

2012 Policy Priorities

PREAMBLE

The National Patient Advocate Foundation advocates for policies that promote access to quality, affordable health care services for patients with chronic, debilitating conditions and policies that balance the advancements and innovations in health care delivery with the economic limitations imposed by society's efforts to contain health care spending. NPAF recognizes and respects the myriad of factors that determine one's access to care; however, its first priority and unwavering commitment is to a better, healthier outcome for patients.

EXECUTIVE SUMMARY

Annually, the National Patient Advocate Foundation (NPAF) releases a policy document that sets forth its legislative and regulatory priorities for the year. Since its inception in 1996, NPAF has focused its policy priorities on those policies determined to have the greatest impact on patients with chronic, debilitating, and life-threatening diseases who are served by NPAF's companion organization, the Patient Advocate Foundation (PAF). Nearly 83,000 patients were served by PAF in 2010; another 4 million through online assistance. From this experience, NPAF is able to determine the most critical hurdles patients face accessing the care they need; e.g., exorbitant medical debt, crippling access to providers, misunderstanding and confusion regarding their right to appeal denied claims, access to specialty providers, and inability to obtain affordable health insurance, etc. and advocate for the legislative and regulatory changes needed to eliminate them.

NPAF's 2012 Policy Priorities are established with the expertise and political acumen needed to successfully predict which policies will have the greatest impact on the patients served by PAF. Some priorities represent a continuation of last year's efforts; others are new and reflect the enormity of regulatory and legislative initiatives stemming from the passage of the Patient Protection and Affordable Care Act (PPACA) in 2010. Although each priority is assigned a programmatic category – Federal Regulatory, Federal Legislative, State Regulatory and Legislative – and are prioritized according to its direct or indirect alignment with NPAF's mission, it is well recognized that most priorities cut across programs and require a comprehensive approach involving NPAF's Executive Board of Directors, Policy Committee, Scientific Board and outside consultants for implementation and success.

Federal Regulatory Priorities

NPAF's 2012 Tier 1 Federal Regulatory priorities include three areas that have been modified or created under the PPACA which have the greatest patient impact. First, the establishment of pre-existing condition insurance plans (PCIPs) has seen limited success due to the high premiums required, and more importantly, the six-month waiting period. Approximately five to seven million Americans are estimated to lack health insurance and have a pre-existing condition. The program will expire in 2014 which makes this a politically challenging issue to amend or repeal. Nonetheless, this is a policy that NPAF has championed and brought to the forefront for years and will continue to do so in 2012. While NPAF's overarching preference is to eliminate the six-month waiting period, given the political unlikelihood of such a wholesale change, NPAF will explore the feasibility of legislation that would 1) enable COBRA beneficiaries operating under federal guidelines to transition to a PCIP plan without the six-month waiting period; and 2) charge CMS with exploring patient rights to appeal denied claims.

Second, patient rights to appeal denied claims were diminished by a Health and Human Services final rule in 2011 that weakened patient rights offered in the proposed rule published in 2010; e.g., allowed plans to provide less information and explanation to patients about why the claim was denied and limited the type of denials that could be challenged. NPAF will actively inform federal officials and other non-patient advocacy groups of the potential hurdles that may result from the final rule.

Finally, the PPACA directs HHS to specify the "essential health benefits" that non-grandfathered plans in the individual and small group markets will be required to cover. The essential health benefit definition is an imperative one as the qualified health plans offered by state health exchanges must comply with the definition. Exchanges are virtual marketplaces where individuals and small businesses can compare health insurance plans as well as purchase health insurance on the state level. Up to 30 million people are expected to enroll in health plans through state health exchanges. Working in collaboration with the NPAF State Government Affairs team, NPAF will draft another White Paper on Essential Health Benefits and push forward on those principles that assure patients with chronic, debilitating and life-threatening diseases have necessary benefits. Included will public comment on the pending rule on "medical necessity" and its importance to assuring patient rights are maintain, including those upon appeal of denied claims.

NPAF's 2012 Tier 2 Federal Regulatory priorities include the premium insurance tax credit, section 340B of the Public Health Service Act, and personalized medicine, including follow-on biologics and genetic testing. First, PPACA introduces premium insurance tax credits which were designed to assure that plans offered through state health "exchanges" in 2014 are affordable by providing a tax credit to help certain health consumers pay for insurance premiums. Eligibility for the tax credit requires that the employer plan must be unaffordable; however, the determination of what is unaffordable is based upon various factors which may discourage the selection of family coverage.. Although not implemented until 2014, NPAF will educate policy members at the Treasury Department regarding the unintended consequences of this policy and provide information through NPAF volunteer programs to patients and consumers.

Second, Section 340B of the Public Health Service Act requires drug manufacturers participating in Medicaid to provide discounted outpatient drugs to certain eligible entities, known as covered entities. Critical concerns have been raised about whether the program is functioning as intended and NPAF will continue actively participating in a 340B policy workgroup to ensure that the medically underserved-focused intention of the legislation is met.

And finally, personalized medicine represents a promising new approach to patient care that will take advantage of individual patient characteristics such as age, coexisting conditions, and patient preferences in choosing a care plan. It also includes the use of advanced individual genomic information in choosing a biologic agent; and the development of therapies tailored to patient needs; e.g. customized monoclonal antibodies and vaccines. PPACA creates a licensure pathway for follow-on biologics that allows a generic company to establish that its drug product is chemically the same as the already approved innovator drug, and therefore meets approval for market use. NPAF welcomes personalized medicine and the potential benefits for patients and will straddle support of policies and positions that both protect patients and further innovations in personalized medicine. NPAF will also continue its participation with the National Comprehensive Cancer Network Biosimilars Work Group.

NPAF's 2012 Tier 3 Federal Regulatory priority involves making available supplemental health insurance plans for those under age 65. To help pay for some of Medicare's cost-sharing requirements most beneficiaries have some type of supplemental health insurance coverage. Privately purchased Medigap is an important source of this supplemental coverage; however, many states no longer offer Medigap policies. NPAF will partner with advocacy partners, specifically the kidney disease organizations to encourage more state insurance commissioners to make plans available.

Federal Legislative Priorities

NPAF's 2012 Tier 1 Federal Legislative priorities address two issues of ongoing importance to assuring adequate provider services and vital drugs to patients. First, the Sustainable Growth Rate (SGR) and Average Sales Price (ASP) are two ways in which Medicare providers are reimbursement for services and drugs used in the outpatient setting. SGR is the formula that determines how much Medicare providers are compensated for treating beneficiaries. SGR was established to provide annual updates to physician reimbursement for services, but because rates are based on the Gross Domestic Product (GDP) and not actual health care practice costs, physician expenditures often exceed the SGR and as a result, their reimbursement rates are cut. NPAF will continue to work in close partnership with patient advocate colleagues to eliminate SGR entirely and to direct Congress to find a more workable solution.

Medicare Part B reimburses medications administered in community oncology clinics, where 80 percent of cancer beneficiaries receive treatment, at Average Sales Price +6%. Recent legislative threats would reduce the amount to ASP +3, or lower to address the budget deficit. NPAF is concerned that this approach would discourage providers from offering services to Medicare beneficiaries, especially in rural areas. NPAF will work collaboratively with members of the ASP Coalition, which includes approximately 20 health care stakeholder groups, to influence legislators to consider the impact that cuts to ASP has on beneficiaries and will host a Congressional briefing on this issue.

Finally, drug shortages or discontinuation of a drug can have a disastrous impact on patients, especially cancer patients and those in need of IV nutritional supplements. Alarming, the number of new prescription drug shortages increased from 211 in 2010 to 267 in 2011. NPAF will continue to respond to requests from Congressional members to explore solutions and will coordinate a Congressional briefing in 2012 to raise awareness of the need for transparency among providers and patients when drugs are in short supply or unavailable.

NPAF's 2012 Tier 2 Federal Legislative priorities include first, passage of the Prescription Drug User Fee Act (PDUFA) and the Medical Device User Fee Modernization Act (MDUFMA), legislation which enable industry to supplement, through user fees, FDA appropriations in order that drugs, biologics and medical devices can be reviewed and approved in a predictable and timely manner. NPAF will closely monitor both pieces of legislation and work with its partners at the Regulatory Education and Action for Patient (REAP) coalition and other coalitions to ensure that any request for an increase in user fees balances patient's expedient and safe access to drugs, biologics and medical devices with the potential to unduly burden and stifle industry.

The second Tier 2 priority concerns chronic disease access to care and survivorship planning. In 2005, 133 million Americans – almost 1 out of every 2 adults – suffered from at least one chronic illness. Seven out of 10 deaths among Americans each year are from chronic diseases. While it is impossible for NPAF to be engaged in every disease-related legislative or regulatory initiative that potentially improves patients' access to care, the following offer great potential for NPAF to positively impact patient access to care outcomes: 1) the Zadroga 9/11 Health and Compensation Act addressing needs of post-9/11 safety workers who develop cancer; the Breast and Cervical Cancer Treatment Program and securing treatment funds; diabetes-related programs identified through the "Early Detection Program; the HEART for Women Act; the enhancement of cancer survivorship management; and support for treatment options for patients with rare diseases.

State Regulatory and Legislative Priorities

NPAF's 2012 Tier 1 State Regulatory and Legislative priorities are intricately related: state health "exchanges" and essential health benefits. First, as noted above, PPACA creates state health exchanges, which are virtual marketplaces where individuals and small businesses can compare health insurance plans as well as purchase health insurance. The purpose of exchanges is to make health insurance more affordable and easier to purchase for small business and individuals. Second, as noted under Federal Regulatory priorities, the PPACA directs HHS to specify the "essential health benefits" that qualified health plans in the exchanges will be required to cover. NPAF will continue to identify how both exchanges and essential health benefits can be developed in a patient-centric manner through public comment, participation in state-based policy discussions and issuing a revised White Paper on essential health benefits in early 2012. NPAF will work directly with state policy makers and will direct its senior level volunteers to interact with state health exchange governing board members, state governors and state policy makers to ensure that essential health benefits successfully meet patient needs.

NPAF's 2012 Tier 2 State Regulatory and Legislative priority involves efforts to assure parity in the reimbursement for orally administered chemotherapy versus those chemotherapy administered intravenously. NPAF has been actively involved in recent years with the passage of parity legislation and will continue to work with coalitions organized by the American Cancer Society and Leukemia Lymphoma Society for passage in additional states. NPAF de-identified patient data will be provided to inform policy and NPAF's State Government Affairs team will personally meet with state legislators and key health officials in target states where passage is likely.

NPAF's 2012 Tier 3 State Regulatory and Legislative priority concerns Medicaid stability and state formularies. PPACA mandated fairly massive reform to the nation's Medicaid program. Individuals and working families that earn up to 133 percent of the federal poverty level will be eligible for Medicaid starting in 2014. The Congressional Budget Office (CBO) estimates that by 2019, 16 million more adults and children will enroll in Medicaid and gain access to affordable coverage as a result. NPAF will track and monitor state legislative challenges and will work with patient advocate partners wherever possible to intercede on behalf of patients when onerous budget cuts are threatened.



2012 National Patient Advocate Foundation

Policy Priorities

INTRODUCTION

Each year, the National Patient Advocate Foundation (NPAF) publishes a comprehensive policy strategy document which includes a listing of its top regulatory and legislative priorities for the year. NPAF's priority identification process assures its financial, as well as human capital, are strategically aligned to optimize the leveraging of resources. Traditionally, the priorities are grouped according to the *primary* area of responsibility or impact and include the following Divisions: Federal Regulatory, Federal Legislative, and State Legislative/Regulatory. As in past years, the 2012 Policy Priorities were informed by an iterative process that involved substantial stakeholder involvement, such as a thorough analysis of the patients served by NPAF's companion organization, the Patient Advocate Foundation (PAF). The NPAF iterative process also includes a number of activities likely to yield qualitative information from those most familiar with patient access issues. For example, NPAF hosted an in-depth meeting with PAF senior professional case managers who collectively have the intellectual and experiential ability to identify the most pressing patient access issues affecting patients nationwide. Other activities included the solicitation of relevant information from healthcare experts in the field such as:

- gathering input from NPAF's Executive Board and Scientific Advisory Committee members whose expertise is recognized nationally;
- reviewing 2011 Policy Priorities to evaluate the status of former priorities to identify which represent completed policy issues as well as identify areas of continued need;
- engaging in collaborative discussions with NPAF legislative and regulatory consultants to identify how best to refine NPAF strategies so that they can continue to influence the quickly-changing political environment; and
- thoroughly assessing state and federal legislation and regulations so that past NPAF policy successes are not vulnerable to future legislative changes.

The NPAF iterative process also benefits from the many meetings NPAF members attend as a result of collegial relationships with peer organizations. NPAF partners with countless non-profit colleague charities that serve patients with chronic, debilitating and life-threatening conditions. These meetings also aid NPAF in its comprehensive assessment of the most salient patient access challenges likely to be

remedied by changes in health policies. The priorities discussed herein represent NPAF's primary areas of legislative and regulatory focus for 2012, prioritized by their ability to positively impact patient access to care outcomes.

The policy environment is an exciting one, as every year represents unique opportunities. In 2012, NPAF Policy Priorities will be influenced by the United States Supreme Court's decision on the Constitutionality of the "individual mandate" in the Patient Protection and Affordable Care Act (P.L. 111-148, "PPACA"),¹ that passed in March, 2010, and a Presidential Election that promises to highlight the domestic priorities that impact all Americans: the economy, jobs, and access to healthcare. Many of the 2012 Policy Priorities transcend multiple disciplines but each has been assigned a *primary* category (i.e. State Legislative/Regulatory, Federal Legislative or Federal Regulatory) to enable proactive alignment of internal and external resources to address the challenges for the coming year.

For the first time ever, NPAF's Policy Priorities include a category for the advocacy and support entitled "*Chronic Disease Access to Care and Survivorship*." This new priority does not represent a new area of focus, as NPAF has a strong track record of influencing policy changes for all patients. Rather, it represents an approach to leverage these successes to benefit a specific cohort of patients. NPAF continues to focus on cancer care and survivorship, while expanding to limited areas of cardiac care and diabetes identification and management. From a policy perspective, this approach provides a valuable opportunity for NPAF to partner with additional leaders in chronic disease management and survivorship to continue our collaboration so as to address mutual issues of concern that may limit access to care for patients on the state or federal level.

