose a plan for additional support and assistance with communications, including advertising and message coordination. The group discussed the need to organize a Congressional briefing and to identify advocates in key states that would share their stories.

Note: Please see the following links to material discussed at the meeting:

[HFA Advocacy Video](#) and [Article on Raising Awareness](#)

[Myth vs. Fact on Health Care Reform](#)
(House Ways & Means Committee)

[The Real Truths About Health Care Reform Proposals Before Congress](#)
(National Health Council)

Attachment: Presentation, Diane Dorman