

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 Legislation

Comparative Clinical Effectiveness Research

The American Recovery and Reinvestment Act of 2009 (H.R. 1) includes additional funding for comparative effectiveness research and provides incentives and penalties to spur the widespread adoption of interoperable health information technology systems. The legislation provides \$1.1 billion in additional money for comparative effectiveness research—\$400 million to the National Institutes of Health, \$400 million in discretionary spending for the Secretary of Health and Human Services (HHS), and \$300 million for the Agency for Healthcare Research and Quality (AHRQ). Although several federal agencies have been conducting comparative effectiveness research for more than a decade and despite the efforts of the Health Care Financing Administration [now the Centers for Medicare and Medicaid Services (CMS)], cost-effectiveness analysis has generally

not been used for coverage policy decisions. However, comparative clinical effectiveness research initiatives with the goal of reducing costs by giving CMS the ability to make coverage decisions and thus reducing costs to the federal government are beginning to be debated with increased intensity on Capitol Hill. Critics of the provision in H.R. 1 argue that the research, without specific limitations, would drive CMS reimbursement decisions and ultimately limit patient access to therapies.

Currently, there has not been any comparative clinical effectiveness research legislation introduced in either the House or the Senate, but PPTA will continue advocating for legislative proposals that supports comparative clinical effectiveness research that both advances the treatment of individual patients and recognizes the unique nature and value of targeted therapies that benefit patients with rare, chronic, and debilitating disease disorders and medical conditions. Furthermore, with respect to certain diseases, including many rare diseases, and therapies used to treat these diseases, comparative clinical effectiveness research has limited utility. For example, because each individual reacts differently to each plasma protein therapy, patients must have access to the complete range of plasma protein therapies in each therapeutic class. Moreover, in this current economic environment, resources expended in the furtherance of comparative clinical effectiveness research should be limited to cases where the information produced from such research has value to the physician in the treatment of their individual patients.

Follow-on Biologics

On March 11, the Chairman of the House Committee on Energy and Commerce, Henry Waxman (D-CA) along with the committee's Health Subcommittee Chairman Frank Pallone (D-NJ) and Health Subcommittee Minority Leader, Nathan Deal (R-GA) introduced the bipartisan follow-on biologics legislation H.R. 1427, "Promoting Innovation and Access to Life-Saving Medicines Act." The bill would give brand biologic makers five or three years of exclusivity (depending on whether a biologic is new or an improvement on an existing product), plus the possibility of an extra six months for significant therapeutic advances, six months for pediatric research and three years for conditions of use. The bill would allow interchangeability, award a six-month biogeneric exclusivity, prohibit "authorized generics" and guard against "evergreening." PPTA is concerned about this bill because of the lack of innovator biological manufacturer protections and safety concerns including the lack of standards for immunogenicity testing or clinical trials.

A more favorable follow-on biologics for innovator biological manufacturers, the 'Pathway for Biosimilars Act' was introduced on, March 17, stemming from the House Energy and Commerce Committee member's, Anna Eshoo (D-CA), Jay Inslee (D-WA) and full committee Minority Leader Joe Barton (R-TX) along with 46 other co-sponsors. Highlights of the bill include 12 years of data exclusivity to the makers of brand bio and additional 2 years if a new indication is approved that offers a significant improvement in the treatment, diagnosis, or prevention of disease within eight years after the biologic is initially approved. The bill does provide a mechanism for the FDA to determine that biological products are interchangeable; however, FDA must issue guidance advising that it is feasible in the current state of scientific knowledge to make a determination of interchangeability for that product class. This aforementioned provision is favorable to PPTA because

plasma derived therapies and recombinant blood clotting factors products raise significant scientific challenges to provide for an abbreviated application process. This holds by the fact that the European Medicines Agency (EMA) in its guidance for abbreviated application approval pathways for biologicals has determined that plasma derived therapies such as clotting factor, immune globulins, alpha1 proteinase inhibitors as well as recombinant blood clotting factor products will not be approved.

Although there has not been legislation introduced in the Senate, observers of this critical issue eagerly await the Health Education Labor and Pensions (HELP) Committee Chairman, Edward Kennedy's (D-MA) benchmark bill that will lay the groundwork for bicameral negotiations for a viable follow-on biologics legislation.

