

Health Policy Update

Federal Action

Medicare Patient Access

PPTA works with Congress, the Centers for Medicare & Medicaid Services (CMS), and the Food and Drug Administration (FDA) in communicating the importance of unfettered consumer access to all plasma derived and recombinant analog therapies (collectively plasma protein therapies) while at the same time educating policymakers on the unique, niche biologics industry that produce these lifesaving medicines.

111th Congress and the New Administration

Barack Obama, the 44th President of the United States was sworn into office on January 20, 2009. The president brings with him a huge mandate for “change” especially in terms of health care reform.

While President Obama is committed to bipartisanship, he will undoubtedly have great flexibility in driving his health care agenda during the 111th Congress because of the partisan make up of both the House and the Senate. Currently, the House is comprised of 255 Democrats and 178 Republicans. Two House seats are currently vacant because of Representative Kirsten Gillibrand’s (D-NY) appointment to the U.S. Senate and Representative Rahm Emanuel’s (D-IL) new roles as President Obama’s Chief of Staff. Venture capitalist Scott Murphy (D) will square off against New York State Assembly Minority Leader James Tedisco (R) for Gillibrand’s seat in an upcoming special election.

In the Senate, the Democrats currently have a 56 to 41 majority. Independent Senators Bernie Sanders (I-VT) and Joe Lieberman, however, both caucus with the Democrats. The trial over Minnesota’s seat in the U.S. Senate entered its second week on February 2nd. Comedian Al Franken (D) leads incumbent Senator Norm Coleman (R-MN) by just 225 votes. At question in the case are rejected absentee ballots that may have been inappropriately rejected.

President Obama’s nominee for Secretary of the U.S. Department of Commerce is Senator Judd Gregg (R-NH). Interestingly, Senator Gregg voted to abolish the agency as a member of the Senate Committee on the Budget, as well as on the Senate floor in 1995. New Hampshire Governor John Lynch, who is a Democrat, has, however, named Republican Bonnie Newman, Gregg’s former chief of staff. Newman will represent New Hampshire until a 2010 special election.

Although Congress remains solidly in the control of the Democrats, some dramatic changes have occurred in the past month at the top of the powerful House Committee on Energy and Commerce with Henry Waxman (D-CA) outmaneuvering the long time committee Chairman and stalwart, John Dingell (D-MI) for the committee’s top spot. Dingell, however, will remain on the Committee as Chair Emeritus and has already introduced H.R. 759, the Food and Drug Administration Globalization Act of 2009.

Representative Frank Pallone (D-NJ), a close political ally of Chairman Waxman and co-sponsor of H.R. 759 with Chairman

Dingell, retained his chairmanship of the subcommittee on health. New Democrat members to the Energy and Commerce Committee include Representatives Donna Christensen (D-VI), who is a physician, Kathy Castor (D-FL), John Sarbanes (D-MD), Chris Murphy (D-CT), Zack Space (D-OH), Jerry McNerney (D-CA) Betty Sutton (OH), Bruce Braley (IA) and Peter Welch (VT). The Republican leadership on the committee remains intact with Representatives Joe Barton (TX) and Nathan Deal (GA) heading the full committee and the subcommittee on health, respectively. Dr. Phil Gingrey (R-GA), an OB-GYN, is the one new Republican committee member.

The House Committee on Ways and Means will be led by longtime Chairman Charles Rangel (D-NY) and health subcommittee Chairman Pete Stark (D-CA). New Democrat members of committee include, Representatives Danny Davis (D-IL), Bob Etheridge (D-NC), Linda Sanchez (D-CA), Brian Higgins (D-NY), and John Yarmuth (D-KY). The GOP's representation on the Ways and Means committee will look a little different this year as well because of the retirement of Jim McCrery (R-LA). Representative Dave Camp (R-MI) will takeover as Ranking Member, while his spot as Ranking Member of the subcommittee on health was given to Wally Herger (D-CA), who was the runner-up to fill McCrery's position. Other new Republicans on the committee include, Representatives Ginny Brown-Waite (R-FL), Geoff Davis (R-KY), Dave Reichert (R-WA), Charles Boustany (R-LA), who is a cardiovascular surgeon, Dean Heller (R-NV), and Peter Roskam (R-IL).

In the Senate, the Committee on Health Education Labor and Pensions (HELP) will again be led by longtime Chairman Ted Kennedy (D-MA). The Democrats will enjoy a three seat majority with new committee members Senators Robert Casey (D-PA), Kay Hagan (D-NC), Jeff Merkley (D-OR), and a Senator to be named. Senator John

McCain (R-AZ) will be the only new GOP member of the committee.