¹ Patient Protection and Affordable Care Act, §1302, p 45. http://frwebgate.access.gpo.gov/cgi-bin/getdoc.cgi?dbname=111_cong_bills&docid=f:h3590enr.txt.pdf. Accessed June 18, 2011

DOCUMENT GUIDE

Defined below are the terms and acronyms used frequently in the 2012 National Patient Advocate Foundation (NPAF) Policy Priorities. Reports, data, and positions presented in this document are current as of December 31, 2011. NPAF staff fully expects that these priorities will expand and contract based upon a myriad of factors of importance to the health policy arena, the patients and their needs that are served by the Patient Advocate Foundation (PAF), and the priorities and issues of importance to the entities that are aligned with NPAF's mission. It is truly a living document, and staff welcomes feedback and insights for additions as the year progresses.

“LEAD” NPAF Division

A total of 14 priority policy issues have been identified. Each has been assigned a “LEAD” NPAF Division (i.e., Federal Regulatory, Federal Legislative, State Regulatory/Legislative); however, it is readily acknowledged that most involve a range of legislative and regulatory activities at both federal and state levels forming a matrix of interconnected responsibilities and actions that cut across the entirety of NPAF's reach.

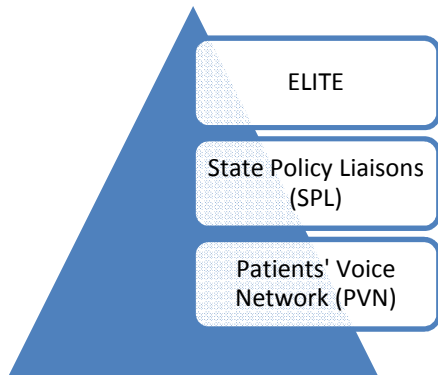
Priority Tier

Each policy issue has been assigned a priority tier based upon the alignment with NPAF's mission and the availability of resources, staff expertise, the advice of consultants, and the potential for impact, whether working solo or in collaboration with others.

- **Tier 1 Policy Priorities** are those priorities which align directly with NPAF's mission and require the greatest amount of NPAF resources, including staff time, data requests, lobbying activities, research, and strategic planning. They have the potential for meaningful impact on patients served by PAF and involve regular, proactive participation with legislators and regulators by NPAF staff working independently, or in concert with its volunteer networks, patient and provider coalitions, and outside consultants.
- **Tier 2 Policy Priorities** are those priorities which substantially align with NPAF's mission and include the direction or participation in coalitions and professional affiliations on a state and federal level to leverage the importance of patient-centric policy formation.
- **Tier 3 Policy Priorities** are those priorities which tangentially align with NPAF's mission and those priorities that arise during the year that need to be closely monitored but are still developmental. They have the potential to align NPAF's resources with other patient advocacy groups and grassroots' volunteer efforts.

NPAF Volunteer Network

Many of the 2012 Policy Priorities refer to the involvement of NPAF volunteers. These individuals are strategically positioned across the country and are trained, at NPAF's request, to provide varying degrees of interaction with policy officials. Volunteers are organized into three levels, including:



ELITE Volunteers – President's Council. Launched in August 2011, this top-tier level of volunteers includes approximately 75 carefully selected advocates who will lobby federal and state legislators, participate in town hall meetings for the 2012 Presidential Election and participate in the 2012 Patient Congress in Washington, DC.

State Policy Liaisons (SPLs) provide assistance with identifying state issues of importance, advocate on the state level through calls and correspondence to elected officials, and help grow the volunteer network.

Patients' Voice Network (PVNs) are the entry-level volunteers who express initial interest in the organization and perform non-political activities as needed (e.g., health fairs, booths).

Patient Data Analysis Report (PDAR)

The PDAR, issued annually, is a nationally recognized resource of trends in patients' experience with access to care from Patient Advocate Foundation's professional caseworkers. The PDAR reports on national and state-by-state healthcare access issues, including insurance obstacles, medical debt crises, and job retention issues confronted by patients in the United States and is a highly respected document in the Washington, DC policy arena. In 2010, the PDAR included data on 82,963 patients serviced by PAF, representing a 49.8 percent increase in patient cases compared to 2009. The PDAR serves as a valuable reference for healthcare policymakers and other stakeholders as they work to finalize and implement the Patient Protection and Affordable Care Act (PPACA). PAF holds an annual briefing on Capitol Hill when the PDAR is released and distributes copies to all Congressional offices, federal officials, and other stakeholders.

Regulatory Education and Action for Patients (REAP) Coalition

The Regulatory Education and Action for Patients (REAP) is an umbrella coalition composed of 47 patient advocacy groups and administered by NPAF. The coalition's goals are to strengthen current relationships and build new relationships with government agencies that are responsible for implementing provisions of the PPACA and other related regulatory changes and to ensure that implementation of these provisions is patient-centric. REAP's mission is to communicate the patient perspective on issues to federal and state regulatory bodies, Congress, healthcare insurers and others that regulate/develop/manage and/or impact healthcare delivery, coverage, cost and availability to the United States population.

United States Congressional Committees of Jurisdiction

Frequently, there are references to “Committees of Jurisdiction” in specific action items for 2012. These are the four Congressional committees that have jurisdiction over Medicare, Medicaid, the Department of Health and Human Services (HHS) appropriations, the Food and Drug Administration (FDA) reauthorization and other programs and budgets related to healthcare delivery in the United States:

- Senate Health, Education, Labor and Pension
- Senate Finance
- House Energy and Commerce
- House Ways and Means

NPAF also engages members of the House and Senate Appropriations Committees through One Voice Against Cancer (OVAC), which delivers a unified message to Congress and the White House on the need for increased cancer-related appropriations. To the extent necessary, NPAF will interface with and be informed by the Senate and House Judiciary Committees concerning the United States Supreme Court’s deliberations and rulings on the constitutionality of the “individual mandate” required by PPACA.

2012 NPAF Policy Priorities

Issue*	Priority Tier
Federal Regulatory	
Patient Protection and Affordable Care Act Implementation	
A. Pre-Existing Condition Insurance Plans ^a	1
B. Patient Rights to Appeal Denied Claims ^b	1
C. Essential Health Benefits	1
D. Premium Insurance Tax Credit	2
E. 340B	2
F. Personalized Medicine ^a (<i>Biosimilars, Genetic Testing</i>)	2
G. Supplemental Plans for Those Under Age 65	3
Federal Legislative	
Physician Reimbursement (Sustainable Growth Rate (SGR) + Average Sales Price (ASP)) ^a	1
Drug Shortages	1
Prescription Drug User Fee Act (PDUFA) and Medical Device User Fee Modernization Act (MDUFMA)	2
Chronic Disease Access to Care and Survivorship (e.g., Zadroga Act; Breast and Cervical Cancer Treatment Program; HEART Act for Women; Diabetes; Survivorship Planning; Rare Disease Drug Development and Approval) ^a	2
State Legislative and Regulatory	
State Health Exchanges	1
Essential Health Benefits	1
Oral Chemotherapy Parity ^a	2
Medicaid Stability and State Formularies ^a	3

* Issues are assigned a “lead” NPAF Division; however, most cut across disciplines of expertise.

^a Carried forward from 2011 Policy Agenda

^b Includes Independent NPAF White Paper

2012 NPAF Federal Regulatory Priorities

Tier 1

- **Patient Protection and Affordable Care Act (PPACA) Implementation**
 - **Pre-Existing Condition Insurance Plans (PCIPs)**

Background: The Trade Act of 2002 appropriated \$100 million for FY2003-FY2004 for state high risk pool funding.² Approximately 35 states have such state high risk pools. State high risk pool eligibility requires applicants to be “medically eligible” for coverage, i.e., an individual must have been denied coverage altogether, charged substantially higher premiums than the rest of the population, or offered basic coverage which excluded services related to treatment of a pre-existing medical condition.³

Section 1101 of PPACA established a federal temporary high risk pool for certain individuals with preexisting health conditions who have been uninsured for six or more months so that they might have health insurance coverage. The temporary pools, referred to as Pre-Existing Condition Insurance Plans (“PCIP”) will operate until 2014 when the prohibition on insurance plan pre-existing condition exclusions begins. Under the program, a new PCIP is established in each state. The Department of Health and Human Services (HHS) afforded states the following choices regarding the PCIP program: (1) operate a new high-risk pool alongside an existing state high-risk pool; (2) establish a new high-risk pool if the state did not currently have one; (3) build upon other existing coverage programs designed to cover high-risk individuals; (4) contract with current Health Insurance Portability and Accountability Act (HIPAA) insurance carriers or insurers of last resort to provide subsidized coverage; or (5) do nothing, in which case HHS would carry out the coverage program in the state.⁴ A total of 29 states plus the District of Columbia chose to operate their own plans, while the remaining 21 states elected to have the Department of Health and Human Services (HHS) administer the plan. To assure the plans are robust, they are required to offer comprehensive coverage with an actuarial value of 65 percent of total allowed cost and with out-of-pocket limits no higher than those permitted for high-deductible health plans accompanying health savings accounts.

Approximately five to seven million Americans are estimated to lack health insurance and have a pre-existing condition.⁵ Congress appropriated \$5 billion to fund the program through 2013, which many predicted would be an inadequate amount to fund the program. Initial predictions for enrollment were higher than what was experienced by the plans. The Congressional Budget Office (CBO) predicted the program to have an average enrollment of 200,000 per year. Even the Centers for Medicare and Medicaid Services (CMS) Office of the Actuary estimated that as many as 375,000 Americans would be covered by the program in 2010. A July 2011 General Accounting Office (GAO) report found that

² P.L. 107-210

³ Tanya Schwartz, Kaiser Comm’n on Medicaid and the Uninsured, Issue Paper, *State High-Risk Pools: An Overview*, Jan 2010, <http://www.kff.org/uninsured/upload/8041.pdf>

⁴ Jost, T. *Implementing Health Reform: Pre-existing Condition Coverage*. Retrieved December 21, 2011 from <http://healthaffairs.org/blog/2010/07/30/implementing-health-reform-pre-existing-condition-coverage/>

⁵ Health Policy Brief: Pre-Existing Condition Insurance Plan. Health Affairs and the Robert Wood Johnson Foundation; 2010. Available at: <http://healthreform.kff.org/scan/2010/august/policy-brief-explores-pre-existing-condition-insurance.aspx>

enrollment and spending for state- and federally run PCIPs have been significantly lower than initial projections. As of November 30, 2011, enrollment had exceeded 21,000, ranging from zero in one state to nearly 3,200 in another state; only 44,852 people have enrolled nationally.⁶

According to the GAO report released mid-2011, monthly premiums ranged considerably—from \$240 in Utah to \$1,048 in Alaska for a 50-year-old enrollee—and were generally lower in the federally run PCIPs.⁷ This outcome is particularly poignant given HHS efforts to increase enrollment by lowering the premiums. In late May, 2011, HHS reduced premiums in the 37 federally run PCIP states and increased its outreach efforts by allowing patients to provide a doctor's note indicating that they qualify for enrollment rather than having to provide evidence of denials of coverage as well as allowing brokers to receive payments for successfully enrolling new PCIP members. Spending was also lower than projected—about 2 percent of total program funding had been spent, or about \$78 million by state-run PCIPs and \$26 million for the federally run PCIP.

NPAF's Role in 2012: Time is of the essence regarding this provision of PPACA. NPAF will continue to monitor the enrollment in PCIPs and learn from PAF professional case managers how program administration may negatively impact patient enrollment, particularly as it relates to plan affordability. This is a signature program for NPAF, as NPAF led efforts to introduce legislation in 2007 and in 2009 addressing pre-existing conditions. NPAF has long been a champion in educating legislators and regulators on the barriers that having a pre-existing condition has on patients seeking to secure affordable and comprehensive health insurance.

NPAF will continue to advocate for the removal of barriers that having a pre-existing condition presents for patients. Rather than remain complacent by the hope that 2014 might afford complete relief with its pre-existing condition exclusion prohibition, NPAF will serve as a convener of patient advocacy groups to position this issue as an imperative issue that must be remedied in 2012. REAP will play a central role in that effort. NPAF will also partner with PAF as it provides patient quantitative as well as qualitative PDAR data that can guide federal policymaker efforts.

NPAF will meet with Center for Consumer Information and Insurance Oversight (CCIIO) officials on a quarterly basis to share its experiential insight regarding additional changes necessary to render the PCIP program a success, but more important, assure patients with pre-existing conditions are afforded the benefit Congress passed with PPACA.

Finally, NPAF will explore the feasibility of introducing federal legislation that would

- (1) allow patients covered under the benefit provisions of the **Consolidated Omnibus Budget Reconciliation Act (COBRA) of 1986**, as amended,⁸ to immediately enroll in PCIPs upon termination of benefits and forego the six-month waiting period; and

⁶ <http://www.healthcare.gov/news/factsheets/2012/01/pcip01132012a.html>

⁷ GAO. *Ibid*

⁸ Employers with 20 or more employees on more than 50 percent of its typical business days in the previous calendar year are subject to COBRA. Source: DOL Guidance; accessed January 23, 2012 at <http://www.dol.gov/dol/topic/health-plans/cobra.htm>

(2) charge the Centers for Medicare and Medicaid Services (CMS) with exploring the feasibility of offering enrollment in Medicare at full cost to the beneficiary during the six-month waiting period prior to PCIP eligibility as a mechanism to address the gap.

○ **Patient Rights to Appeal Denied Claims**

Background: Section 10101(g) of the Patient Protection and Affordable Care Act (PPACA) amends section 2719 of the Public Health Services Act to establish a federal right to appeal health insurance coverage determinations and claims for plans that are not grandfathered.⁹ Pursuant to the law, consumers have the right to appeal decisions to deny, reduce or cancel benefits made by group health plans and individual claim market plans. The law governs the process by which insurance companies administer initial appeals as well as consumer requests for reconsideration of payment denial decision processes. If an insurance company upholds its decision to deny payment, the law provides consumers with the right to appeal the decisions to an outside, independent decision-maker, regardless of the type of insurance or state residence of the enrollee.

