

Health Policy Update

Federal Action

Medicare Patient Access

Health Care Reform

Congressional health care reform initiatives are currently being debated at a torrid pace. The Senate Health Education Labor and Pensions (HELP) Committee continues to mark-up the Affordable Health Choices Act while the Senate Finance Committee is slated to begin their mark-up of its health care reform legislation in the weeks immediately after the July 4 congressional recess. In the House of Representatives, the 'tri-committee' health care reform discussion draft legislation that includes the Committee on Energy and Commerce, Committee on Ways and Means and the Committee on Education and Labor are expected to mark-up its bill in the coming weeks as the key committees finish-up numerous hearings on some of the bigger aspects of the Democrat scribed health reform draft bill such as the public health insurance plan option, physician payment reforms and key financing aspects of the unprecedented health care expansion bill.

The association is continuing to advocate for the inclusion of several provisions on issues of importance to PPTA in the various health care reform proposals including comparative effectiveness research, follow-on

biologics and consumer led initiatives such as eliminating lifetime insurance caps and IVIG access via the Medicare Patient IVIG Access Act, H.R. 2002 and S. 701.

Comparative Effectiveness Research

The marker legislation coming from the Senate Finance Committee, S. 1213, introduced by Senators Baucus (D-MT) and Conrad (D-ND) is a great improvement on S. 3408 of the 110th Congress. This new legislation incorporated PPTA's recommendation in its March 2, 2009 letter to Senators Baucus and Conrad regarding a permanent advisory panel on rare diseases. Specifically, when there is a proposed CER study for a rare disease, the new bill would require the Patient-Centered Outcomes Research Institute to "appoint an expert advisory panel for purposes of assisting in the design of such research study and determining the relative value and feasibility of conducting such research study."

PPTA had recommended in section 4 of the Appendix of its letter (see attached hyperlink to document): "(C) Permanent Advisory Panel for Rare Disease.-For the purpose of establishing a comparative effectiveness research agenda for rare diseases, determining the relative value and feasibility of conducting such research on a particular rare disease, and advising the Institute on designing studies, where

appropriate, for rare diseases, the Institute shall appoint a permanent advisory panel that shall include representatives of patients that suffer from rare diseases and physicians with clinical experience in treating rare diseases."

For this rare disease panel established in the reintroduced Baucus-Conrad bill, as well as the expert advisory panel for primary research established in the bill, the individuals appointed must include: practicing and research clinicians, patients, and representatives of patients. In addition, both of these expert advisory panels may include a representative from each manufacturer of each medical technology that is included in the relevant research topic project or category for which the panel is established.

Provisions related to this rare disease panel include a requirement that any agency, instrumentality, or other entity managing or conducting the CER study for a rare disease must consult with the expert rare disease advisory panel. The statement of purpose also recognizes that any CER must consider variations in patient subpopulations. Additionally, the Institute would now be directed to take into account changes in the standard of care and advances in medical technology when it provides periodic review and update of evidence. CER studies would also now be required to account for genetic and molecular subtypes. This new bill also puts increased emphasis on using health IT in light of the \$19 billion invested in its adoption in the American Recovery and Reinvestment Act - data networks, electronic health records and patient registries are of particular importance to note here.

The Senate HELP Committee's comparative effectiveness research provisions in its health care reform draft legislation, the Affordable Health Choices Act, contains numerous amendments from Senator Mike Enzi (R-WY) including one with a rare disease advisory panel. While the rare disease panel is not as strong as the Baucus-Conrad language, it was important to get it adopted so the HELP bill is at least consistent with the approach for treating these types of patients in S. 1213, which is certainly PPTA's preference for what ultimately ends up on the Senate floor after the committees have reported out their respective bills. Comparative effectiveness research provisions that protect rare diseases in the Senate HELP and Finance Committees' will certainly enhance PPTA's efforts in the House of Representatives as the association continues to support several plasma protein therapies' consumer's effort to include a viable rare disease panel in legislation that will provide numerous safeguards for patients and physicians from indiscriminate restrictions on accessing the most appropriate and individualized treatment options.

