

Personalized Medicine and Healthcare Reform: Policy that Protects Innovation While Improving Value and Quality

Policies intended to bring about healthcare reform could have significant implications for the adoption of personalized medicine. The Personalized Medicine Coalition (PMC) has developed a set of policy recommendations to ensure that the potential for personalized medicine to improve healthcare quality and affordability is appropriately reflected in policies that may emerge as a result of the healthcare reform debate.

The PMC represents a broad spectrum of academic, industrial, patient, provider, and payer communities, and it supports healthcare reforms that improve quality and affordability while fostering continued medical progress. PMC believes meaningful healthcare reform must encourage continued advancements in personalized medicine and that, to help achieve the goals of high-quality, affordable care for all Americans, health reform should:

- Support and incentivize medical research in the public and private sectors;
- Establish a national initiative to advance collaboration in support of personalized healthcare across federal health agencies;
- Encourage adoption of a national health information infrastructure;
- Research and identify care delivery and management models that encourage effective, evidence-based disease prevention and care coordination;
- Develop and apply research findings in ways that empower providers and patients by reflecting differences in individual needs; and
- Ensure that relevant provider performance measures and incentives support the adoption of new personalized medicine interventions.

Reform of our national healthcare system has gained fresh prominence as a top-line issue for Americans and will be high on the agenda of the new Congress and presidential administration. Covering the uninsured, improving healthcare quality, and controlling rising healthcare costs all will be central themes in this debate.

The renewed discussion of healthcare reform coincides with a time when significant and rapid advancements in genomics and other relevant areas of science and technology are accelerating the emergence of personalized medicine. Personalized medicine uses new methods of molecular analysis to better manage a patient's disease or to assess that patient's predisposition toward a disease. The field includes genetic tests and other types of diagnostics, as well as targeted therapies. It helps providers and patients achieve optimal health outcomes by preventing or intervening early in the onset of disease and by identifying the approaches to treatment and care that are best for each individual.

Medical advances arising through the science of personalized medicine, particularly when combined with health information technology, hold great promise for improving the quality and value of healthcare. In turn, healthcare reform holds significant implications for personalized medicine. In particular, policy measures designed to control costs and expand access—but that fail to encourage continued development and adoption of personalized medicine—could substantially delay or diminish opportunities for meaningful, measurable improvements in healthcare value and quality.

Thinking Differently about Healthcare

There is no question that the U.S. healthcare system needs reform. Almost 46 million Americans are uninsured.¹ Nearly \$2 trillion is spent annually on healthcare in the United States, and national health spending as a percentage of gross domestic product is projected to hit 20 percent by 2016.²

When considering the growing burden healthcare costs place on our national economy, our healthcare and research infrastructure, and on our individual finances, policymakers may be tempted to focus on seemingly expedient fixes, such as aggressive government price controls and access restrictions, applying rigid evidence standards to achieve short-term cost-containment goals, or by cutting back on covered benefits.

While such solutions may provide some short-term relief in healthcare spending (likely accompanied by a sacrifice in quality and health outcomes), they would ultimately raise system-wide costs, stifle valuable opportunities for improvements in care and outcomes, and undermine the continued development and adoption of personalized medicine as the model for effective, state-of-the-art healthcare delivery in the United States. For example:

- Proposals that seek to contain costs by promoting the least expensive treatment on average, rather than the best care for the individual, will discourage the development and adoption of gene-based diagnostic tests and targeted therapies, which can have a higher up-front cost but will offer substantial clinical and economic benefits over the long-term;
- Cost containment proposals that impose access restrictions based on average, population-wide study results will overlook the different needs of individual patients and discourage adoption of personalized tests and therapies based on these differences;
- “Pay for performance” programs focused on short-term provider efficiency could discourage physicians from using gene-based tests and targeted therapies to optimize care for the individual; and
- A focus on cutting costs in narrow healthcare sectors will fail to optimize patient care and improve care coordination across care settings, which is essential to achieving the promise of personalized medicine.

Alternative approaches are available that support medical progress in areas like personalized medicine while improving healthcare quality and affordability. An overview of such approaches is provided below.

¹ “Household Income Rises, Poverty Rate Unchanged, Number of Uninsured Down.” U.S. Census Bureau. http://www.census.gov/Press-Release/www/releases/archives/income_wealth/012528.html. August 28, 2008.