The National Council of State Legislators explains the purpose of the law as follows:

“Because medical needs vary widely for patients and services vary significantly among providers, patients may face a denial or limitation on a particular treatment or receive what may be perceived as insufficient care. While many states already have “patient rights” laws, millions of insurance enrollees—especially those in self-insured and federally regulated plans—had no guarantee of an independent or “external” appeal process to address denial of coverage.”¹⁰

The Act’s right to appeal language created an important patient right that was at first interpreted favorably for patients by regulations promulgated in 2010¹¹ but was unfortunately somewhat eroded by a final rule issued in 2011.¹²

In July 2010, interim final regulations were published in a manner favorable to patients by

- expanding the adverse benefit determinations definition made subject to claims review,
- reducing the allowable maximum response time for urgent care decisions from 72 to 24 hours,
- requiring the inclusion of additional descriptive claim information in the notice of claims denial, and finally,

⁹ Public Health Service Act Sec. 2719, as added by Sec. 1001 of the PPACA, and folded into ERISA Sec. 715 and IRC Sec. 9815

¹⁰ National Council of State Legislatures, *Right to Health Insurance Appeals Process*, www.ncsl.org

¹¹ “Requirements for Group Health Plans and Health Insurance Issuers Relating to Internal Claims and Appeals and External Review Processes Under the Patient Protection and Affordable Care Act” *Federal Register* 2719 (23 July 2010): 43330-43364

¹² Group Health Plans and Health Insurance Issuers: Rules Relating to Internal Claims and Appeals and External Review Processes” *Federal Register* 76 (24 June 2011): 37208-37234

- providing a “strict adherence” standard which compels the insurer to comply with every aspect of the appeals process or risk the interpretation of a claimant having deemed exhaustion of his appeal rights. This interpretation enables the patient to be entitled to a *de novo* external review or raises his right to sue under section 502 of the Employee Retirement Income Security Act.

The 2011 final rule interpreted the same section of the law in a manner potentially *less* favorable to patients. Specifically, it

- provided beneficiaries less time to prepare their appeals,
- allowed plans to provide less information and explanation to patients about why the *claim* was denied, and
- limited the type of denials that could be challenged.

Essentially, the final rule returned to a 72 hour maximum response time for urgent care claims so long as the attending provider defines the claim as in need of “urgent care.” It also eliminated the requirement that insurers provide additional descriptive claim information for denied claims; however, it does require insurers to notify patients that they may request codes and their definitions in order to better understand why the claim was denied.

The final rule also precluded consumers from being able to appeal a denial to a outside entity if the compliance error was *de minimus*, or non-prejudicial, or attributable to good cause or matters outside of the plan’s control, or made in the context of an ongoing, good-faith exchange of information, or not reflective of a pattern or practice of noncompliance. NPAF is concerned that under the final rule, patients’ appeal rights may erode further or the confusion and inconsistencies in plans adherence to the final rule will lead to protracted or less stringent appeal processes that may limit access to care.

NPAF’s Role in 2012: NPAF will schedule quarterly meetings with officials at the Centers for Medicare and Medicaid Services (CMS) Center for Consumer Information and Insurance Oversight (CCIIO) and CMS coverage officials monitor the impact of protracted appeal processes on patients’ access to care as well as the impact of the final rule. During these meetings, NPAF will share quarterly PAF data regarding the real-time challenges patients are experiencing with their ability to avail themselves of the appeal processes. NPAF will develop a White Paper that educates policy leaders on this issue from a patient perspective quantified by Patient Data Analysis Report (PDAR) data.

Patient appeal rights is an issue that transcends disease categories. Therefore, NPAF will engage Regulatory Education and Action for Patients (REAP) in its efforts to assure the patient voice resonates within CCIIO as it considers whether the revised regulations impede patient access to care. REAP members will be encouraged to elicit information from their membership regarding whether the intent of Section 10101(g) of the PPACA is being realized by patients as the 2011 regulations are implemented. CCIIO members will be invited to address REAP members at a meeting in 2012 to review what its internal data reveals regarding the regulations and patient

ability to appeal health insurance coverage determinations and claims. Because there is such a close nexus between HHS' recently released bulletin which allows states to use existing health plans as benchmarks for benefits that must be offered in individual and small group health insurance plans inside or outside state exchange markets and the right of patients to appeal benefit denials, NPAF will plan to have a member of CCIIO discuss how it plans to track patient appeal data at an upcoming Policy Consortium.

Finally, to assure patients who are covered under public programs are also favorably positioned, NPAF will explore the feasibility of introducing federal legislation that would require CMS to define "reasonable and necessary" through a formal rulemaking process by 2014.

- **Essential Health Benefits (EHB)**

Background: PPACA creates state health exchanges ("exchanges"), which are virtual marketplaces where individuals and small businesses can compare health insurance plans as well as purchase health insurance on the state level. The products offered through exchanges are referred to as Qualified Health Plans (QHPs) which may vary in coverage levels yet are required to meet certain standards of care and achieve limits on patient cost-sharing. The PPACA directs HHS to specify the "essential health benefits" (EHBs) included in the "essential health benefits package" that QHPs will be required to cover (effective 2014) based on the scope of benefits offered by a typical employer plan.

The drafting of these EHBs are to be informed by a myriad of healthcare stakeholders, including the patient advocacy community. In addition, the PPACA requires HHS to periodically review the essential health benefits and address any gaps in access to coverage. As part of the deliberation process, HHS commissioned the Institute of Medicine (IOM) to make recommendations on its method to define the benefits to be included in the EHB package. The report was released in the summer of 2011 and garnered much attention from patient advocacy groups because of its attempt to strike the right balance between comprehensiveness and affordability.¹³ See www.npaf.org for NPAF comments regarding policy efforts to define essential health benefits.

On December 16, 2011, HHS issued a guidance document intended to give more flexibility and freedom to states to implement EHBs.¹⁴ (see section on "State Regulatory and Legislative" below) The guidance bulletin suggests an approach where states would be able to choose a benchmark plan that it deems reflective of a 'typical employer plan' in terms of services offered. The benchmark can consist of one of four options:

- 1) One of the three largest small group plans in the state by enrollment
- 2) One of the three largest state employee health plans by enrollment
- 3) One of the three largest federal employee health plan options by enrollment
- 4) The largest HMO plan offered in the state's commercial market by enrollment

¹³ Institute of Medicine. "Essential Health Benefits: Balancing Coverage and Cost." October 7, 2011. A report commissioned by the Department of Health and Human Services (HHS) to instruct on a method for the Department to define and update EHBs

¹⁴ Center for Consumer Information and Insurance Oversight. "Essential Health Benefits Bulletin." December 16, 2011. Accessed at: http://cciio.cms.gov/resources/files/Files2/12162011/essential_health_benefits_bulletin.pdf

NPAF's Role in 2012: NPAF, as the voice of the patient advocate community for over 15 years, has voiced its concerns regarding EHBs in both an HHS listening session as well as a White Paper widely distributed at policy meetings, Spring and Fall Policy Consortiums, and to members of Congress.¹⁵ The White Paper includes 16 recommendations, most importantly that the health benefit inclusion deliberation must consider the health consumer as a future patient. Other recommendations include that the process should consider patient debt crisis issues and be mindful of the severity of a patient's illness; that there should be proper education of consumers as to availability and real-world consequences of the benefit package; that any format determined for EHBs should be standard for comparability purposes, easy to understand, and unambiguous; and that EHBs should be comprehensive enough to include any and all health benefits that diagnose, including diagnostic imaging, treat and improve the health and well-being of patients suffering from chronic, debilitating and life-threatening diseases. Subsequent NPAF recommendations include requiring the essential health benefit definition to include 6 classes of drugs with 2 drugs per class to assure pharmacologic therapies reflect the patient populations' diversity in drug response and interaction.

In 2012, NPAF will seek to reassert these principles through comment on initial guidance documents, the proposed rule, and the final rule, as well as by issuing a revised White Paper in 2012 to be widely distributed. NPAF will make EHBs a top issue at the Spring 2012 Policy Consortium attended traditionally by 50 unique patient advocacy groups, providers, industry and federal officials. NPAF will also influence this issue by harnessing the power of the REAP members through their public comment process.

In 2012, there will be greater pressure on CMS to define "medical necessity," as the program pays for services deemed "reasonable and necessary."¹⁶ In 2012 NPAF will work with its Scientific Committee to help develop a definition of "medical necessity," that balances the needs of patients with the realities of affordability within the existing health care system.

Tier 2

- **Patient Protection and Affordable Care Act (PPACA) Implementation**
 - **Premium Insurance Tax Credit**

Background: The Patient Protection and Affordable Care Act (PPACA)¹⁷ and the Healthcare and Education Reconciliation Act of 2010¹⁸, afford individuals and small businesses the ability to purchase private health insurance through state-based competitive marketplaces called "Exchanges." Section 1401 of the PPACA amended the *Internal Revenue Code* by adding section 36B, which allows for a refundable premium tax credit to help individuals and families afford health insurance coverage.

¹⁵ National Patient Advocate Foundation. "Issue Brief: Essential Health Benefits and Patient Centricity." July 2011. This White Paper urged HHS to generate EHBs that will improve the health of consumers when they become patients

¹⁶ http://www.ssa.gov/OP_Home/ssact/title18/1862.htm

¹⁷ Public Law 111-148

¹⁸ Public Law 111-152

Although section 36B has been amended a number of times¹⁹ its purpose is to assure Qualified Health Plans (QHPs) offered through the Exchange are affordable by reducing a taxpayer's out-of-pocket premium costs. An Exchange makes an advance determination of credit eligibility for individuals enrolling in coverage through the Exchange and seeking financial assistance. Using information available at the time of enrollment, the Exchange determines (1) whether the individual meets the income and other requirements for advance credit payments, and (2) the amount of the monthly advance payments.

Health insurance premium tax credit eligibility is based upon meeting three criteria necessary to be considered an applicable taxpayer.²⁰ Applicable taxpayers are those who have household income for the taxable year between 100 percent and 400 percent of the Federal Poverty Level (FPL) for the taxpayer's family size. The 100 to 400 percent FPL for a single person in 2011 is \$10,890 to \$21,780. For a family of six, that same range equates to \$29,990 to \$59,980. The two other applicable taxpayer criteria are that the person may not be claimed as a dependent by another taxpayer and he or she must file a joint return if married.

The premium tax credit offers an incredible benefit to patients. The Congressional Budget Office estimates that, when the Affordable Care Act is fully phased in, the credit will help 20 million Americans afford health insurance. Patient Advocate Foundation's Patient Data Analysis Report reveals medical costs and financial burden as a central barrier to quality care for patients. Medical debt crisis was the largest category of PAF case management patient issues in 2010.²¹ NPAF recognizes the importance of assuring this section of the PPACA is codified in a manner that benefits patients and maintains the legislative intent of making health insurance coverage affordable for those who meet the statutory criteria. In August of 2011, the Department of the Treasury issued a proposed rule²² on its implementation of the health insurance premium tax credit and NPAF submitted comments²³ regarding the proposed rule's impact on patient tax credit eligibility, among other issues.

As stated above, patients who have access to affordable coverage elsewhere generally do not qualify for subsidized Exchange coverage. The Department of Health and Human Services (HHS) has interpreted section 36B(c)(i) of the *Code* which states an employee's employer-sponsored health plan is not affordable if "the employee's required contribution with respect the plan exceeds 9.5 percent of the applicable taxpayer's household income" narrowly. For an individual employee, employer-sponsored insurance coverage is considered affordable if the employee's share of the premium would consume less than 9.5 percent of family income. Unfortunately, the proposed rule uses the cost of employee-only coverage to determine whether a family has access to affordable coverage, even if the cost of the family coverage would qualify for eligibility. For illustrative purposes only, consider a family of six with an annual income of \$59,980, which as explained above is at 200% of FPL. According to the Kaiser Family Foundation and the Health Research Education Trust, the average annual premiums for employer-

¹⁹ Medicare and Medicaid Extenders Act of 2010, Public Law 111-309; the Comprehensive 1099 Taxpayer Protection and Repayment of Exchange Subsidy Overpayments Act of 2011, Public Law 112-9 ; and the Department of Defense and Full- Year Continuing Appropriations Act, 2011, Public Law 112-10

²⁰ Public Law 111-148, section 36B(c)(1)

²¹ Patient Advocate Foundation, 2010 Patient Data Analysis Report

²² Internal Revenue Service. "Health Insurance Premium Tax Credit." REG-131491-10. Posted to Federal Register August 17, 2011

²³ National Patient Advocate Foundation. "RE: Health Insurance Premium Tax Credit." Submitted October 31, 2011. This comment highlighted our concerns over eligibility and credit computation while suggesting potential solutions. Accessed at: <http://www.npaf.org/files/IRS%20Health%20Insurance%20Premium%20Tax%20Credit.pdf>

sponsored health insurance in 2011 are \$5,429 for single coverage and \$15,073 for family coverage. The employee would not qualify under the single coverage because the single coverage is \$5,429 or 9% of household income. However, if the employee were to purchase family coverage which would be \$15,073 or 25% of the family income, the family would still not qualify because *eligibility is based on employee-only coverage*. No member of the family would be eligible for premium tax credits or cost-sharing subsidies when one member has an affordable offer of employee-only coverage. The proposed rule essentially limits the definition of affordability solely to the cost of individual coverage and excludes the cost of family coverage which is more likely to exceed the 9.5% threshold. Pursuant to the regulation text, family coverage that exceeds the 9.5 percent threshold by *any* amount will be rendered affordable. This outcome is in direct opposition to the afore-stated goal of the PPACA to expand the number of Americans that have health insurance. There are other challenges with the tax credit such as ineligibility based on short term income changes like seasonal work or sporadic overtime pay as well as the resultant difficulty in determining eligibility as a result of Medicaid churning.

NPAF's Role in 2012: Affordability of health insurance is an important issue that affects all patients. NPAF will engage the collective voice of the Regulatory Education and Action for Patients (REAP) to elevate the importance of this issue to federal legislators. NPAF's approach will be a proactive one as it will educate federal policymakers on the unintended consequences of the proposed rule before the final rule is promulgated. NPAF is concerned that the policy leaders at the Department of Treasury are not well versed in health access issues. NPAF will once again assume its leadership position in educating policymakers regarding the unintended consequences of a well-intended proposed rule. NPAF will comment again when a final rule is issued in 2012, and will draft materials to educate consumers about the opportunities and process. Once education materials have been developed with PAF Mission Delivery, NPAF will utilize the resources at its disposal, including public affairs consultant Schmidt Public Affairs, the State Policy Liaison (SPL) and Patient Voice Network (PVN) volunteer network, social media, the NPAF website, op-eds, etc. to educate the general public about the availability of the tax credit and the parameters for eligibility. As noted above, NPAF will also influence this issue by harnessing the power of REAP members, utilizing this broad coalition to educate consumers.