In the House of Representatives, the tri-committee's health care reform discussion draft includes comparative effectiveness research provisions that include a clinical perspective advisory panel to advise CCER (Center for Comparative Clinical Effectiveness Research) for national research priorities. There is also a section for 'Stakeholder Input' that mandates stakeholder input on research questions and methodology and of which the CCER must consult with patients, health care providers, health care consumer representatives and other appropriate

stakeholders with an interest in the research through a transparent process. Moreover, there is language creating a "Patient Ombudsman," who "shall serve as an available point of contact for any patients with an interest in proposed" CER studies and "ensure that any comments from patients regarding proposed" CER studies are reviewed by the CER Commission. Also the bill's CER provisions recognizes unique subpopulations by requiring research to "be designed, as appropriate, to take into account the potential for differences in the effectiveness of health care items and services used with various subpopulations such as racial and ethnic minorities, women, different age groups, and individuals with different comorbidities." Although the House language is comprehensive in providing stakeholder input and recommendations, there are no provisions that give rare disease patients the kind of essential role that is encompassed in both the Senate HELP Enzi Amendment and Finance Committee's CER marker legislation S. 1213.

Follow-on Biologics

The Senate Health Labor and Pensions (HELP) Committee has a placeholder section for follow-on biologics provision in Subtitle A of the Title VI of the Affordable Health Choices Act. The language, however, has not been released to the public as of print time of this health policy update. It is probable that the language in the HELP draft bill will be primarily based on last Congress' S. 1695, that was passed out of the committee with bipartisan support. Although the bill did include a robust data exclusivity period of 12 years for innovator products, it did lack some of the safeguards for plasma derived

protein therapies and recombinant blood clotting factors that are encompassed in H.R. 1548, the House bi-partisan bill introduced earlier this year by representatives Eshoo, Barton and Inslee.

In the House, follow-on biologics initiatives are even more up in the air because the two competing bills are both from Energy and Commerce Democrats, including Chairman Waxman's H.R. 1427 and Representatives, Eshoo, Barton and Inslee's H.R. 1548. Chairman Waxman (D-CA) is facing pressure from nine Democrats on his committee to negotiate on follow-on biologics or face a move by the lawmakers to attach their own brand-backed bill to health reform legislation. The lawmakers wrote to Chairman Waxman demanding a meeting on the issue, and their resolve to push an amendment signals the Chairman lacks the votes needed to move his own generic-friendly follow-on biologics bill through committee. The lawmakers have noted that the Senate plans to include an approval path for follow-on biologics in its health reform bill and tell Chairman Waxman the House should formulate its own position prior to conference. They point out that their bill is supported by 19 members of the Energy and Commerce Committee and cosponsored by 100 House lawmakers, from both parties. Waxman's follow-on biologics bill has only 12 cosponsors. PPTA is on record of supporting H.R. 1548 because it includes safeguards that require FDA to create product class specific guidance for follow-on biologics and would also make FDA state whether there is current science feasibility to create an abbreviated pathway for products like plasma derived protein therapies and recombinant blood clotting factors.

Medicare Patient IVIG Access Act

The Medicare Patient IVIG Access Act is not incorporated in any of the health care reform draft proposals including the Senate Finance Committee and the House 'tri-committee' bills. PPTA along with the many of the interested stakeholders supporting the legislation will work with allied Members of Congress to include this key piece of legislation in any negotiated health care reform proposal that will eventually hit the House and Senate floors later this summer or early fall (depending on the timeframe and continuous pressure from the White House to get a final bill to the President).

Limiting Life Time Private Insurance Capitations

In early May 2009, the Senate Finance Committee released its policy options for expanding health care coverage that briefly described a policy option in section II of the plan that would mandate that "private insurance plans could not include lifetime limits on coverage or annual limits on any benefits and cannot charge cost-sharing (e.g., deductibles, copayments) for preventive care services". However, this policy option is only an outline for what the committee intends to include in its health care reform draft legislation that has yet to be released to the public.

Despite these initial positive steps in the Senate Finance Committee's policy options for expanding health coverage, PPTA and supporters of the S. 443/H.R. 1085, 'The Health Insurance Protection Act', are advocating for the bills inclusion in Senate Finance and the House 'tri-committee' health care reform discussion drafts.