Senate Committee on Finance Chairman Max Baucus (D-MT) will be joined by new members Senators Bill Nelson (D-FL), Robert Menendez (D-NJ), and Tom Carper (D-DE). On the Republican side of the Finance Committee, Senators Mike Enzi (R-WY), who is the Ranking Member on the HELP Committee, and John Cornyn (R-TX) are the new additions.

Health Care Reform: Comparative Clinical Effectiveness and Health Information Technology

President Obama nominated former Senate Democrat Leader Tom Daschle (D-SD) to be the Secretary of U.S. Department of Health and Human Services (HHS). Unfortunately, Senator Daschle, whose confirmation hearing before the Senate Finance Committee was scheduled for Tuesday, February 10, 2009, withdrew as nominee on February 3rd. It is unclear at this time if he will be leading the newly created Office of Health Reform at the White House, as he was scheduled to do.

The expansion and reauthorization of SCHIP is the first health care priority for the new administration. Both the House and the Senate have overwhelmingly passed SCHIP legislation, but the differences between the two bills must be reconciled in a conference committee before lawmakers can present it to President Obama for his signature. The only major obstacle is a provision in the House version that would ban new specialty hospitals from opening and require physicians to divest their financial interest in these hospitals. House Committee on Ways and Means Subcommittee on Health Chairman Pete Stark (D-CA) drafted this language, which the Senate did not include after pressure from the American Medical Association. The Senate conferees are Senate Committee on Finance Chairman Max Baucus (D-MT) and Senators Jay Rockefeller (D-WV), Kent Conrad (D-ND),

Charles Grassley (R-IA) and Orrin Hatch (R-UT).

Health Information Technology (IT) and comparative clinical effectiveness language have found their way into new economic stimulus bills, the American Recovery and Reinvestment Act, coming out of the House (H.R. 1) and the Senate (S. 336).

H.R. 1 passed the House on January 28, 2009 by a vote of 244 yeas to 188 nays, while Representative Brown-Waite did not vote on the measure. Six members of the fiscally conservative Blue Dog Coalition, Representatives Allen Boyd (D-FL), Jim Cooper (D-TN), Brad Ellsworth (D-IN), Collin Peterson (D-MN), Heath Shuler (D-NC), and Gene Taylor (D-MS), as well as Representative Paul Kanjorski (D-PA) and four freshman, Representatives Bobby Bright (D-AL), Parker Griffith (D-AL), Frank Kratovil (D-MD), and Walt Minnick (D-ID), who will likely be part of the Blue Dogs moving forward, joined the Republicans in voting against this bill. The Senate began considering the legislation the week of February 2, 2009. Instead of bringing up S. 336 to the floor, Senate Leadership brought up H.R. 1, struck everything after the enacting clause, and substituted the language from S. 336. This maneuver was likely done to avoid going to conference committee, which would be very challenging because of the very precise technical differences, especially with regard to the health IT language, between the two bills.

If the Senate's version of H.R. 1 is not filibustered, it will certainly pass and then the House will consider it. The House will likely make several amendments and pass it, which will force the Senate to consider the newly amended version. This process of sending amended versions back and forth could last several weeks and well into the scheduled recess of the week of February 16th.

H.R. 1 and S. 336 provide \$1.1 billion in additional money for comparative

effectiveness research -- \$400 million to the National Institute of Health (NIH), \$400 million in discretionary spending for the Secretary of HHS, and \$300 million for Agency for Healthcare Research and Quality (AHRQ). It would also allow the Secretary to spend up to \$1.5 million on an IOM study of comparative effectiveness research priorities. In its report accompanying H.R. 1, the House Committee on Appropriations suggests that treatment cost should be a component of comparative effectiveness research, which would ultimately be used for coverage determinations. The committee explained that a chief goal of comparative effectiveness research is that "more expensive [drugs] will no longer be prescribed."

Although several federal agencies have been conducting comparative effectiveness research for more than a decade and despite efforts of the Health Care Financing Administration [now the CMS], cost-effectiveness analysis has generally not been used for coverage policy decisions. Moreover, when Congress appropriated \$50 million in the Medicare Prescription Drug Improvement and Modernization Act (MMA) of 2003 [Pub L. 108-173, § 1013, 117 Stat. 2066, 2441 (codified at 42 U.S.C. § 299b-7)] to AHRQ for the purpose of conducting and supporting research with respect to "the outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs)," it expressly prohibited the CMS from "withhold[ing] coverage of a prescription drug" based on findings from this research. The conference report accompanying the MMA suggests that CMS is banned not only from issuing national coverage determinations based on the data, but also from using the data to establish a pay-for-performance paradigm.