- **Section 340B of the Public Health Service Act**

Background: In 1992, The Veterans Healthcare Act amended section 340B of the Public Health Service (PHS) Act and created a program known as the 340B Program.²⁴ The 340B Program requires drug manufacturers participating in Medicaid to provide discounted covered outpatient drugs to certain eligible entities, known as covered entities. The Congressional intent of the program was to allow savings from 340B Program discounted drugs purchases “to enable [participating] entities to stretch scarce Federal resources as far as possible, reaching more eligible patients and providing more comprehensive services.”²⁵ It is clear that the program was not to benefit every provider but rather to support providers caring for the medically underserved. This intent is evidenced in Committee language that stated, “The Committee bill also provides protection from drug price increases to specified Federally-funded clinics and public hospitals that provide direct clinical care to large numbers of uninsured Americans.”²⁶ The statutory language supports an interpretation of Congressional intent that

²⁴ Veterans Healthcare Act of 1992, P.L. 102-585 § 602; PHS Act § 340B; 42 U.S.C. § 256b

²⁵ H.R. Rep. No. 102-384, at 12 (1992)(Conf. Rep.)

²⁶ H.R. Rep. No. 102-384 (II)

originally the 340B beneficiary was to be an entity that served the medically underserved as narrowly defined as those patients not eligible for Medicare and Medicaid. “Private, not-for-profit hospitals meeting other eligibility requirements must have a contract with state or local governments to “provide healthcare services to low income individuals who are not entitled to benefits under Title XVIII [Medicare] of the Social Security Act or eligible for assistance under the State plan under this subchapter [Medicaid].”²⁷

The Medicaid rebate program requires drug companies to enter into a Rebate Agreement with HHS as a precondition to conducting business with Medicaid. In addition, each manufacturer with a Rebate Agreement must sign a Pharmaceutical Pricing Agreement with the Health Resources and Services Administration’s (HRSA) Office of Pharmacy Affairs agreeing to sell all of its drugs at an upfront discount to covered entities identified in Section 340B for use in those providers’ *outpatient* operations. Under ordinary circumstances, drugs purchased under the 340B Program are not to be used in an inpatient setting. The required 340B discount is designed to approximate the net price that Medicaid pays for drugs through the operation of the rebate program. As a result, most 340B entities can purchase the expensive single source drugs and biologics used in the treatment of cancer at discounts that average about 50 percent.

The 340B Program does obligate 340B entities to bill Medicaid at the 340B acquisition price or, alternatively, to buy drugs used by Medicaid patients at commercial prices so that manufactures are not placed in the position of selling a drug to the 340B entity at discount and then having to further discount the drug through the payment of a Medicaid rebate on its utilization. Some 340B entities are now aggressively negotiating with state Medicaid programs to be allowed to share the 340B discount with the state in the name of giving the 340B entities even more funding to further their missions. Concerns have been voiced that some 340B disproportionate share hospitals have stretched the intent of the program beyond the generation of a profit to support non-drug aspects of the entity’s mission by treating prison inmates in state jails as entity patients and continuing to supply the prisons with discounted drugs for in-prison use. A few states have also tried to co-opt the 340B Program as a way to obtain deeply discounted drugs for state employees enrolled in the state’s employee health benefit plan.

While inappropriate use of the program has not been documented, there are claims that some entities may encourage the use of observation services before a patient is admitted to a hospital rendering the hospital ineligible for the 340B drug discount. While NPAF supports the appropriate use of observational services, it will encourage federal policymakers to assure admission preferences are aligned with optimal patient care.

Second, although neither a correlation nor causation has been established, there need to be assurances that inappropriate use of the 340B Program is not contributing to the nation’s drug crisis. The 340B Program has afforded some entities an advantage financially that does not provide the maximum

²⁷ 42 U.S.C. 256b(a)(4)(L)(i)

benefit to patients intended by Congress. A recent Government Accounting Office (GAO) report evidences greater interest in the program and the oversight responsibility of the Health Resources and Services Administration by members of Congress.²⁸

The GAO report found the Department of Health and Human Services' (HHS) oversight of the 340B Program to be inadequate because, among other reasons, it engaged in few activities to oversee the 340B Program. For example, HHS does not periodically confirm eligibility for all covered entity types, and has never conducted an audit to determine whether program violations have occurred. This lack of oversight means there is no evidence to substantiate the benefit patients are deriving from the 340B Program, particularly in light of the perceived cost-shifting that occurs. The aforementioned patient challenges associated with the 340B Program are substantial ones as evidenced by the GAO's report on the growth of the 340B Program. There are currently over 16,000 participating covered entity sites in the 340B Program.

NPAF's Role in 2012: NPAF staff will continue to partner with an array of stakeholders to assure the 340B Program savings inure to intended beneficiaries by actively participating in a 340B workgroup. The workgroup is an informal effort on the part of multi-sector, private, and not-for-profit organizations that share a common interest in ensuring that the 340B Program accomplishes its intended mission—access to discounted, outpatient pharmaceuticals for the benefit of indigent, uninsured patients—in ways that avoid unintended consequences injurious to other key stakeholders in the overall healthcare environment. Its goal is to explore participants' thoughts and concerns regarding the program, and to educate policy- and law-makers as to the common concerns of its member organizations. In 2012, the workgroup will work toward becoming a coalition.

NPAF will also educate REAP membership on the importance of assuring the 340 program remains consistent with its original goal to provide pharmaceutical access to the medically underserved and how members might likewise participate in the newly formed coalition. The patient voice is a particularly powerful one when one considers the Congressional intent of the 340B Program. The workgroup has met with Congressional staff, all of whom were most interested in the challenges the current administration of the 340B Program places on patients. The workgroup is interested in revising the 340B Program definition of patient in 2012 and greater representation of patient advocate groups will assure the definition is appropriately comprehensive yet thwarts the current undue 340B program expansion.

- **Personalized Medicine** (*Follow-on Biologics or Biosimilars and Genetic Testing*)

Personalized medicine is a term used to describe the consideration of characteristics such as age, coexisting conditions, preferences, and beliefs in crafting an individual management strategy; the use of advanced individual genomic information in choosing an expensive biologic agent; and the development of therapies biologically tailored to patient needs, such as customized monoclonal antibodies and vaccines.²⁹

²⁸ GAO, *State Medicaid Policies and Oversight Activities Related to 340B-Purchased Drugs*, GAO-09-296 (Washington, D.C.: June 2011)

²⁹ Garber, A and Tunis, S., Does Comparative-Effectiveness Research Threaten Personalized Medicine? *N Engl J Med* 2019 360:1925-1927

Several sections of the Patient Protection and Affordable Care Act (PPACA) are supportive of research that will support personalized medicine as it offers great patient benefit and may reduce healthcare costs.³⁰ The PPACA builds upon the Institute of Medicine document, entitled “*Crossing the Quality Chasm*” which offers six aims for health care center improvement, one of which encourages the patient-centered care, of which personalized medicine is an example. “Patient-centered” is defined in the report as “providing care that is respectful of and responsive to individual patient preferences, needs, and values and ensuring that patient values guide all clinical decisions.”³¹ NPAF is committed to contributing patient perspectives of safety, costs and surety of supplies to national dialogues on this issue.

Personalized medicine may also reduce health care costs. According to NIH Chief Francis Collins, “While [personalized medicine] may not bend the cost curve overnight, it is something that everyone agrees we should do more of, if we're serious both about giving people a chance to live long and healthy lives and also reducing health care costs.” A keen understanding of health care costs is imperative in effective health policy development. The issue of cost is often complex, as reducing costs in one area often results in elevated costs in another area. NPAF’s policy activities in personalized medicine will reflect an understanding of not only costs to the patient, but the downstream costs associated with any activity that improves access to care.

The PPACA sections most likely to elevate the application of personalized medicine are those concerning biologics, particularly section 7001 which establishes a new regulatory authority within the Food and Drug Administration (FDA) by creating a licensure pathway for follow-on biologics. A biologic is a preparation, such as a drug or a vaccine that is made from living organisms. A *follow-on biologic* is similar but not identical to the brand name or innovator product made by the pharmaceutical or biotechnology industry; *biosimilar* is the term used in the European Union.³²

Policies regarding patient access to follow-on biologics are directly impacted by laws affecting their manufacturing and marketing. The PPACA provides direction regarding both. Section 7001 of the PPACA establishes the Biologics Price Competition and Innovation Act (BPCIA) which limits exclusivity for new biologics to twelve years, compared to 20 years for traditional pharmaceuticals. Data exclusivity is the period of time during which the manufacturer of a follow-on product, in the preparation of its application for Food and Drug Administration (FDA) approval, is blocked from referring to the data submitted in the original application to FDA for the approval of the brand-name product. This results in a period of exclusive marketing for the brand-name product. The law provides a 12-year exclusivity period from the date on which the reference product was first approved. If a reference product has been designated an orphan drug, an application for a biosimilar or interchangeable product may not be filed until the later of (1) the seven-year period of orphan drug exclusivity described in the Federal Food, Drug, and Cosmetics Act (FFDCA) or (2) the 12-year period established by the Senate bill.³³ Thus, brand-name biologic companies are afforded a 12-year period during which generic companies would be barred from introducing a competing product, known as a follow-on biologic or biosimilar.

³⁰ P.L. 107-210

³¹ *Crossing the Quality Chasm: A New Health System for the 21st Century*. Washington, DC: The National Academies Press, 2001

³² CRS. *FDA Regulation of Follow-On Biologics* CRS-7-5700 (Washington, D.C.: April 2010)

³³ CRS, *Ibid*

The new regulatory pathway is premised upon the FDA’s authority for approving generic chemical drugs under the Drug Price Competition and Patent Term Restoration Act of 1984.³⁴ This law allows the generic company to establish that its drug product is chemically the same as the already approved innovator drug, and therefore relies previous FDA finding of safety and effectiveness for the approved drug. This approach allows the generic drug industry to achieve cost savings by avoiding the expense of clinical trials. Savings are also achieved by foregoing the initial drug research and development costs that were incurred by the brand-name manufacturer.

While patient savings are important, NPAF supports efforts that provide assurances new biosimilar development has the same clinical trial rigor as the initial product. Safety and outcomes considerations are imperative as serious concerns have arisen whether biosimilars are atomically identical to their reference as are generic small-molecule drugs. NPAF wholeheartedly endorses the NCCN recommendation that policymakers draft biosimilar regulations that assure 1) a biosimilar product is indeed “highly similar” to the reference product and 2) systems are established to identify and mitigate any unintended consequences. This approach offers the assurance that potential biosimilar savings do not put any patient, and particularly cancer patients, at risk.

Other sections of the PPACA also support clinical advancements aligned with personalized medicine. Section 3113 of PPACA authorizes the Secretary to conduct a demonstration project to allow separate payment under Medicare Part B, at rates to be determined by the Secretary, for covered complex diagnostic tests (as defined by the Act) that link a patient’s genetic makeup to a cancer chemotherapy where no alternative test is available having equivalent performance characteristics, under certain limited circumstances.

Advancements such as these are often discussed in the context of Comparative Effective Research (CER) defined by the Department of Health and Human Services (HHS) Agency for Healthcare Research and Quality (AHRQ) as, “research designed to inform health-care decisions by providing evidence on the effectiveness, benefits, and harms of different treatment options.” While most agree on the value of personalized medicine, concerns have been raised that outcomes of CER will influence coverage decisions, thus, limiting the discretion of physicians and restricting patients’ access to different treatments.

Section 3011 of PPACA prioritizes the establishment of a national strategy to improve the delivery of healthcare services, patient healthcare outcomes, and population health. The national strategy must enhance data by integrating comparative effectiveness information. CER is the cornerstone of section 6301 which establishes a nonprofit corporation to be known as the Patient-Centered Outcomes Research Institute (PCORI). PCORI assists in the analysis of the health outcomes and the clinical effectiveness, risks, and benefits of more medical treatments such as therapies, diagnostic tools, and pharmaceuticals (including drugs and biologicals). Congress provided it a strong personalized medicine component by requiring PCORI research to consider the potential for differences in the effectiveness of healthcare treatments in various subpopulations.

³⁴ P.L. 98-417

The availability of personalized medicine and its resultant health information cannot be used to penalize patients who have pre-existing conditions. Section 1201 of the PPACA prohibits denial of healthcare coverage based on the individual's genetic information.

NPAF's Role in 2012: NPAF will straddle a policy approach regarding personalized medicine that protects patients yet encourages pharmaceutical innovation. This approach will be implemented in a manner that assures the multivariate issue of cost is considered and informs each activity. NPAF's approach will encourage federal agencies to collaborate because neither goal will be achieved without such collaboration. NPAF will work hard to assure Food and Drug Administration (FDA) Commissioner Margaret Hamburg's promise outlined in the *New England Journal of Medicine* regarding personalized medicine is kept. Commissioner Hamburg states, "To make progress, the National Institute of Health (NIH) and the FDA will invest in advancing translational and regulatory science, better define regulatory pathways for coordinated approval of co-developed diagnostics and therapeutics, develop risk-based approaches for appropriate review of diagnostics to more accurately assess their validity and clinical utility, and make information about tests readily available."³⁵

NPAF will work closely with Regulatory Counsel Larri Short in assuring the complex policy issues that are inherent to personalized medicine are not compromised at the peril of patients. NPAF will also assure resultant regulations do not frustrate the right to hope that new pharmaceutical innovations represent for patients. Once again, these goals will be accomplished in a manner that elevates the role cost plays in successful policy development.

Personalized medicine provides great promise for patients, no matter the disease they suffer. Therefore, NPAF will monitor the popular press, journal articles as well as AHRQ's priorities for CER evidence-based outcomes and PCORI's research agenda to assure that critical findings are not inappropriately used for coverage determinations, but rather advance clinical decision-making for patients with chronic, debilitating diseases. NPAF recognizes that clinical-decision making does not occur in an economic vacuum and will consider the cost of furthering personalized medicine. Although still in developmental discussions, the concept of personalized medicine holds great promise for patients and NPAF will call upon its Scientific Advisory Committee for ongoing guidance as new research findings emerge. NPAF should also monitor the PCORI website to not only identify possible future funding streams for research elevating the PDAR, but to educate PCORI staff of the utility of PDAR data in identifying patient-centric issues.

NPAF has considerable interest in the application of personalized medicine in the development of biosimilars and the FDA's regulation of biosimilars. NPAF is a member of a biologics coalition comprised of a broad array of stakeholders. It will capitalize on its membership by identifying a multitude of issues for submission of comments on FDA's Biologics Price Competition and Innovation Act of 2009: Options for a User Fee Program for Biosimilar and Interchangeable Biological Product Applications for Fiscal Years 2013 – 2017. NPAF will ensure that approval of pioneer biotechnology products is based on rigorous standards of safety, purity and potency recognizing that clinical trial evidence and data are fundamental for evaluating and demonstrating safety and effectiveness of a follow-on biologic, and must be required for all biosimilars.