'The Health Insurance Protection Act' (S. 442 / H.R. 1085)

On February 13, S. 442 was introduced by Senator Byron Dorgan (D-SD) and co-sponsors Olympia Snowe (R-ME) and Sheldon Whitehouse (D-RI). A day earlier, on February 12, H.R.1085 was introduced by Representative Anna Eshoo and eighteen other cosponsors. Both bills 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 bill is 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. PPTA supports the leading plasma protein therapies consumer organizations in advocating for these important pieces of legislation.

'Medicare Patient IVIG Access Act of 2009'

Advocates of the intravenous immune globulin therapies community eagerly await the introduction of the Medicare Patient IVIG Access Act of 2009. The introduction of this bill will be a significant step in the efforts to restore access to lifesaving immune globulin replacement therapies for Medicare beneficiaries. Garnering support for this legislation has been a priority for many consumers in the immune globulin therapies community including PPTA since the introduction in the 110th Congress of H.R. 2914 and S. 2990, the Medicare IVIG Access Act, by Representative Kevin Brady (R-TX) and Senator John Kerry (D-MA) respectively. H.R. 2914 had more than 50 co-sponsors from both the Democrat and Republican sides of the aisle.

The new and updated bill to be introduced any day now, is an important piece of legislation that directs the Secretary of Health and Human Services (HHS) to review the 2007 OIG and ASPE reports and other surveys to update the Medicare payment to provide appropriate reimbursement for providers related to the furnishing of IVIG in both the physician office and the hospital outpatient setting. Another key provision in the bill provides for an IVIG home infusion benefit that will help Medicare beneficiaries with primary immune deficiency diseases access IVIG therapies in their own homes.

To help garner support for the Medicare Patient IVIG Access Act of 2009, the Immune Deficiency Foundation (IDF) will hold its lobby day in Washington, D.C. on March 26, 2009. This is a great opportunity for legislators and their staff to hear from patients and their loved ones about the positive impact that immune globulin

therapies have on their lives. PPTA applauds these efforts and will work to support its consumer allies in helping to secure and restore Medicare patient's access to this vital treatment.

New Leadership

Health and Human Services

On March 2, The White House nominated Kansas Gov. Kathleen Sebelius (D) as Secretary of HHS. In addition, to her experience as Governor, Ms. Sebelius was the Kansas insurance commissioner. Unlike previous Secretary-designate Tom Daschle, Sebelius will not take on the role of White House health reform czar.

White House: Director of Health Reform — "Health Czar"

On March 2, The White House announced that Nancy Ann DeParle will lead the Administration's health care reform efforts as the new health 'czar'. DeParle was the administrator at the Health Care Financing Administration and before that a policy advisor to President Clinton and HHS Secretary Donna Shalala. Interestingly, Ms. DeParle has long been an advocate to use comparative effectiveness research to allow Centers for Medicare and Medicaid Services (CMS) to use cost-effectiveness data in making coverage decisions.

Food and Drug Administration

On March 12, President Barack Obama nominated former New York City health commissioner Margaret Hamburg to lead FDA and tapped Baltimore health commissioner Joshua Sharfstein as her principal deputy drew praise from consumer advocates. Dr. Hamburg's most recent experience has involved the public health threats posed by chemical and biological weapons. She was named vice president for biological programs at the Nuclear Threat Initiative in 2001. Hamburg also worked in the Clinton administration, signing on in 1997 as the assistant secretary for policy

and evaluation at HHS. She is a member of the Institute of Medicine.

FDA Commissioner-designate Margaret Hamburg and deputy commissioner-designate Joshua Sharfstein have been praised by the Obama Administration for their public service and public health administrative credentials. PPTA looks forward to working with the new FDA leadership when they are confirmed.

Centers for Medicare and Medicaid Services

The Obama Administration has yet to nominate the top Administrator position at Centers for Medicare and Medicaid Services.

State Action

Market Access

Florida

The 2008 Legislature passed a law eliminating Medicaid eligibility for Medically Needy and MEDS A/D individuals effective July 1, 2009. This included 33 people with hemophilia.