- Advocacy

PPTA and several consumer organizations either signed on to letters from a broad coalition of stakeholders opposing the report

language attached to H.R. 1, or submitted their own letters speaking directly to how the use of comparative clinical effectiveness research data in making coverage determinations could have an adverse affect on their specific patient population's access to therapy. The result of this action was quite successful in terms of not only the report language accompanying S. 336, but also the legislative language of S. 336.

During the Senate Appropriations Committee mark up, Senators Frank Lautenberg (D-NJ) and Arlen Specter (R-PA) introduced an amendment that specified that research Congress was funding was comparative *clinical* effectiveness, which is consistent with the congressional intent of the MMA language that initially began funding AHRQ for this purpose. These changes were made to both the legislative language and report language. Additionally, the S. 336 report language includes no reference to cost-effectiveness, which is a much appreciated departure from the misguided H.R. 1 report language. The outreach by the consumer organizations, PPTA, and other stakeholders was instrumental in achieving this positive outcome, but continued vigilance on this issue is critical.

Regardless of the positive outcome with the stimulus legislation in the Senate, cost containment is the ultimate goal and comparative clinical effectiveness research will continue to be an issue even if the American Recovery and Reinvestment Act containing comparative effectiveness language is signed into law. The blue print for using research data for cost containment is the United Kingdom's National Institute for Comparative Health and Excellence ("NICE"). Patient advocacy organizations, especially those representing patients suffering from rare, chronic, and debilitating diseases, disorders, and medical conditions, have been highly critical of the NICE program for making coverage decisions based purely on the cost of the prescription drugs.

As you may remember, several bills addressing comparative effectiveness research appeared during the 110th Congress. The leading legislation, S. 3408, which Senators Baucus and Conrad introduced before the August 2007 recess, could very likely be introduced in the 111th Congress. On January 27, 2009, Senators Baucus, Conrad, Hatch, Enzi, Menendez, and Carper engaged in a colloquy on comparative effectiveness research during a Finance Committee Executive Session. Chairman Baucus clearly stated his intention to reintroduce S. 3408 with Senator Conrad.

S. 3408 of the 110th Congress is certainly a good framework from which to begin expanding comparative effectiveness research, but it needs some significant refinement in order to protect the interests of patients and physicians. For example, although S. 3408 expressly prohibits the independent comparative clinical effectiveness research entity that the legislation creates from establishing practice guidelines or policy recommendations, it does not expressly prohibit CMS from making coverage decisions based on the research.

– Link to Health IT

Additionally, because of the health IT included in H.R. 1 and S. 336, the move toward a rapid-learning health system to fill the existing knowledge gaps on prescription drugs is clearly progressing. Very likely, the \$400 million in funds to be transferred from AHRQ to NIH for comparative effectiveness research will be used toward establishing a national rapid learning system consisting of electronic health record databases. The HHS Secretary could also very likely use the discretionary \$400 million for these purposes. Comparative clinical effectiveness research data will be an important component of a rapid-learning system. If the widespread adoption of health IT becomes a reality in the next decade, physicians will then be able to search these various databases of electronic health

records and data from studies for similar patient profiles to instantly determine which course of treatment could be most effective. Cost effectiveness will continue to be an issue.

PPTA will continue to work to ensure that any expansion of comparative clinical effectiveness research will not adversely affect a patient's ability to work closely with their physician to establish a treatment plan best suited for them.

State Action

Market Access

According to the Center on Budget and Policy Priorities at least 46 states are facing deficits and the combined budget gaps for the next two years are estimated to be more than \$350 billion. This means legislatures will have to reduce health care expenditures. Common strategies to reduce budgets involve limiting patient access to certain products and limiting the site of service for therapies.

Patient access to their medically appropriate therapies is always top of mind for PPTA State Affairs. We are focused on helping patients maintain open access to all plasma protein therapies and their recombinant analogs.

PPTA State Affairs have targeted our attention on **California, Florida, Minnesota and Texas**. In California, we are concerned about the possible implementation of a restrictive formulary for clotting factor. We continue to monitor the situation and are working with Committee of Ten Thousand (COTT), Hemophilia Federation of America (HFA), and National Hemophilia Foundation (NHF) to ensure that a restrictive formulary is not implemented.

Quality of care legislation has been filed in Minnesota to protect patient access from private insurer and Medicaid cost

containment strategies. Senator Kathy Sheran introduced Senate Bill 339 and Representative Kim Norton introduced the identical legislation as House Bill 410. Working in concert with the Immune Deficiency Foundation (IDF) and the Alpha-1 Association, we will now begin the advocacy effort necessary to promote the bill.