S. 443 and H.R. 1085 phase in an increase in lifetime caps to \$10 million with an annual inflationary index. The bill exempts health plans offered at businesses with fewer than 20 employees, but would require that health plans meeting the parameters of the bill be offered to a small business at the employer's request. The bills are important to many plasma protein therapy consumers because of the high value medicines used to treat chronic conditions such as hemophilia and other bleeding disorders, genetic emphysema and primary and secondary immune deficiencies. Many patients with the aforementioned conditions may reach their lifetime cap within a few years or sooner if they have complications. Even if patients are able to find a way to maintain coverage, in the process they are often forced to make drastic choices that affect their employment, place of residence or even family life.

State Action

Market Access

Florida

Florida Legislature adjourned Regular Session on May 8, 2009. This was a one week extension brought on by the tight budget year. Federal Stimulus Funds allowed the Florida Medicaid Program to avoid eliminating Medicaid eligibility for certain groups that include individuals with hemophilia. These groups were funded with non-recurring funds, so the threat to their eligibility will be a concern during the 2010 Regular Session.

A screening and detection program for Alpha-1 Antitrypsin Deficiency funded through the Florida Department of

Health survived the Session without a reduction in funding. This was a minor miracle given the number of programs that were reduced as a result of the lean budget year.

Texas

The Texas Legislature passed an increase to the Medicaid clotting factor dispensing fee by 5 cents per unit. It is hoped that this increase in reimbursement for specialty pharmacies will ensure patient access to the therapies that is best for them.

The Texas Legislature also adopted a resolution declaring August Plasma Protein Therapies Month.

A bill to extend the Texas Bleeding Disorders Advisory Council (SB 1837) died on the calendar during the last day of Session. It was one of many to fail to pass because of a lengthy debate on the voter ID bill.

Communications

Webinars

PPTA continues to partner with consumer groups to co-host informational webinars designed to stimulate a dialogue about key topics facing access to plasma protein therapies. Already, PPTA has worked with the Hemophilia Federation of America to host a Plasma Protein Therapies 101 webinar with its leadership and is following up with two more this year. We will deliver the initial webinar program to a broader audience of patients and grassroots advocates.

The next webinar will be an issue-based presentation that will cover key topics

regarding federal health care reform efforts and what overarching changes in health care may mean for the users of plasma protein therapies. Additionally, PPTA is working with the Alpha-1 Association, The GBS/CIDP Foundation International and the Immune Deficiency Foundation to collaborate on several webinar programs this year. This initiative marks a new way to deliver important and timely information to consumers who are striving to ensure access to their lifesaving therapies, and PPTA appreciates the opportunity to work together with consumer organizations on this program.

Politico Advertorial

Building off of the successful April 28 advertorial that appeared in the Politico newspaper, a Capitol Hill-based publication and online newspaper (www.politico.com/preservepatientaccess), PPTA developed a series of advertisements that ran in Politico in mid-June, corresponding with the timing of the Senate HELP Committee mark-up of health care reform legislation. The advertisements reinforced key messages including the need to protect access to therapies for individuals with rare diseases and the need for a collaborative approach to on health care reform legislation. Click [here](#) to see PPTA's advocacy advertising series.

Media Outreach

PPTA distributed a press release to media on June 16 promoting the findings of the recent White Paper on follow-on biologics and the impact pending federal legislation may have on plasma protein therapies. PPTA continues to follow up with reporters who are covering health policy on our

position with respect to follow-ons and will explore doing so on other pressing issues affecting patient. Click [here](#) to read the release and the White Paper.

Calendar

The Advisory Committee on Blood Safety and Availability (ACBSA) meeting is tentatively scheduled for October 1-2 at the Universities at Shady Grove (9630 Gudelsky Drive, Rockville, MD 20850).

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Visit: <http://www.donatingplasma.org/>

This PPTA publication aims to keep Stakeholders apprised of evolving state and federal health policy developments. **To provide feedback or to add colleagues to the distribution list, please contact Diana Krueger at the Association.**

PPTA Staff is always available to attend consumer organization functions, make presentations, and assist in developing advocacy strategies and messages, as appropriate, with consumers. Please contact PPTA Staff at any time to discuss these activities.

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