² Poisal JA et al. Health Spending Projections Through 2016: Modest Changes Obscure Part D's Impact. *Health Affairs*. 21 February 2007;W242-253. Via “Health Insurance Costs.” National Coalition on Health Care. <http://www.nchc.org/facts/cost.shtml>. Accessed October 21, 2008.

The Policy Agenda: Delivering on the Promise of Personalized Medicine

Reforms to achieve care that is preventive, coordinated, evidence-based, and personalized hold the greatest promise for improving healthcare quality and affordability. To support continued progress in personalized medicine, policymakers should support healthcare proposals that:

1) Invest in the science and practice of personalized healthcare by:

a) Supporting basic research in the public and private sectors by creating new incentives for private sector research and expanding funding for the National Institutes of Health and public-private initiatives to accelerate the science of personalized medicine. Of particular importance is the growth of federal investment and resources for educating and training the next generation of research scientists, especially those focused on genomics and personalized medicine.

b) Support federal research and policy to identify and support timely adoption of the medical interventions and health system tools needed to support the emergence of personalized medicine through a national "Personalized Medicine Advisory Commission" (PMAC). The advisory commission would oversee and provide input on federal activities to conduct and synthesize outcomes research in support of evidence-based, personalized health care decisions by physicians, other providers and patients. Commission activities would include: recommending research priorities on the range of medical and health system interventions (such as diagnostic tests, therapies, and approaches to organizing and managing care) that are important to the advancement of personalized medicine; supporting development of methods for conducting and communicating research in ways that enable or enhance the delivery of personalized medicine; and recommending policy approaches that support delivery of personalized medicine.

2) Support for research to identify what works in healthcare (comparative effectiveness research) that:

a) Encompasses all of the elements of care relevant to high-quality, personalized healthcare, including research on diagnostic tests and therapies, processes of care, chronic care prevention and management programs, and approaches to healthcare delivery and benefit design.

b) Generates and communicates evidence in ways that support personalized medicine by accounting for differences in treatment response and preferences among individuals and sub-groups.

c) Applies evidence in ways that support personalized medicine by ensuring that emerging health information technology platforms and performance measurement initiatives support the physician's ability to optimize individual care based on the range of treatment options.

3) Support coverage for preventive services that are facilitated through emerging personalized medicine advances. The science of personalized medicine promises new tools—like genetic predisposition testing—that enhance the ability of individuals and caregivers to engage in early disease prevention and preemption. Steps should be taken to identify and support evidence-based screening, predisposition, and risk-assessment tools that can help predict an individual’s risk for future disease. Additionally, as described above, health reform proposals should include provisions to identify approaches to care delivery and coordination and benefit design that support adoption of predictive and preventive care for these patients.

4) Adopt federal health information technology (HIT) and e-prescribing standards that inform treatment decision-making based on the range of treatment options—taking individual genetic characteristics and other factors into consideration.

Adoption of HIT is an important building block to support preventive, predictive medicine and early disease intervention and to enable higher quality, more efficient healthcare. According to the RAND Corporation, HIT adoption could save and improve many patients’ lives, as well as cut up to \$81 billion a year in health costs.³

HIT and e-prescribing standards adopted by the federal government should provide for transmitting and communicating information on personalized healthcare technologies. HIT decision-support platforms, including e-prescribing, should facilitate communication of information in ways that enable physicians and patients to consider risk and benefit trade-offs of a range of treatment options and to understand how these trade-offs may vary depending on an individual’s genetic profile. Tools that obscure these differences by applying overly simplistic population-based comparative evidence or narrow cost-cutting targets will discourage the evolution of personalized healthcare.

To give patients and providers all of the information that they need to deliver personalized care, e-prescribing platforms should be integrated with the individual’s electronic medical record (so that information on diagnostic test results, co-morbidities, etc. can be used for optimal treatment decision-making). HIT and e-prescribing standards also should enable rapid appeals and prior authorization decisions based on results of molecular diagnostics and other personalized medical information.

5) Adopt performance measures/incentives that facilitate healthcare interventions based on personalized healthcare technologies. Healthcare performance measures adopted by federal agencies should help physicians tailor interventions (including tests, treatments, and care management approaches) based on personalized healthcare technologies. Healthcare measures that cover a broad “episode of care” and longer-term outcomes, rather than point-in-time interventions and short-term outcomes, may be one step that supports this approach.

