

Cost-containment and Deficit Reduction Policy Principles

The United States government is facing pressure to control federal spending and lower the national debt, with health care costs as a focal point of debate to address this pressure. The path that policymakers take will have a significant impact on continued biomedical progress, the role of U.S. companies as global leaders in life science innovation, and the quality of patient care and physician choice.

Personalized medicine presents challenges to health care policymakers because conventional policy tends to change slowly and rely upon population averages, whereas personalized medicine leads to increasingly rapid changes in medicine by enabling new insight into differences in individual patients and patient subgroups. Recent scientific progress has brought major treatment advances that will improve patient outcomes with the greatest impact among those patients with serious and life-threatening conditions and with unmet medical needs. While progress in personalized medicine is more complex and challenging than ever before, this continued progress will be part of the solution to health care cost challenges as it brings efficiencies into the care-delivery system.

To ensure that cost-containment policies do not undermine personalized medicine and protect innovation, the physician-patient relationship, and patient values and choice, the Personalized Medicine Coalition (PMC) supports the following principles that are highlighted here and described more fully below.

In brief, policies should:

- 1. Support increased emphasis on patient-centeredness, patient engagement, and the patient-physician relationship;**
- 2. Incentivize prediction, preemption, and prevention of cancer and other chronic, serious, and life-threatening diseases;**
- 3. Support approaches that accommodate the multiple dimensions of and perspectives on value in personalized medicine instead of centralized standards based on average comparative clinical or cost effectiveness;**
- 4. Expand and make effective use of informatics and “learning health systems”;**
- 5. Sustain funding for basic research at the National Institutes of Health (NIH);**
- 6. Match public sector research investments with incentives for innovation in the private sector;**
- 7. Reform assessment tools (in particular, comparative effectiveness research and health technology assessment) to better align with emerging science and patient-centered health care;**
- 8. Protect innovation and value in new payment models; and**
- 9. Maintain resources for continued public-private sector collaboration.**

In greater detail, policies should:

1) Support increased emphasis on patient-centeredness, patient engagement, and the patient-physician relationship;

As the health care delivery system continues to evolve, it is vital that measures and standards be implemented that appropriately define and protect value with the understanding that a good outcome must be defined in terms of what is meaningful and valuable to the individual patient. Further, policy should recognize and support the central role of physicians and other caregivers in advancing personalized, evidence-based, and high-quality care. Therefore, PMC believes that a deliberate and comprehensive shift away from health care based on population averages and towards patient-centered care is central to improving health care outcomes and addressing a patient's perception of value.

2) Incentivize prediction, preemption, and prevention of cancer and other chronic, serious, and life-threatening diseases;

Individuals with chronic diseases and other serious, life-threatening diseases account for roughly 84 percent of U.S. health care spending¹. PMC believes that policies should promote improved care management throughout the continuum of care. A health system that focuses on care that is predictive, preemptive, and preventive has the potential to revolutionize health care by allowing clinicians to individualize therapy for patients through the early diagnosis of disease and risk assessment in order to optimize clinical outcomes and to better manage patients before disease symptoms appear. This course requires a system-wide approach to care management, and continued development and use of diagnostic tests for biomarkers to enable patients and their health care team to predict, preempt, or prevent chronic, serious, and life-threatening disease.

3) Support approaches that accommodate the multiple dimensions of and perspectives on value in personalized medicine instead of centralized standards based on average comparative clinical or cost effectiveness;

Coverage or reimbursement policy based on "one-size-fits-all" definitions of comparative clinical or cost effectiveness fail to recognize differences in patient needs and preferences and is misaligned with the progress in personalized medicine. This type of policy has resulted in significant barriers to access to the treatment options that are best for the individual and chills medical progress.

Policies that rely on clinical "superiority" standards fail to recognize the multiple dimensions of value in personalized medicine as those standards are often based on population averages that do not reflect individual response, preferences, or needs. PMC believes that continued promotion and focus on personalized medicine will help improve health outcomes and will also reduce the overall cost burden.