³⁵ Hamburg, M. and Collins, N. The Path to Personalized Medicine. *N Engl J Med* 2010; 363:301-304

Other NPAF activities include continued participation in the development and periodic updates to the National Comprehensive Cancer Network (NCCN) White Paper concerning biosimilars as well as participating in future NCCN Oncology Policy Summits on Biosimilars. Related activities will involve reviewing white papers from a range of stakeholders to keep abreast of developments in this area.

Finally, NPAF will monitor products going through parallel review so that the concept of “minimal benefits” considered in the Centers for Medicare and Medicaid Services (CMS) National Coverage Determination process does not become a standard for coverage as it would threaten beneficiary access to medically necessary items and services.

The FDA has recently released guidance regarding its proposed approach to determining biosimilarity and the critical scientific and quality issues that members of the pharmaceutical and biologic industry will need to address when they submit their follow-on biological products applications. Those guidance documents can be accessed at:

Scientific Considerations in Demonstrating Biosimilarity:

<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM291128.pdf>

Quality Considerations in Demonstrating Biosimilarity:

<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM291134.pdf>

Question and Answers Regarding the Implementation of the BPCI Act of 2009:

<http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM273001.pdf>

Tier 3

- **Patient Protection and Affordable Care Act (PPACA) Implementation**

- **Supplemental Plans for Those Under Age 65**

Background: To help pay for some of Medicare’s cost-sharing requirements as well as some benefits not covered by Medicare parts A or B, most Medicare beneficiaries have some type of supplemental coverage. Privately purchased Medigap is an important source of this supplemental coverage. Medigap policies are available for all Medicare beneficiaries, and some are tailored to people under age 65 who are eligible for Medicare because of a disability or end-stage renal disease. Federal law does not require insurance companies to offer Medigap policies; however, 29 states do require that insurance companies operating within their state offer at least one plan. Medigap insurance (also known as Medicare Supplement Insurance) is voluntary and patients are responsible for the monthly or quarterly premium. Not all plans are available in all areas however, and three states – Massachusetts, Minnesota and

Wisconsin – have their own Medigap policies that are different from the standard Medigap plans. There should be state uniformity in Medigap policies available to individuals between the age of 55-65 enrolled in Medicaid because of disability or end-stage renal disease.

NPAF's Role in 2012: NPAF will work in conjunction with advocacy partners, specifically the kidney disease and renal failure organizations, (e.g., National Kidney Foundation (NKF), End-Stage Renal Disease (ESRD) Programs) to educate state insurance commissioners and encourage more states to require Medigap plans for the under-65 and disabled. In addition, NPAF will look for opportunities in the pending state health “exchanges” for these patients to be served. Finally, NPAF will focus on how this issue is addressed in efforts to better align Medicare and Medicaid benefits for patients eligible for both programs, known as dual eligibles. There will be considerable attention paid to this population in 2012 federal health policy activities.

2012 NPAF Federal Legislative Priorities

Tier 1

Physician Reimbursement and its Impact on Patients' Access to Care

Introduction: For the last 15 years, NPAF has expressed concern that reductions in Medicare reimbursement rates to physicians will ultimately threaten the sustainability of facilities and providers who deliver care to Medicare beneficiaries and result in a reduction in access to care. In 2010, Medicare patients represented one out of four beneficiaries served by PAF (a 31.18 percent increase from 2009); approximately two-thirds of them living on annual, household incomes of less than \$23,000.³⁶ The decision by some physicians not to accept Medicare beneficiaries because of low reimbursement rates is particularly worrisome for beneficiaries living in rural settings who may already have limited access to care.

In 2004, NPAF established the Global Access Project (GAP), a research forum whose mission is to conduct independent research projects designed to provide insights in reimbursement, access to care, national trends in clinical trials, changes in treatment sites, and research to define deficiencies in coding during both the period before and after the enactment of the Medicare Modernization Act (MMA). A GAP study conducted by Duke University Clinical Research Institute and published in *JAMA* (July 2008) concluded that beneficiaries in rural communities tended to have to wait longer to initiate chemotherapy after diagnosis than before the bill passed; wait times increased as much as five times from 2003-2006.³⁷ In short, cuts to physician reimbursement provides doctors with less incentive to treat Medicare beneficiaries and is creating decreased access to necessary care for Medicare beneficiaries in the community.

Two critical issues are essential to maintaining physician reimbursement at levels that do not compromise patients' access to care: the Sustainable Growth Rate (SGR) and Average Sales Price (ASP).

Background (SGR): Section 4503 of the Balanced Budget Act of 1997³⁸ established the Sustainable Growth Rate (SGR) provision. SGR is the formula that determines how much Medicare providers are compensated for treating beneficiaries. SGR was established to provide annual updates to physician reimbursement for services, but because rates are based on Gross Domestic Product (GDP) and not actual healthcare practice costs, physician expenditures often exceed the SGR and as a result, their reimbursement rates are cut.³⁹ Medicare providers were scheduled to absorb a 27 percent cut in reimbursement on January 1, 2012, which is when a short term patch to avoid cuts was scheduled to expire. Congress narrowly avoided this deadline by passing a two-month SGR patch as part of the payroll tax cut extension on December 23, 2012, making doctors wait until the last minute to determine whether they would be able to financially continue to see Medicare beneficiaries in 2012.

³⁶ Patient Advocate Foundation. "2010 Patient Data Analysis Report". Pg 27, April, 2011

³⁷ NPAF Global Access Project. "Association between the Medicare Modernization Act of 2003 and Patient Wait Times and Travel Distance for Chemotherapy." Duke University Clinical Research Institute (*JAMA*, 2008). 300(2):189-196. doi: 10.1001/jama.300.2.189.

³⁸ Pub. L. 105-33

³⁹ American Medical Association, "Medicare and the Sustainable Growth Rate"

Legislation has overridden the formula's results in each of the past four years, and the prospect of future, year-after-year rate reductions raises the question of whether the SGR formula is a viable mechanism—and if not, what alternatives might be appropriate. Each year that Congress postpones repealing the SGR, the formula's deficit grows. In 2005, the formula could have been repealed for less than \$50 billion; today the number stands at just under \$300 billion.⁴⁰ The American Medical Association (AMA) estimates that by 2016 the cost of permanent repeal will be \$600 billion.⁴¹ On November 21, 2011, The Joint Select Committee on Deficit Reduction ("Super Committee") failed to come to agreement on \$1.2 trillion in cuts to the budget over ten years and many groups, including NPAF, had urged the Committee to permanently correct SGR in their agreement. NPAF has consistently advocated for a permanent fix (repeal) of the SGR citing concerns over patient access to care.

NPAF's Role in 2012: Due to the failure of Congress to permanently repeal SGR in 2011 and the subsequent passage of the two-month patch, NPAF will continue to work in close partnership with a broad range of stakeholders, including leading provider organizations such as the American Society of Clinical Oncology (ASCO), US Oncology (USON), the Association of American Cancer Centers (ACCC) and the Community Oncology (COA), to influence policy makers on Capitol Hill through direct lobbying and a Congressional hill briefing to emphasize the impact of physician reimbursement and continual threats to significantly reduce the level of reimbursement on beneficiaries' access to care. SGR will be a top priority message to Congressional offices of health jurisdiction and NPAF will continue to provide Patient Data and Analysis Reports (PDARs), patient stories and de-identified patient cases to highlight the consequences patient face with this unresolved issue.

The impact of physician reimbursement cuts on patient access to care will also be the top priority issue for NPAF's 13th Annual Patient Congress during 2012, where patients, caregivers, healthcare professionals and non-profit patient advocacy representatives participate in the legislative process by meeting with their elected officials in Washington, DC. NPAF will continue to work with a SGR coalition and national provider organizations to engage colleagues in the patient advocacy community to reiterate the consequences of continued cuts to Medicare provider reimbursement referencing 2011 PDAR Medicare data. NPAF will also participate in high level SGR discussions through the Coalition for Affordable Health Coverage (CAHC), a broad-based coalition whose membership includes organizations representing small and large employers, manufacturers, insurers, brokers and agents, physician organizations and individual consumers.

Background (ASP): Medicare Part B helps cover medically-necessary services like doctor's services and outpatient care. Part B reimburses medications administered in community oncology clinics, where 80 percent of cancer beneficiaries receive treatment, at Average Sales Price +6 percent (ASP+6). Recent discussions by Congress to reduce the budget deficit have included the possibility of reducing the ASP payment approach under Medicare Part B from ASP +6 to ASP +3 percent, or lower.

⁴⁰ American Medical Association. "SGR Repeal and the Joint Select Committee on Deficit Reduction Frequently Asked Questions"

⁴¹ *Ibid*

NPAF is concerned that this approach would discourage providers from offering services to Medicare beneficiaries, especially in rural areas. A recent study conducted by the Community Oncology Alliance entitled “*Community Oncology Cancer Care Practice Impact Report – Documented Impact on Community Oncology Practices*” reported that 199 community-based cancer clinics have closed in the past three years and 369 practices, with multiple clinic locations, are struggling to remain open.⁴² These closures force provider consolidation into urban hospital systems which forces beneficiaries to travel longer distances to receive care, and absorb more out-of-pocket expenses. Another study performed by Milliman entitled “*Site of Service Cost Differences for Medicare Beneficiaries Receiving Chemotherapy*” found that receiving chemotherapy in a hospital outpatient setting costs \$6,500 more per patient every year than receiving care in a physician’s office. Additionally, patient co-pay amounts were found to be approximately 10 percent higher for outpatient care, which totaled more than \$650 per patient per year.⁴³ Simply put, the study showed total Medicare spending on chemotherapy beneficiaries to be significantly lower in the physician’s office, amounting to an extra \$623 million saved per year; cutting ASP is more expensive for both beneficiaries and the entire healthcare system.

NPAF’s Role in 2012: NPAF will work collaboratively with members of the ASP Coalition, which includes approximately 20 healthcare stakeholder groups, to influence legislators to consider the impact that cuts to ASP has on beneficiaries, especially those Medicare beneficiaries diagnosed with chronic, debilitating and life-threatening diseases whose conditions make changes in point of service disruptive or impossible to meet due to travel constraints or economic hardship.

NPAF will continue to participate in Hill meetings with Congressional leadership in the health committees of jurisdiction, partake in ASP Coalition lobby days throughout 2012 and participate in the development of various media mechanisms to educate the public on this issue. As with SGR, NPAF will incorporate ASP within the impact of physician reimbursement cuts on patient access to care during NPAF’s 13th Annual Patient Congress and will coordinate a Congressional hill briefing on this issue. Finally, NPAF will provide subject briefings and issue seminars to other patient advocate groups, policy makers and national level opinion leaders about the potential challenges Medicare patients face if reimbursement to physicians results in their discontinuation of services.

- **Drug Shortages**

Background: The abrupt shortage or discontinuation of a drug can have a disastrous impact on patients, especially cancer patients and those in need of intravenous (IV) nutritional supplements. Shortages in historically effective chemotherapy agents, such as Cytarabine, Cisplatin, Doxorubicin and other agents have been widely reported, with many lacking an effective substitute. Alarming, the number of new prescription drug shortages increased from 211 in 2010 to 267 in 2011.^{44 45} Many causes of the shortage have been cited, including little or no incentive for industry to manufacture low cost generics, manufacturing and quality problems, delays and discontinuations, lack of active pharmaceutical ingredients and consolidation of drug manufacturers.

⁴² Community Oncology Alliance – Community Impact Report Updated March 2011 - <http://www.communityoncology.org/COAStudies.aspx>

⁴³ Milliman, Inc., NY. “Site of Service Cost Differences for Medicare Patients Receiving Chemotherapy.” October 19, 2011

⁴⁴ University of Utah Drug Information Services, 1/3/2012

⁴⁵ Government Accountability Office. “Drug Shortages – FDA’s Ability to Respond Should be Strengthened.” December 15, 2011

On October 31, 2011 President Obama issued Executive Order 13588, “Reducing Prescription Drug Shortages” that directs the Food and Drug Administration (FDA) to take steps that will help to prevent and reduce current and future disruptions in the supply of lifesaving medicines.⁴⁶ Congress has also taken an active interest in this issue. The Preserving Access to Life-Saving Medications Act of 2011 was introduced by Senators Amy Klobuchar (D-MN) and Robert Casey (D-PA) on February 7, 2011 and Reps. Diana DeGette (D-CO-01) and Tom Rooney (R-FL-16) on June 21, 2011. These bills require manufacturers to notify FDA within 180 days of a shortage and to provide the FDA with improved capacity to prevent drug shortages.⁴⁷ The FDA would be required to publish notifications of shortages on the FDA website, and under the House bill, manufacturers that fail to submit appropriate notification within 180 days will be subject to a penalty of up to \$10,000 for each day of the violation up to \$1.8 million (the Senate bill establishes that Department of Health and Human Services (HHS) regulations will schedule civil penalties).

A Government Accountability Office report entitled “*Drug Shortages – FDA’s Ability to Respond Should be Strengthened*” found that many causes of drug shortages were the result of manufacturing problems and that FDA lacks the authority to require manufacturers to provide the agency and the public with information about shortages, or require that manufacturers take certain actions to prevent, alleviate, or resolve shortages.⁴⁸

Senator Orrin Hatch (R-UT) has explored the possibility of increasing Medicare reimbursement for drugs in short supply and is currently developing legislation to that effect. Senator Hatch is working to define which “medically necessary” drugs would be eligible for incentives, how many of these incentives manufacturers would be eligible for and other details.⁴⁹ Tax credits have also been suggested to build manufacturing facilities or upgrade aging factories. In addition, Senator Charles Schumer (D-NY) has announced he intends to introduce legislation that would make it a federal crime to engage in prescription drug price gouging when drugs are in short supply in an effort to eliminate the “gray market”. Both bills are expected to be introduced early 2012.