Provider incentives based on performance measures should allow for exceptions based on individual genetic variations. This allowance will ensure that physicians are not penalized for delivering optimal care for patients who differ from the “average” patient population.

Performance measures that define economic outcomes should account for differences in treatment cost that may arise as the result of delivering optimal, personalized care based on genetic test results and other information.

³ “RAND Study Says Computerizing Medical Records Could Save \$81 Billion Annually and Improve the Quality of Medical Care.” RAND Corporation. <http://www.rand.org/news/press.05/09.14.html>. September 14, 2005.

6) Improve care coordination. Health policies that shift the focus to disease prevention and care coordination will support the adoption of personalized medicine and also offer a key solution for healthcare quality and affordability. Ultimately, through policies that help move us toward consistent delivery of the right treatment for the right patient at the right time, the entire system will benefit from higher quality, more affordable, personalized care.

Developing chronic care management tools that make use of the science of personalized medicine, for example, can help the more than 133 million Americans who suffer from one or more chronic conditions.⁴ They also can make healthcare more affordable: By making basic improvements in preventing and managing chronic disease, the United States could save \$1.1 trillion in 2023, including \$218 billion in savings from direct treatment costs.⁵

Personalized Medicine: A Critical Element of Healthcare Reform

Advances in personalized medicine exemplify the opportunity for meaningful improvement and greater value in the healthcare system—central objectives of healthcare reform. As a result of targeted therapies and other advances in cancer care, for example, survival times of metastatic breast cancer increased by 30 percent during the 1990s.⁶ New, biomarker-based treatments are dramatically enhancing diagnosis and increasing effectiveness and safety of medical interventions, which in turn can help contain overall healthcare costs by avoiding expense from complications that might otherwise result from the wrong diagnosis or treatment, improving patient adherence to therapy, and helping to prevent disease before it emerges.

In contrast, health reform approaches that seek to contain costs by restricting access to and delaying the adoption of medical innovation will hamper the continued development of personalized medicine and ultimately perpetuate outdated approaches and inadequacies that continue to drive system-wide costs up.

Based on our growing understanding of human genomics and related fields, personalized medicine can give us an unprecedented ability to address unmet health needs in ways that:

- Prevent disease by identifying an individual’s likelihood of developing it in the future and by enabling individualized approaches to address key risk factors;
- Detect the onset of disease at the earliest stages based on new biological markers and changes at the molecular level to preempt disease progression; and
- Tailor treatments to each patient based on genetic and other factors, so each individual receives the safest, most effective care available for them.

Advances in personalized medicine can help bring about a new era in which medicine is preemptive, predictive, and patient-centered. Policies crafted with personalized medicine in mind will empower clinicians with the tools and information they need to deliver the right treatment to the right patient at the right time (the *first* time)—and ultimately benefit patients by significantly enhancing the quality, value, and safety of the treatment and care that they receive.

⁴ “The Growing Crisis of Chronic Disease in the United States.” Partnership to Fight Chronic Disease. <http://www.fightchronicdisease.org/pdfs/ChronicDiseaseFactSheet.pdf>. Accessed October 21, 2008.

⁵ “An Unhealthy American: Economic Burden of Chronic Disease – Charting a New Course to Save Lives and Increase Productivity and Economic Growth.” The Milken Institute. <http://www.chronicdiseaseimpact.com/> and <http://www.milkeninstitute.org/>. October 2007.

⁶ Chia SK et al. The impact of new chemotherapeutic and hormone agents on survival in a population-based cohort of women with metastatic breast cancer. *Cancer*. 2007;110(5):973-979.

Conclusion:

America stands on the cusp of significant scientific advances that promise to usher in a new era of personalized medicine. We also stand at the threshold of a new debate about how to address challenges with healthcare access, quality, and cost.

A supportive policy framework is needed to foster and help sustain research, development, and adoption of personalized medicine-based technologies and treatments. The Personalized Medicine Coalition supports healthcare reforms that improve quality and affordability while fostering continued medical progress, and it commits to advancing patient care through development and adoption of evidence-based, personalized medical technologies and care delivery models. By incorporating the elements described above, healthcare reform can facilitate the advancement and adoption of new personalized medicine technologies while meeting the challenge of improving access, quality, and affordability.

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