Threats to patient access will emerge as state legislatures begin their budget development process. Please consider PPTA as a resource as you develop your advocacy efforts to combat these threats. PPTA would like to expand its advocacy partners. If you would like more information on advocacy efforts of the Association please contact bspeir@pptaglobal.org

Communications

PPTA is developing a series of informative Webinars this year that aim to provide information and create awareness among a grassroots consumer audience about plasma protein therapies, how they are manufactured, their cost structure, their uniqueness, and the health policy issues that policy makers are considering that could impact their access to care. We invite all consumer groups to participate with us in this program.

Using an Internet-based technology not only is less expensive to use to reach a widespread audience, but allows for tremendous flexibility. PPTA will develop the content and the slides and has webinar conferencing capabilities through its conference call vendor. Consumer groups can choose to host the program from your office, allowing the executive director or CEO to introduce and participate in the program, further connecting with the grassroots audience and encouraging discussion – the technology allows participants to ask questions of the presenters. Or, PPTA can co-host the program as well – choose what works best

for your members. PPTA will work individually with consumer groups on the content of each program allowing messages to be tailored for specific audiences.

In order to get the word out, PPTA is relying on consumer groups that would like to participate to “push” information about the Webinars and their content (which PPTA will provide) through email and other communication vehicles such as websites, magazines, news blasts, conference calls, peer/chapter meetings, etc., to your members. PPTA also will post information on its website www.pptaglobal.org. Neither the association nor any member company will have or is asking for any direct access to consumer contact information, and this is not a product marketing endeavor, rather it is specifically intended to help inform consumers about the nature of their highly specialized therapies and to explore strategies to assure their access to plasma protein therapies.

Here’s a sneak peek at some of the content that will be included the first program:

- 1) Introduction of Webinar participants and an explanation of the technology (i.e. how to ask questions);*
- 2) What is PPTA? (discussion of the mission of the association and a mention of the companies it represents;*
- 3) Plasma protein therapies (outline the classes of therapies that replace proteins that are missing or deficient in an individual’s plasma);*
- 4) How therapies are made (discussion of donated plasma, safety innovations, regulations and standards, the manufacturing process [will include a discussion on recombinant blood clotting factors with a bleeding disorder audience]); and*
- 5) Why the therapies are unique (summary of the unique aspects of the therapies that set them apart from traditional, chemical pharmaceuticals).*

We hope you will join PPTA in making the Webinar series a success. This is a new

endeavor for us and PPTA needs the support from its Stakeholders to be successful. In order to be successful, the Webinars must reach the grassroots within each consumer organization. Many organizations are already working effectively to engage the grassroots on legislative issues through such tools as Cap Wiz and issuing action alerts. These tactics are excellent and should continue. The Webinars complement these types of advocacy tools.

In the 111th Congress and in the states, we will have to work hard as a community to assure that patient access to plasma protein therapies is not impaired by legislation or regulations put in place to control spiraling budget deficits. The grassroots will be fundamental to our advocacy efforts. In order to have an impact in a legislator’s office, they must hear from their constituents, your members.

PPTA feels it is the industry association’s responsibility to explain and create a working knowledge among all users of plasma protein therapies of plasma-derived therapies and/or recombinant analogs and why unfettered patient access is a priority. It is PPTA’s job to explain to the grassroots: What is Plasma?; How is it collected?; Is it safe?; How is the therapy that I infuse made?; What is Congress or a state legislature going to do to control health care spending and how will that effect me?; and How do I make my voice heard in the states and on Capitol Hill? The primary goal of the Webinars is to create an informed and empowered grassroots network of plasma protein therapeutics users, who will be heard by policymakers on the crucial issue of assuring patient access to care.

If you are interested in working with PPTA to make informational Webinars available to your constituency and have not already spoken with us, please contact Kym Kilbourne (kkilbourne@pptaglobal.org). We have discussed this concept with many of you, but if there is an organization that

receives this newsletter that would like to explore the use of the PPTA webinar series as a component of your advocacy or as a means to raise awareness and inform your constituency, please contact Kym.

Calendar

Save the Date
June 2 and 3, 2009



PPTA is hosting the **2009 Plasma Protein Forum** June 2 and 3, 2009 at the Washington Marriott (1221 22nd Street NW; Washington, DC). Program and Registration information can be found at: <http://www.plasmaproteinforum.com/>

The Advisory Committee on Blood Safety and Availability (ACBSA) meetings are tentatively scheduled for April 30 – May 1 and October 1-2 at the Hilton Rockville Hotel (1750 Rockville Pike; Rockville, MD).

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

PPTA launched an educational website ([DonatingPlasma.org](http://www.donatingplasma.org/)) that provides information and boost awareness for source plasma donation. Please link to http://www.donatingplasma.org from your website, so that more people become aware of the value of plasma and its importance in producing therapies for individuals coping with rare, chronic, often genetic diseases

and disorders—and will consider becoming committed donors.

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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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