NPAF’s Role in 2012: There is a strong possibility that any drug shortage legislation will be attached to the Prescription Drug User Fee Act (PDUFA) reauthorization. However, many experts believe the immediacy and visibility of the issue will force Congress to take action well before the passage of PDUFA. NPAF will continue to respond to requests from Congressional committees of jurisdiction for meetings to explore solutions and work with FDA, and the Centers for Medicare and Medicaid Services (CMS) to find resolution to the drug shortage problem. NPAF discussions will rely heavily upon the guidance of the NPAF Scientific Advisory Committee, chaired by Dr. Marc Stewart of the Fred Hutchinson Cancer Research Center. Solutions will also be based on PDAR data, patient stories, provider experiences, and de-identified patient cases specifically addressing the cost burden and deleterious impact patients face forced to switch to a higher price brand medication due to a shortage of a lower-priced generic medication.

⁴⁶ President Barack Obama, “Executive Order 13588 -- Reducing Prescription Drug Shortages.” October 31, 2011

⁴⁷ S. 296 “Preserving Access to Life-Saving Medications Act of 2011” Senators Amy Klobuchar and Robert Casey, February 2, 2011

⁴⁸ Government Accountability Office. “Drug Shortages – FDA’s Ability to Respond Should be Strengthened.” December 15, 2011

⁴⁹ American Society of Hematology, “Senate Working on Drug Shortage Bill that Includes Reimbursement Changes”, November 15, 2011

NPAF will provide consultation, insight and education in regard to drug shortages for patients to national policy makers. NPAF will coordinate a Congressional briefing in 2012 to raise awareness of the need for transparency among providers and patients when drugs are in short supply or unavailable. NPAF will also continue to collaborate with Regulatory Education and Action for Patients (REAP) which submitted a principles document on drug shortages from the patient perspective to Congressional and regulatory leaders. The document called out that patients, physicians and the general public are entitled to receive meaningful advance warning of potential drug shortages and to be told when an anticipated shortage has materialized, how long that shortage is expected to last, and the treatment implications of the shortage.⁵⁰

Tier 2

Prescription Drug User Fee Act (PDUFA) and Medical Device User Fee Modernization Act (MDUFMA)

PDUFA

Background: Two decades ago, the Food and Drug Administration (FDA) suffered from a severe backlog of new drug applications which was obstructing countless potentially lifesaving medicines from reaching patients. To address this crisis, Congress passed the Prescription Drug User Fee Act (PDUFA) in 1992 which was intended to address the severe backlog of new drug applications. PDUFA was renewed in 1997 (PDUFA II), 2002 (PDUFA III), 2007 (PDUFA IV) and is scheduled to be reauthorized in 2012. PDUFA authorizes FDA to collect fees from companies that produce certain human drug and biological products. Since the passage of PDUFA, user fees have played an important role in expediting the drug approval process. In FY2003, FDA's human drug review program received approximately \$200 million in user fee funding. That figure has increased every year since and reached approximately \$600 million in FY2010, comprising 62 percent of the program's funding.⁵¹

On September 1, 2011, the Department of Health and Human Services (HHS) released the Prescription Drug User Fee Act (PDUFA) Reauthorization Performance Goals and Procedures for fiscal years 2013 through 2017, which increased user fees by 6 percent. NPAF was pleased to see that the agreement was consistent with NPAF recommendations in addressing promoting innovation through enhanced communication between FDA and sponsors during drug development, advancing development of drugs for rare diseases, measuring the effectiveness of Risk Evaluation and Mitigating Strategies (REMS) and standardizing and better integrating REMS into the healthcare system.⁵²

⁵⁰ Regulatory Education and Action for Patients. "Patient Principles on Drug Shortages." November 8, 2011

⁵¹ Food and Drug Administration, "Prescription Drug User Fee Act Reauthorization (FY 2013-FY2017)

⁵² Food and Drug Administration. "Proposed PDUFA V Reauthorization Performance Goals and Procedures: FY 2013 through 2017. September 1, 2011

In order to avoid a reduction in staff levels at the FDA, Congress must reauthorize PDUFA well in advance of PDUFA IV's expiration in September 2012. Because PDUFA is considered "must-pass" piece legislation, various stakeholders may try to attach riders to the bill prior to passage, thus creating political tension in a Presidential election year. In addition, Congress will likely take up generic drug user fees and biosimilar user fees next year. It is unclear whether these will be freestanding bills or included in PDUFA.

NPAF's Role in 2012: NPAF will closely monitor the status of reauthorization and work with Congressional committees of jurisdiction to urge passage, while emphasizing the extreme importance of adequately funding for the FDA, both through federal appropriations and user fees. Regulatory Education and Action for Patients (REAP) will also continue its efforts to inform legislators if needed to support proposals from the committees of jurisdiction and the FDA that have reached mutual agreement. REAP was honored to have Dr. Theresa Mullin, FDA's Associate Director for Planning and Informatics (Center for Drug Evaluation and Research) address REAP members on PDUFA from FDA's perspective on July 21, 2011 and compose an article on PDUFA Reauthorization for the NPAF semi-annual Policy Newsletter. NPAF looks forward to building upon our discussions with Dr. Mullin in 2012 and stand ready to provide the patient perspective on PDUFA Reauthorization as needed.

Delayed PDUFA passage would prove disastrous to patients if FDA functions were halted or compromised. Without legislation, 90 days in advance of its expiry, approximately 2,000 FDA employees will be laid off and the FDA will lose the huge amount of funding it receives from drug and device developers through PDUFA.⁵³ NPAF will continue to actively work with the Alliance for a Stronger FDA and National Health Council's FDA Reform working group, as well as other patient advocacy groups, especially Friends of Cancer Research, who have been leaders in PDUFA Reauthorization, as well as those actively engaged in the development and market access to biosimilars.

MDUFMA

Background: The Medical Device User Fee Modernization Act (MDUFMA) is a set of agreements between the FDA and the medical device industry to provide funds for the FDA to review medical devices and to provide certainty and predictability to companies seeking to have their devices approved for market use. The MDUFMA program is reauthorized by Congress and relies upon a FDA and industry negotiated agreement on fees and commitments as the basis for the legislation. The current user fee program is authorized through FY2012, and will expire on September 30, 2012. In FY2003, the Food and Drug Administration (FDA) was appropriated approximately \$25 million in MDUFMA user fee funding. That figure has increased every year since and reached approximately \$57 million in FY2010.⁵⁴

Industry supports this program, which was started to ensure the agency has adequate funding to bring the latest and most innovative medical treatments to the market and patients. User fees fund pre-market review of medical devices such as pumps used to administer drugs and nutrition and imaging devices used to diagnose diseases. These fees are essential to determine if a device is safe and effective for patient use. Fees vary depending on the class of the device (low-risk Class 1 devices, moderate risk

⁵³ Redfearn, Suz "Industry Watchers Nervous as PDUFA V Deadline Looms", November 21, 2011. CenterWatch News Online

⁵⁴ Food and Drug Administration, "FY2010 MDUFMA Financial Report" May 27, 2011

Class 2 devices, which are generally approved through the 510k premarket notification process and high-risk Class 3 devices that require a Premarket Approval (PMA)). The standard 510k submission fee is approximately \$4,300 and due to the relative complexity of the submission, the standard PMA submission fee is significantly higher -- \$236,298. There are discounted submission fees for small businesses so as not to stifle innovation and dissuade start-up companies from entering the market.

While the pharmaceutical negotiations between industry stakeholders and the FDA for PDUFA concluded in 2011, MDUFMA negotiations will continue in 2012. FDA's position is that given the complexity and volume of submissions for approval, the agency needs significantly higher user fees to meet performance measures. Industry has argued that they increased fees in the last reauthorization only to find declining performance in terms of predictability of the review process and the timeline for decisions. Several Members on both sides of the aisle have been critical of the review and approval process in general with specific concerns about the length of time it takes to get 510k and PMA devices to market.

Both the House Energy and Commerce Committee and the Senate Health, Education, Labor and Pensions (HELP) Committee have held ongoing hearings related to FDA performance and the approval process in 2011, and expect to begin hearings on MDUFMA in early 2012. There has been bipartisan criticism of the timing and predictability of the Food and Drug Administration (FDA) as the agency moves devices through the approval process. On the Senate HELP Committee, Senators Hatch (R-UT) and Burr (R-NC) have been critical of the increase in review times since the last reauthorization process. Senators Hagan (D-NC), Franken (D-MN) and Bennet (D-CO) have also expressed concerns with the process. Senator Harkin (D-IA) has argued that user fees should increase and has suggested that they should also be available to fund post-market surveillance to continue evaluation of the products once they reach the patient market. When the Chairman raised the issue at a recent hearing one witness argued that using fees to fund post market activity would amount to a tax on innovation.

In the House Energy and Commerce Committee, Republicans held a series of roundtable discussions with industry groups and stakeholders that culminated in the introduction of ten individual bills that would amend existing medical device statutes. The process in the Senate has been more bipartisan, and legislation on similar issues has been introduced. While the overall authorization bill will not be introduced until the user fee agreement deal is reached, several bills have been introduced in the House and the Senate that demonstrate many of the additional issues that may be debated in the user fee authorization process. The Majority expects to attach these bills to MDUFMA reauthorization when it is considered in the House. These bills fall into a few broad categories including:

- **Streamlining and Improving the FDA Approval Process** (H.R. 3204, the Guidance and Accountability and Transparency Act, Rep. Guthrie (R-KY), H.R. 3214, The Food and Drug Administration Mission Reform Act, Rep. Rogers (R-MI), H.R. 3209, Premarket Predictability Act, Rep. Shimkus (R-IL), S. 1972, a bill to amend the Food and Drug Administration's Mission, Sen. Coats (R-IN), S. 1700, Sen. Klobuchar (D-MN).)

- **Streamlining the DeNovo Application Process** (H.R. 3203, Rep. Brian Bilbray (R-CA), S. 1943, The Novel Device Regulatory Relief Act of 2011, Senator Scott Brown (R-MA).)
- **Third Party Input and Conflict of Interest Rules** (H.R. 3205, the FDA Renewing Efficiency from Outside Review Management Act, Rep. Paulsen (R-MN), H.R. 3206, the Cultivating Scientific Experts to Foster Innovation Act (Rep. Burgess, R-TX), S. 1700, The Medical Device Regulatory Improvement Act (Sen. Klobuchar, D-MN), and S. 1865, The Patient Access to Medical Innovation Act (Sen. Franken, D-MN).)
- **Updating the Humanitarian Device Exemptions to Remove Profit Prohibitions** (H.R. 3211, The Humanitarian Device Reform Act, Rep. Bass (R-NH) and S. 1865, The Patient Access to Medical Innovation Act, Sen. Franken (D-MN).)
- **Lab Test Standards** (HR. 3207, the Modernizing Laboratory Test Standards for Patients Act, Rep. Burgess (R-TX).)
- **Clear Pre-Amendment Devices** (H.R. 3208, the Patients Come First Act, Rep. Shimkus (R-IL) to require FDA to finish their evaluation of pre-amendment devices.)
- **Harmonizing to Global Standards** (H.R. 3230, Keeping American Competitive through Harmonization Act, Rep. McMorris Rodgers (R-WA) to require the FDA where possible to enter into agreements with tier-one countries on ways to harmonize regulatory requirements.)

NPAF Role in 2012: NPAF will closely monitor the status and movement of the above legislation with counsel from Kountoupes Associates to identify those bills and sponsors that are most aligned with NPAF's mission. NPAF will utilize Patient Data Analysis Report (PDAR) data and patient experiences obtained from PAF to support the FDA's request for an increase in user fees in a responsible manner that does not impede innovation or unduly strap medical device industries with unachievable deadlines and unnecessary approval requirements. NPAF will emphasize the importance of an FDA-approval process that considers the perspective of patients in determining risk and fosters a collaborative working relationship between industry and regulators. NPAF will collaborate with REAP leadership and membership to determine the feasibility of Regulatory Education and Action for Patients' (REAP) involvement in securing MDUFMA passage.

Also, NPAF is a member of the Alliance for a Stronger FDA, the National Health Council's FDA Reform working group and the coalition for Imaging and Bioengineering Research, three coalitions of patient advocacy groups where NPAF's positions can be leveraged. NPAF will collaborate with AdvaMed and include a guest editorial on the deliberations underway with MDUFMA in its semi-annual Policy Newsletter. Also, NPAF can use its extensive database to identifying patients with compelling stories who have benefitted from the innovations in the medical device arena and to avail these patients to members of Congress for testimony at hearings likely to occur in early 2012.

- **Chronic Disease Access to Care and Survivorship (e.g. Zadroga Act; Breast and Cervical Cancer Treatment Program; HEART Act for Women; Diabetes; Survivorship Planning; Rare Disease Drug Development and Approval)**

In 2005, 133 million Americans – almost 1 out of every 2 adults – suffered from at least one chronic illness.⁵⁵ Seven out of 10 deaths among Americans each year are from chronic diseases. Heart disease, cancer and stroke account for more than 50 percent of all deaths each year.⁵⁶ Because chronic diseases are indeed chronic in a patient’s lifespan, their existence diminishes quality of life and reduces productivity in society at large. Chronic diseases also thwart potential economic output and increase the possibility of secondary complications and reoccurrences of life-threatening acute conditions. Most chronic diseases can be better managed or prevented. In 2010, PAF served patients across 278 different disease states; 70 percent were cancer-related.

The Patient Protection and Affordable Care Act (PPACA) takes a strong stance on prevention and wellness and establishes numerous incentive-based programs to identify, diagnoses, and treat chronic diseases in their early stages to aid in long-term health and wellbeing and minimize the economic impact of disease management on society. While it is impossible for NPAF to be engaged in every disease-related legislative or regulatory initiative that potentially improves patients’ access to care, NPAF has been approached about a limited number of timely initiatives that are consistent with NPAF’s mission across federal and state government affairs. While the list is not exclusive, the key initiatives are:

Zadroga Act: Cancer is not on the list of illnesses covered by the James Zadroga 9/11 Health and Compensation Act, which has set aside \$4.3 billion to treat, compensate and monitor those suffering from health problems associated with the 9/11 attacks on the World Trade Center, like asthma and other respiratory ailments.⁵⁷ However, a study released in *Lancet* in mid-2011 reveals firefighters who toiled in the wreckage of the World Trade Center in 2001 were 19 percent more likely to develop cancer than those who were not. This study provides the strongest evidence to date of a possible link between work at ground zero and cancer. NPAF will work with Kountoupes Consulting to secure meetings with the Act’s lead sponsor Representative Carolyn B. Maloney (D-NY-14), to discuss possible interpretations of the legislation which will favor the inclusion of cancer patients in the bill’s reach.