Personalized medicines will help tailor therapies to what is most appropriate for an individual patient, with the potential to increase the efficacy and safety of medications.

¹ Anderson, Gerard. "Chronic Care: Making the Case for Ongoing Care." RWJF.org. Robert Wood Johnson Foundation. March 2010. P.16.

4) Expand and make effective use of informatics and “learning health systems”;

Clinical innovation has continually outpaced standards of care, resulting in patients not taking advantage of new treatments and technologies that may dramatically improve their health outcomes while reducing long-term health care costs. Policies that set unrealistic evidence standards that require long-term studies can significantly impede the adoption of innovative medical advances and delay patient access to new, lifesaving treatments. PMC believes that there must be a continual learning system to aggregate, analyze, and apply evidence-based knowledge to patient care. PMC believes that health informatics and electronic health record (EHR) systems must be used to promote continual learning systems that will help improve patient care, reduce costs, and accelerate the process of drug development.

5) Sustain funding for basic research at the National Institutes of Health (NIH);

Investments in scientific research have led to countless discoveries and medical breakthroughs and deficit reduction proposals must not be achieved by hindering future discovery of new tests and treatments for unmet medical needs. Current policies already leave the NIH vulnerable to dramatic budget cuts that would not only stall medical innovation, but could trigger a loss of jobs and critical economic activity. PMC believes that funding for basic research at the NIH must be sustained.

6) Match public sector research investments with incentives for innovation in the private sector;

Continued support for basic research will prove futile if not paired with coverage and payment policy that provides incentives for continued innovation. Blunt reimbursement and payment cuts will work to discourage continued development of advances in personalized medicine and other new technologies. PMC believes that there must be an appropriate pathway to ensure timely coverage and adequate payment for novel diagnostic tests, targeted therapies, and treatment combinations that are at the forefront of advances in personalized medicine. Policymakers must support coverage and reimbursement policy that promotes the development of new technologies that can harness the health and economic benefits of personalized medicine.

7) Reform assessment tools (in particular, comparative effectiveness research and health technology assessment) to better align with emerging science and patient-centered health care;

Comparative effectiveness research (CER) and health technology assessment (HTA) must align with patient response, needs, and values, as well as with emerging science and changing clinical practices. If conducted properly, CER and HTA can help optimize decision-making by patients and clinicians by ultimately providing appropriate clinical and patient-centered information that better reflects individual preferences and needs. To achieve improved health outcomes and efficient use of resources, PMC believes that CER and HTA must shift from a retrospective, static paradigm to a prospective, dynamic paradigm. Such a shift will allow for the rapid pace of change in personalized medicine to emerge and improve patient care. It will also improve understanding of how the optimal clinical role and value of new tests and treatments evolve after initial introduction. By guiding patient-centered research focused on the biological differences among individual patient populations and by disseminating meaningful clinical information, CER and HTA can help patients and clinicians make informed health care decisions that will improve health outcomes and address long-term health care costs.

8) Protect innovation and value in new payment models;

New payment models, such as accountable care organizations (ACOs) and bundled payments, which promote coordination and integrated care, hold potential to shift incentives to high-quality, high-value care for patients. However, if improperly designed, such models will set payment based on current standards of care and discourage advances in medical technology and medical practice. New payment models must be careful not to unintentionally undermine the development of and patient access to innovative treatments. Quality measures that properly recognize the value of care from a patient perspective as well as mechanisms to incentivize adoption of innovations and advances need to be developed and incorporated in these new payment models. PMC believes that value in health care is measured by the outcomes achieved, not the volume of services delivered.

9) Maintain resources for continued public-private sector collaboration.

There are currently thousands of new drugs and diagnostics working their way through the scientific pipeline aimed at addressing diseases and chronic conditions like cancer. In order for these innovative treatments to enter clinical practice and improve patient care, there must be a transparent and efficient regulatory pathway toward approval of new drugs and technologies. PMC believes that agreements negotiated between the U.S. Food and Drug Administration and industry must be maintained.

The Personalized Medicine Coalition

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system. For more information about PMC, visit www.PersonalizedMedicineCoalition.org.