The federal stimulus money for Medicaid requires states taking the stimulus funds to maintain eligibility for anyone eligible for Medicaid effective July 1, 2008. In speaking with Florida budget staff, they've all confirmed that the state will take the federal funds. This would result in the Medically Needy and MEDS A/D individuals maintaining their Medicaid eligibility. PPTA staff will continue to monitor the situation and lobby for the eligibility of these individuals who were reliant on plasma protein therapies and their recombinant analogs.

Minnesota

PPTA and the patient communities have been working relentlessly to promote a standards of care bill, Senate File 339. **The bill was heard by the Senate Health, Housing and Family Security Committee on March 18th.** After hearing testimony from the sponsor, Senator Kathy Sheran, and the patients, the Committee proposed an amendment that would limit the bill to the Board of Pharmacy creating rules based on medical guidelines that would govern standards of care for individuals with alpha-1 antitrypsin deficiency, primary immunodeficiency diseases and von Willebrand disease. The bill has been temporarily postponed while the patients decide on whether or not they want to have the bill amended. Obviously the bill contained more than just the rulemaking but given all the strong efforts by all the proponents of the bill, rulemaking could be the first step in protecting patient access to their medically appropriate therapy.

Texas

PPTA staff met with leading decision-makers in Texas including Chris Traylor, Director of Texas Medicaid, and Rep. John Zerwas, Chairman of the House Health Appropriations Subcommittee. We took the opportunity to discuss our concerns that the state's policies may impact patient access to care. PPTA staff took this opportunity to educate the decision-makers on the nature and use of plasma protein therapies and their recombinant analogs, and how critical it is for patients to access their medically appropriate therapy.

As threats to patient access emerge during the legislative session in your state, 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 Bill Speir (bspeir@pptaglobal.org).

Communications

Politico Advertorial Coming Soon...

PPTA is developing an 8-page special supplement in the April 28, 2009 edition of Politico. This piece will inform members of Congress, Congressional staff, members of the new Administration and others inside and outside of Washington, D.C. who are making decisions about healthcare reform and public-payer insurance programs, and plasma protein therapies, who they treat, how they are produced, why they are different from traditional pharmaceuticals, and the importance of patient access.

The content in the special section will be a "pull out" that can be used throughout the year for advocacy and to inform audiences of the importance of open access to all plasma protein therapies (plasma-derived therapies and their recombinant analogs) in all sites of service and the differences between plasma protein therapies and traditional, chemical pharmaceuticals and other biologics. PPTA will receive 5,000 reprints that the association will share with consumer groups for use in their advocacy as well.

Beyond a one-day print run, the content of the special section will be featured on Politico's online newspaper indefinitely. PPTA is collaborating with several national consumer groups as well to help these important audiences learn more about the importance of therapies and the individuals they treat.

About Politico

Politico is a multiplatform news source for people interested in politics. The newspaper caters to the demands of the 24-hour political news consumer. Politico publishes Tuesday through Thursday when Congress is in session. The Politico reaches an estimated 50,000 readers per issue. Politico.com launched two years ago and caters to the nation's 24-hour political news

consumer. The site received more than 2.2 million unique visitors in 2007 monthly - more traffic than all of its primary Washington D.C. competitors combined. As of March 2008, Politico.com is reaching nearly 6 million unique visitors per month. Politico.com also is the 10th most popular newspaper website in the country.

Information Webinars Hosted This Spring

PPTA has shared the first webinar slide deck with national consumer group representatives after a discussion of the new initiative at the previous two stakeholder meetings and gotten excellent feedback. With that input from the national groups, PPTA has tailored and edited the basic program and is poised to help any consumer organization present this program to their members. Already PPTA is working with three national groups that have expressed interest in providing this valuable information about the plasma protein therapies they are using, and initiating this framework for additional educational programs in the future that may help inform and empower grassroots consumer advocates. We invite all consumer groups to participate with us in this joint program and welcome an opportunity to explore how this would work best for your consumer organization.

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 these Webinars as a component of your advocacy or as a means to raise awareness and inform your constituency, please contact Kym Kilbourne.

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 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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