Breast and Cervical Cancer Treatment Act: In 2000, Congress passed the Act which gives states the option to offer women in the state-funded screening program access to treatment through Medicaid. To date, all 50 states and the District of Columbia have approved this Medicaid option. But criteria vary among states for eligibility and only those patients identified through the “Early Detection Program” are eligible for treatment dollars. NPAF will work with Kountoupes Consulting to host a Congressional briefing and series of awareness (e.g., briefing with women health advocates) actions to draw attention to these variations and disparities in access. NPAF

⁵⁵ Wu SY, Green A. Projection of chronic illness prevalence and cost inflation. Santa Monica, CA: RAND Health; 2000

⁵⁶ Kung HC, Hoyert DL, Xu JQ, Murphy SL. Deaths: final data for 2005. National Vital Statistics Reports 2008;56(10). Available from: http://www.cdc.gov/nchs/data/nvsr/nvsr56/nvsr56_10.pdf

⁵⁷ Available at <http://zadroga-act.com/docs/BILLS-111hr847enr.pdf>

will also initiate discussions with the Department of Health and Human Services Office on Women's Health, the Society for Women's Health Research and other patient advocacy groups to raise awareness of the issue.

Diabetes Awareness: According to the Centers for Disease Control and Prevention, 25.6 million, or 11.3 percent of all people in the United States over the age of 20, have diabetes and it is the leading cause of new cases of blindness among adults aged 20–74 years.⁵⁸ In 2007, the total direct and indirect costs to the United States healthcare economy were \$174 billion. Average medical expenditures among people with diagnosed diabetes were 2.3 times higher than what expenditures would be in the absence of diabetes.⁵⁹ It is estimated that 1/3 of the patients with adult-onset (Type II) diabetes are undiagnosed.

The top 10 chronic and/or debilitating conditions of Medicare patients served by the PAF in 2010, 56.6 percent were diabetes related.⁶⁰ NPAF will work in conjunction with patient advocate partners to monitor state legislation and identify opportunities to provide advocacy-related services to patients suffering from chronic diseases, especially diabetes. NPAF will continue to actively participate in the Partnership to Fight Chronic Disease (coalition) and work with state government affairs officials to collaborate on initiatives of importance to diabetic patients. NPAF will also encourage the Regulatory Education and Action for Patients (REAP) leadership to recruit more members affiliated with the diabetes community.

HEART Act for Women: Re-introduced late 2011 by Representative Lois Capps (D-CA-23) the Heart disease, Education, Analysis and Research, and Treatment (HEART) for Women Act (H.R. 3256) would expand funding eligibility from the current 20 states to all 50 for the Center for Disease Control's (CDC) WISEWOMAN screening program to identify women at risk of heart disease, the leading cause of death among women in the United States. The program targets low income and uninsured women. WomenHeart (the National Coalition for Women with Heart Disease) seeks NPAF support for this legislation which also aims to educate women and providers on heart disease prevention and diagnosis, as well as make Food and Drug Administration (FDA) requirements concerning reporting of race and gender data more strict. Additionally, NPAF will provide public comments on the FDA guidance document released at the end of 2011 aimed at increasing gender representation in clinical trials, most notably cardiac medical devices.⁶¹

NPAF will also actively raise awareness of the "Million Hearts" initiative, a public/private partnership to eliminate 1 million heart attacks and strokes in the next 5 years. Launched in September, 2011, the campaign— involving CDC, CMS, NIH, SAMHSA, the "Y", Walgreens and United Healthcare and others – will involve more community education, increased Health IT, access to free or more affordable tests, changes in nutritional information, and utilization of evidence-based measures. NPAF included an announcement of the program in its semi-annual Policy Newsletter (2011) and will participate in a public event in January 2012 with WomenHeart

⁵⁸ Centers for Disease Control and Prevention. National diabetes fact sheet, 2007. Atlanta, GA: United States Department of Health and Human Services; 2008. Available from: <http://www.cdc.gov/Diabetes/pubs/factsheet07.htm>

⁵⁹ American Diabetes Association, 2003. Economic Costs of Diabetes in the United States in 2002. *Diabetic Care* 26(3):917-932)

⁶⁰ Patient Advocate Foundation: Patient data analysis report, 2010, p.32

⁶¹ Available at : <http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm283453.htm>

and officials from the sponsoring organization, the Department of Health and Human Services. NPAF's volunteer network will also be engaged to disseminate information about the campaign on state and local levels.

Cancer Survivorship Meets Chronic Disease Management: With more than 11.1 million cancer survivors in the United States today, researchers and doctors are grappling with the challenge of helping patients maintain or regain a sense of well-being and looking at chronic care models for possible clues. The National Coalition for Cancer Survivorship (NCCS) seeks support for a bill to establish a Medicare service for comprehensive cancer care planning, establish discretionary programs for professional training and continuing professional education related to cancer care planning and symptom management, and encourage investment in research on topics related to cancer care coordination, symptom management, palliative care, and comprehensive survivorship care. NPAF will actively engage with NCCS for passage of this legislation and look for opportunities to translate key components of the legislation into a chronic disease model for broader applicability. NPAF will coordinate its discussions in cancer survivorship with the American Cancer Society, the George Washington Cancer Institute, and the LIVESTRONG Foundation.

Rare Disease Drug Development and Approval: NPAF has long advocated for speedier approval of therapies designed to treat rare diseases (as defined by the Orphan Drug Act as diseases that affect less than 200,000 individuals a year) and has partnered with other patient advocacy organizations to work with industry and Food and Drug Administration (FDA) to achieve this aim. An October 2010 Institute of Medicine (IOM) Report entitled "*Rare Diseases and Orphan Products – Accelerating Research and Development*" stated that rare diseases collectively affect millions of Americans of all ages and that because each rare disease affects a relatively small population, it can be challenging to develop drugs and medical devices to prevent, diagnose, and treat these conditions.⁶²

The Prescription Drug User Fee Act (PDUFA) Reauthorization proposal includes an enhanced focus on rare disease drug approval, by increasing staff in the Center for Drug Evaluation and Research (CDER) and in the Center for Biologics Evaluation and Research (CBER) within FDA. Recently, The National Organization for Rare Disorders (NORD) released a landmark report entitled "*Quantum of Effectiveness Evidence in FDA's Approval of Orphan Drugs - Cataloguing FDA's Flexibility in Regulating Therapies for Persons with Rare Disorders*" which documented that conducting the clinical studies required to develop treatments for rare diseases poses special challenges to medical researchers, but also showed flexibility in the FDA review of potential treatments for patients with rare diseases.⁶³

NPAF will continue to work with its non-profit colleagues and partners, particularly NORD, who are engaged in this issue and offer our support in their mission to provide therapeutic options to patients diagnosed with rare diseases. Specifically, NPAF supports NORD's request for the FDA to

⁶² Institute of Medicine "Rare Diseases and Orphan Products – Accelerating Research and Development" October, 2010

⁶³ National Organization for Rare Disorders "Quantum of Effectiveness Evidence in FDA's Approval of Orphan Drugs - Cataloguing FDA's Flexibility in Regulating Therapies for Persons with Rare Disorders", October 11, 2011.

develop formal policy for approval of rare disease drugs; currently one does not exist. Former Senator and current Governor Sam Brownback (R-KS) included an amendment in the FY2010 Senate Appropriations bill calling for the FDA to release draft guidance on orphan product review. The report was due December 27, 2011 and upon its release, NPAF will collaborate with NORD to ensure the guidance adequately addresses concerns from the perspective of the rare disease patient community. NPAF, through REAP, will also support PDUFA's rare diseases provisions and actively advocate for greater flexibility for FDA when approving rare disease drugs by evaluating unique endpoints and biomarkers for rare diseases that have a clear unmet medical need.

2012 NPAF State Legislative and Regulatory Priorities

Tier 1

- **State Health Exchanges**

Background: Section 1311(b) and section 1321(b) of the Patient Protection and Affordable Care Act (PPACA) create state health exchanges (“exchanges”), which are virtual marketplaces where individuals and small businesses can compare health insurance plans as well as purchase health insurance on the state level. The purpose of exchanges is to make health insurance more affordable and easier to purchase for small business and individuals. The legislation mandates that states will create their own health exchanges by 2014, or have them created by the federal government. States can choose to operate their own exchange or participate in a multi-state health exchange. Exchanges may also be government entities, quasi-government operations or private entities.⁶⁴ Thus far, 23 states either have health exchanges or are taking steps forward to create them. As of year-end 2011, the following state actions have occurred:

- 2 states (Massachusetts and Utah) passed legislation to create health insurance exchanges prior to the passage of PPACA.
- 13 states (California, Colorado, Connecticut, Hawaii, Illinois, Maryland, Nevada, North Dakota, Oregon, Virginia, Vermont, Washington and West Virginia) have passed legislation as a result of the PPACA to create health exchanges.
- 5 states and the District of Columbia (North Carolina, New Jersey, New York, Pennsylvania and Michigan) have legislation introduced that is pending.
- 14 states (Alabama, Alaska, Arizona, Georgia, Iowa, Maine, Minnesota, Missouri, Mississippi, Montana, New Hampshire, Oklahoma, South Carolina and Texas) have witnessed legislation that has failed, been withdrawn or expired.
- 1 state (New Mexico) witnessed legislation vetoed by the governor.
- 3 states (Indiana, Minnesota and Rhode Island) have started to create health exchanges by executive order.
- 12 states (Delaware, Florida, Idaho, Kansas, Kentucky, Louisiana, Nebraska, Ohio, South Dakota, Tennessee, Wisconsin and Wyoming) have seen no legislative or executive action to create a health exchange.

⁶⁴ Patient Protection and Affordable Care Act of 2010; Part II; Section 1311

NPAF Role in 2012: NPAF staff, ELITE volunteers and State Policy Liaisons will be actively engaged in proactive advocacy efforts with state leaders (such as governors, state policy makers, state insurance commissioners and state health exchange board members) in assuring state exchange development activities reflect the best interests of patients. NPAF staff and volunteers will also work for the creation and direction of effective coalition efforts on behalf of patients with advocacy partners such as: Coalition for Affordable Health Care, Leukemia & Lymphoma Society, International Myeloma Foundation, Lung Cancer Alliance, WomenHeart: the National Coalition for Women with Heart Disease, International Cancer Advocacy Network, American Heart Association, American Diabetes Association, Partnership to Fight Chronic Disease, Patient Equal Access Coalition, Susan G. Komen for the Cure, Cancer Leadership Council and others that have a desire to join NPAF's efforts; e.g., provider organizations. The key activities to be supported include the appointment of patient advocates to health exchange boards, the development of essential benefits packages, and the internal development of exchange rules and procedures. NPAF will take the lead where necessary and will work in partnerships whenever possible.

The focus of the NPAF Fall 2011 Policy Consortium was on states' implementation of exchanges with representative and officials from Connecticut, Nevada, Rhode Island, and Washington. The issues developed at the conference will continue to shape the Policy Consortium agenda in 2012. Additionally, NPAF will continue educating State Policy Liaisons (SPLs) on monthly conference calls about exchanges and request that they provide information from the local and state level.

- **Essential Health Benefits (EHB)**

Background: As noted in the Federal Regulatory Section (see above), PPACA creates state health exchanges ("exchanges"), which are virtual marketplaces where individuals and small businesses can compare health insurance plans as well as purchase health insurance on the state level. HHS is required to specify the "essential health benefits" (EHBs) included in the "essential health benefits package" that QHPs, operating within a state exchange, are to cover effective 1/1/14.

On December 16, 2011, HHS issued a guidance document intended to give more flexibility and freedom to states to implement EHBs.⁶⁵ The guidance bulletin suggests an approach where states would be able to choose a benchmark plan that it deems reflective of a 'typical employer plan' in terms of services offered. The benchmark can consist of one of four options:

- One of the three largest small group plans in the state by enrollment
- One of the three largest state employee health plans by enrollment
- One of the three largest federal employee health plan options by enrollment
- The largest HMO plan offered in the state's commercial market by enrollment

If states choose not to select a benchmark, HHS proposes that the default benchmark be one of the largest small group plans in the state by enrollment. Consequently, those benefits offered by the selected benchmark plan would define the EHBs for that state. States would be able to modify the EHBs as long as the value of coverage is not reduced.

⁶⁵ Center for Consumer Information and Insurance Oversight. "Essential Health Benefits Bulletin." December 16, 2011. Accessed at: http://cciio.cms.gov/resources/files/Files2/12162011/essential_health_benefits_bulletin.pdf

NPAF's Role in 2012: As much of the decision making process will be at the state level, NPAF State Government Affairs team will work directly with state policy makers and will direct ELITE volunteers and State Policy Liaisons to interact with state health exchange governing board members, state governors and state policy makers to ensure that EHB packages successfully meet patient needs. These activities will be important to pursue in nearly every state but most particularly in the key states (referenced above under State Health Exchanges) where legislation (or executive orders) has been recently passed to establish a health exchange or in states where legislation or executive orders are imminent. NPAF will work to establish patient coalitions in key states (see State Health Exchanges above) in conjunction with ongoing advocacy partners such as the Coalition for Affordable Health Care, the National Health Council, the Leukemia and Lymphoma Foundation, Susan G. Komen for the Cure, the Cancer Leadership Council and others advocating for inclusive EHB packages.

Tier 2

- **Oral Chemotherapy Parity Legislation**

Background: Parity in the reimbursement for orally administered chemotherapy vs. IV administered chemotherapy remains an important issue for patients. There is substantial pharmacoeconomic data to show that oral chemotherapy regimens have both a cost effective and clinical efficacy advantage over same-compound and established I.V. regimens.^{66, 67, 68, 69, 70, 71} In addition, the issue continues to be important to patients, many of whom contact PAF on a regular basis in need of assistance to cover the cost of oral chemotherapy medications. Legislative action on the issue to date is as follows:

- 10 states (Colorado, Connecticut, the District of Columbia, Hawaii, Indiana, Iowa, Kansas, Minnesota, Oregon and Vermont) passed legislation requiring coverage of oral chemotherapy agents when coverage existed for intravenous (IV) or injectable chemotherapy agents prior to 2011.
- 5 states (Illinois, New Mexico, New York, Texas and Washington) passed legislation in 2011. In January of 2012, legislation was passed and signed by the governor in New Jersey.
- 11 states (California, Georgia, Massachusetts, Michigan, Missouri, Ohio, Oklahoma, Pennsylvania, South Carolina, Tennessee and Wisconsin) have legislation filed although not passed by December, 2011.

⁶⁶ Galewitz, P. Kaiser Health News. Henry J. Kaiser Family Foundation. November 5, 2010

⁶⁷ Best JH, Garrison LP. Economic evaluation of capecitabine as adjuvant or metastatic therapy in colorectal cancer. Expert Rev Pharmacoeconomic Outcomes Res. 2010 Apr; 10(2):103-14

⁶⁸ Bradbury, PA, Tu, D, et. al. Economic Analysis: Randomized Placebo controlled Clinical Trial of Erlotinib in Advanced Non-Small Cell Lung cancer. JNCI. Mar 2010 (102-5): 298-306

⁶⁹ Sargent DJ, for the Adjuvant Colon Cancer Endpoints (ACCENT) Group. Time dependent patterns of failure and treatment benefit from adjuvant therapy for resectable colon cancer. Lessons from the 20,800 patient ACCENT dataset. Proc Am Soc Clin Oncol. 2007; 25:165s. Abstract 4008

⁷⁰ O'Connell MJ, for the Adjuvant Colon Cancer Endpoints (ACCENT) Group. Survival following recurrence in patients with adjuvant colon cancer: findings from the 20,800 patient ACCENT dataset. Proc Am Soc Clin Oncol. 2007; 25:165s. Abstract 4009

⁷¹ Twelves C, Wong A, Nowacki MP, et al. Capecitabine as adjuvant treatment for stage III colon cancer. N Engl J Med. 2005; 352:2696-2704

NPAF Role in 2011: NPAF staff will work directly with those 11 states where legislation was filed in 2011 plus Virginia and Maryland. NPAF staff and volunteers will work in many states in collaboration with the American Cancer Society, the Leukemia Lymphoma Society, and other patient-focused advocacy groups for passage of oral chemotherapy parity legislation in additional states. NPAF patient de-identified data will be provided to inform legislative deliberations. It is essential that NPAF work in conjunction with advocacy partners and motivate volunteer engagement in the target states in 2012 and to take the lead in the key states referenced above when needed. The success of legislation in 16 states on this issue to date points to the effectiveness of past NPAF efforts to work with advocacy partners as well as the need to continue to take the lead in speaking out for patients on critical issues that affect their care and wellness. This will present an outstanding opportunity for NPAF ELITE President's Council volunteers to engage in direct advocacy efforts with their state policy makers and to work with state coalitions for the passage of oral chemotherapy parity legislation in target.

Tier 3

- **Medicaid Stability and State Formularies**

Background: The Patient Protection and Affordable Care Act (PPACA) mandated fairly massive reform to the nation's Medicaid program. As a result of the legislation, individuals (under age 65) and working families that earn up to 133 percent of the federal poverty level (\$29,400 for a family of 4, or \$14,400 for an individual) will be eligible for Medicaid starting in 2014. The Congressional Budget Office (CBO) estimates that by 2019, 16 million more adults and children will enroll in Medicaid and gain access to affordable coverage as a result. To finance the coverage for the newly eligible beneficiaries, states will receive 100 percent federal funding for 2014 through 2016, 95 percent federal financing in 2017, 94 percent federal financing in 2018, 93 percent federal financing in 2019, and 90 percent federal financing for 2020 and subsequent years. States that have already expanded eligibility to adults with incomes up to 100 percent of Federal Poverty Level (FPL) will receive a phased-in increase in the federal medical assistance percentage (FMAP) for non-pregnant childless adults so that by 2019 they receive the same federal financing as other states (93 percent in 2019 and 90 percent in 2020 and later). States had the option to expand Medicaid eligibility to childless adults beginning on April 1, 2010, but will receive their regular FMAP until 2014. In addition, increase Medicaid payments in fee-for-service and managed care for primary care services provided by primary care doctors (family medicine, general internal medicine or pediatric medicine) to 100 percent of the Medicare payment rates for 2013 and 2014. States will receive 100 percent federal financing for the increased payment rates. (Effective January 1, 2014).⁷²

Because Medicaid is a federal/state partnership, the majority of states, witnessing shrinking revenues and budgets, will be actively engaging in their own efforts to realize budget savings, such as Florida's legislation to move all Medicaid "fee-for-service" care into Medicaid Managed Care plans in 2011.⁷³ As more states look for ways to manage Medicaid budgets -- which could involve cutting benefits and raising eligibility requirements -- the challenges patients face in monitoring Medicaid coverage will be elevated. It will be important for NPAF to monitor and track budget cutting efforts in the states as well

⁷² Center on Budget and Policy Priorities: Medicaid Expansion in Health Reform Not Likely to "Crowd Out" Private Insurance; Matt Broaddus and January Angeles, June 22, 2010

⁷³ Florida House Bill 7109, 2011

as the various approaches to re-organize Medicaid at the state level which could result in cutting benefits or eligibility for patients. An important aspect of this effort will include the availability of critical medications on state Medicaid formularies and close monitoring of specialty tier pricing which impedes patients' access to the drugs they need. Although states are currently required by law to cover off-label use of anticancer drugs if they are listed as active drugs in one of the accepted pharmaceutical compendia or the peer-reviewed literature, they may decide to utilize only one of the compendia, thereby limiting the medications that are covered. Based on the most recent information available, the four Centers for Medicare and Medicaid Services (CMS)-recognized compendia include: The American Hospital Formulary Service Drug Information® (AHFS-DI®); Thomson Reuters DrugDex®; the National Comprehensive Cancer Network (NCCN) Drugs & Biologics Compendium™; and Gold Standard/Elsevier's Clinical Pharmacology.⁷⁴ The utilization of these compendia is crucial to PAF case managers who rely upon them to assist patients with denied claims.

NPAF Role in 2011: NPAF will track and monitor challenges that may arise at the state level in 2012 and will work with patient advocate partners wherever possible to intercede on behalf of patients when onerous budget cuts are a threat. NPAF will work to convene meetings with state Medicaid directors and other key staff in states based on 2012 bills filed that would pose a threat to patients in regard to the limitation or elimination of benefits or the downsizing of Medicaid roles based on eligibility (bill filings in most states are not finalized until the two weeks preceding the start of the legislative session). The NPAF State Government Affairs Team will work with advocacy partners to pursue pro-active legislation for the purpose of mandating the use of multiple drug compendia in the development of state Medicaid drug formularies in key states. ELITE volunteers will be asked to interact with state policy makers (legislators and state Medicaid Directors) as necessary and State Policy Liaisons (SPLs) will track possible policy threats in their respective states. NPAF will also monitor specialty-tier pricing and legislative efforts to minimize any negative impact on patient access.

⁷⁴ United States Department of Health and Human Services: Center for Medicare and Medicaid Services, 2010

SUPPLEMENTAL

White Papers (to be developed throughout 2012)

- **Standardize Application for Enrollment in Public Assistance**

Background: The administrative process for patients to enroll in public medical and social programs creates undue burden, duplicity, confusion and ultimately a delay in accessing health care services in a timely and efficient manner. Patients often are forced to complete numerous applications in various formats that are difficult to understand, time-consuming, and receive little or no immediate understanding of their eligibility. This hardship has been recognized by Patient Advocate Foundation (PAF) case managers increasingly for the past several years and represents a very real, frustrating hurdle to providing the advice and support that patients need to move forward with the care they need.

The value to standardize the administrative aspects of eligibility and coverage and the potential benefit to patients are prominent themes in the Patient Protection and Affordable Care Act (PPACA). For example, Section 1104 of PPACA amends HIPAA to provide that notification standards be *uniform* and that administrative *simplification reduce clerical burden on patients, health care providers and health plans*.⁷⁵ Furthermore, PPACA requires that operating rules, to the extent feasible, *enable determination of an individual's eligibility and financial responsibility prior to or at the point of service*. PPACA also requires that the Secretary develop a *standardized format for enrollment in state health exchanges* (implemented 1/1/14) for the presentation of information (web-based) relating to coverage option.⁷⁶

While primarily useful for Medicaid, enrollees in Medicare could also benefit from a standardized application process that is uniform and provides real-time feedback on the need for supplement plans (e.g., MediGap). Application processes should be in compliance with federal standards for HIT; available online; respectful of differing language and educational levels; and with real-time feedback.

NPAF Role in 2012: NPAF's position is that enrollment in *existing* and future public assistance programs should be standardized in order that patients' applications, assessment and notification are efficient and timely. Undue delays in access to care due to paperwork requirements are untenable. NPAF will issue a White Paper addressing the advantages of standardizing the application process and will meet with Medicaid officials to emphasize the opportunities available under the PPACA that may be applicable to existing enrollment processes. NPAF will provide expertise from PAF's management of patients burdened by duplicitous and timely applications.

- **Medical Bankruptcy/Debt**

Combating medical debt is an issue central to the mission of NPAF and PAF. PAF, through its experiences working with nearly 83,000 people in 2010, repeatedly finds medical costs and financial burden as a central barrier to quality care. Medical debt crisis accounted for 63 percent of issues reported by PAF

⁷⁵ Patient Protection and Affordable Care Act of 2010; Part I; Part B: Section 11104

⁷⁶ Ibid. Section 1103

patients. Furthermore, 77 percent of the patients reporting a medical debt crisis had some type of private or public health insurance. These statistics are not surprising considering studies repeatedly indicate that medical debt is the leading cause of bankruptcy in the United States.⁷⁷

NPAF's Role in 2012: There is no current "Bankruptcy Protection" legislation under consideration in Congress, and there is not likely to be legislation introduced in 2012. However, due the prevalence of this issue as reported by PAF case managers on the front lines of patient assistance and the ongoing threats to the economic stability of our country, NPAF will take the following steps to address the subject in 2012:

- First, NPAF will issue a White Paper on the issue to further inform the policy makers and the general public of the growing incidence of medical debt and the burden it places on patients at a time of critical need. Medical debt is not only responsible for over 62 percent of bankruptcies, but the number of bankruptcies that can be attributed to medical debt is increasing.⁷⁸
- Second, NPAF will work with Kountoupes Consultants to identify key legislators to introduce legislation in 2013 who are sensitive and receptive to taking action to halt the pervasive incidence of medical debt that results from the treatment of chronic, debilitating and life-threatening diseases.
- Third, NPAF volunteers will raise the importance of this issue at town hall meetings across the country.
- Fourth, NPAF will discuss with Kaiser and Harvard about expanded data acquisition in order to further verify NPAFs assertions of the issue's severity.
- Finally, NPAF will find ways to encourage hospitals to increase their efforts to educate patients of available charity care.

Regulatory Education and Action for Patients (REAP) members will be encourage to contribute to this effort by contacting legislators and mobilizing volunteers, as the issue of medical debt is considerable and crosscutting across patient groups.

- **The Individual Mandate**

Under PPACA, starting in 2014, individuals must be enrolled in a health insurance plan that meets basic minimum standards or face the possibility of a penalty (i.e., "fee"). This requirement, commonly referred to as the "individual mandate", has become one of the most politically-charged aspects of PPACA with supporters and opponents across political, legal, economic and geographic lines. In November 2011, the

⁷⁷ David U. Himmelstein, MD, Deborah Thorne, PhD, Elizabeth Warren, JD, Steffie Woolhandler, MD, MPH. "Medical Bankruptcy in the United States, 2007: Results of a National Study." American Journal of Medicine, 2009.

http://www.pnhp.org/new_bankruptcy_study/Bankruptcy-2009.pdf

⁷⁸ David U. Himmelstein, MD, Deborah Thorne, PhD, Elizabeth Warren, JD, Steffie Woolhandler, MD, MPH. "Medical Bankruptcy in the United States, 2007: Results of a National Study." American Journal of Medicine, 2009.

http://www.pnhp.org/new_bankruptcy_study/Bankruptcy-2009.pdf

United States Supreme Court announced it would, in 2012, take up a lawsuit brought by 26 states and the National Federation of Independent Businesses who accuse Congress of overstepping its constitutional boundaries by including this mandate in the Patient Protection and Affordable Care Act (PPACA) and requiring states to considerably expand Medicaid programs as part of the healthcare overhaul. Collectively, the Court will rule on four key questions: whether the “individual mandate” is constitutional, whether the Medicaid expansions are constitutional, whether challenges to the law are premature under a provision known as the Anti-Injunction Act, and whether overturning the “individual mandate” would render the entire legislation unconstitutional due to the lack of adequate provisions for severability.

The Court has scheduled arguments on the Anti-Injunction Act for March 26 and arguments on the “individual mandate” for March 27. On the final day of oral arguments, March 28, they will hear arguments pertaining to the Medicaid expansion and whether the rest of the law can stand without the “individual mandate”.⁷⁹ Of these issues, of particular relevance to NPAF’s policy work and the patients served by PAF is the constitutionality of the “individual mandate” and its enforceability, both for its economic impact on patients and its potential to aid the nearly 81 million Americans who were either uninsured or underinsured in 2010.⁸⁰

NPAF’s Role in 2012: NPAF will review the current writings and literature – both scholarly and of public opinion – on key issues of relevance to the constitutionality of PPACA’s requirement for individuals to obtain health insurance starting in 2014. Internal deliberations will include discussions with legal counsel and NPAF Executive Board and Scientific Advisory Committee members. It is NPAF’s preliminary position that the “individual mandate” is viable and should be upheld. To articulate its position and inform policy makers and the general public, NPAF will release a White Paper on the issue in early 2012 giving careful note to the arguments that are most favorably aligned with the interests of patients.

In consultation with legal counsel, NPAF will seek to explain in the White Paper why the “individual mandate” is sustainable and how its existence bolsters the patient protection provisions of PPACA and provides the underpinning of many of the benefits that patients served by PAF presently need and will continue to need in the future when additional provisions of PPACA become effective. The White Paper will be distributed to policy officials, coalition members, and Congressional members and serve as the official position of the organization in all media requests and public discussions. NPAF will also inform others of its position through blog posts, guest editorials, radio and television appearances, and guidance to volunteers. NPAF will utilize the services of Schmidt Public Affairs for the placement and dissemination of the White Paper publicly and encourage other patient-advocacy organizations, such as those involved in REAP, to endorse the White Paper and its premise.

⁷⁹ *Supreme Court to Hear Healthcare Lawsuit in March*, The Washington Post, December 19, 2011; available at: <http://www.washingtontimes.com/news/2011/dec/19/supreme-court-hear-health-care-suit-march/>

⁸⁰ *Affordable Care Act Reforms Could Reduce the Number of Underinsured United States Adults by 70 Percent*, *The Commonwealth Fund*, September 8, 2011; available at: <http://www.commonwealthfund.org/Publications/In-the-Literature/2011/Sep/Reduce-Uninsured.aspx>

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