PMC
PERSONALIZED MEDICINE COALITION
Celebrating 10 Years

Policy Position: Payer Principles

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Recognizing that a broad range of molecular-based products and services form the basis of the new field of personalized medicine (PM) and that the Personalized Medicine Coalition (PMC) represents a diverse group of stakeholders committed to the advancement of personalized medicine,

Recognizing that personalized medicine is unique in its potential to both improve health outcomes and the efficiency of health care delivery through the application of targeted interventions based on knowledge of an individual's molecular makeup and disease state,

Also recognizing that there are both short-term and long-term health benefits of personalized medicine diagnostics and therapeutics, and that it is frequently in the patient's best interest for payers to have a long-term, system-wide view when making coverage and reimbursement decisions,

Also recognizing that reimbursement practices governing new technologies have a profound impact on both patient access and incentives for investors and industry to develop innovative companies, products and services,

Convinced that the PMC and payers have a shared interest in improving the health of all beneficiaries by ensuring access to new personalized technologies while encouraging continued medical progress,

Also convinced that the pathway to resolution of reimbursement issues is through multidisciplinary dialogue conducted in a transparent and deliberative fashion,

The PMC affirms the following principles governing reimbursement decisions:

1. Reimbursement decisions covering new PM products and services should be evidence-based.

PMC supports the use of evidence-based medicine (EBM) to inform patient and physician choices about the PM options that best meet the needs of the individual. EBM fundamentally represents the appropriate use of best available evidence to support good decision-making by patients and physicians, and integrates current available evidence, clinical expertise and patient understanding and values. Depending on the specific type of intervention, the nature and breadth of evidence available may vary widely (see Section 2a below). In addition, where EBM has sometimes been criticized as promoting a "one size fits all" approach to clinical management, the PMC believes that PM research results can help provide the evidence to support variations in individual patient management decisions.

EBM efforts at all levels (individual and policy-level) should help inform and support decisions made by patients and physicians. Application of EBM at the policy level should support, not replace, the ability of patients and physicians to make personalized treatment

decisions to meet the needs and values of the individual, and should have quality improvement as its central goal.

2. Third party coverage and reimbursement are essential to ensure appropriate access to PM products and services.

PMC believes that, given the potential of personalized medicine, both public and private payers should work cooperatively with PMC members to ensure that medical technologies and patterns of care delivery are available to support optimal patient outcomes, permit targeted therapy and reduce adverse drug events.

a. Evidence

When new personalized medicine technologies come to market based on strong clinical evidence (e.g., prescription medicines, which are approved by FDA based on rigorous clinical trial data), they should be made available to patients. If payers seek additional evidence, they should work collaboratively with innovators to ensure that evidence requirements are appropriate and do not create barriers to patient access to new personalized medicine technologies. In some instances, (e.g., diagnostic tests) additional evidence can be particularly difficult to generate. In these situations, payers should work with innovators to develop appropriate evidentiary pathways for these promising technologies.

As with most new technologies, the evidence available early in their life cycle might not be of the highest level or as conclusive as that available for other diagnostics and therapeutics. However, if insurers make use of these promising technologies in "real world" situations, valuable experience can be gained with respect to their clinical and health economic impact. Given the potential for savings associated with PM approaches, PMC believes that payers should forge creative partnerships with technology sponsors to translate this clinical potential into clinical practice.

b. Coverage & Payment

PMC believes that payer processes to consider whether or not to include PM approaches and technologies as covered benefits should be made through transparent and timely review processes that permit stakeholder participation.

Whenever possible, payers should focus on long term outlays in addition to short term outlays when making coverage and reimbursement decisions, because personalized medicine technologies have the potential to produce results over the longer term and potential cost savings may not be realized immediately.

3. PMC encourages novel approaches by federal agencies to both promote research efforts for personalized medicine as well as the use of personalized medicine products and services to improve the quality and value of care. PMC notes that a number of federal agencies have recognized the potential benefits of PM and are currently exploring new roles for personalized medicine in their regulatory and research activities. PMC supports these activities.

a. FDA: PMC supports the FDA's efforts to move beyond its traditional drug safety approach in its regulation of pharmaceuticals as evidenced by the 2005 Guidance for Industry on Pharmacogenomic Data Submissions. We are similarly encouraged by the decision to provide funding for the FY 2007 Critical Path to Personalized Medicine initiative.

PMC understands that FDA would like to supplement (and eventually replace) traditional drug safety approaches in the regulation of pharmaceuticals, utilizing PM tools. FDA recognizes that PM has the potential to provide better results than relying on current FDA "trial and error" dosing approaches and reactive drug surveillance systems. The FDA Critical Path initiative recognizes a key role for pharmacogenomics in identifying those at high

risk for side effects from new drugs and targeting drug therapy. PMC supports FDA efforts to identify the patient subgroups with a high probability of a positive response through PM approaches.

b. NIH: PMC supports NIH efforts to foster basic and translational research in personalized medicine.

The NIH Roadmap is intended to accelerate medical discovery by providing the research community with wide access to technologies, databases and other scientific resources needed to advance our understanding of biological systems and build a better "toolkit" for medical research in the 21st century. PMC sees the NIH Roadmap initiative as a valuable resource promoting the development of new technologies used in personalized medicine. To realize the broader goals of the Roadmap will require investment in novel, integrative approaches within academia to both promote translational research and train the researchers of the future, such as the new Clinical and Translational Science entities. In addition, these efforts will often require public-private partnerships such as the newly created Genetic Association Information Network, which is designed to study the genetic risk factors for complex diseases. The PMC recognizes the value of both types of strategies to develop the necessary expertise and infrastructure to deliver on the promise of personalized medicine.

c. CMS: PMC believes that PM approaches would prove valuable to the agency as it works to ensure the quality of, and access to, appropriate care for its beneficiaries.

CMS Administrator Mark McClellan has stated that Medicare does not know enough about the benefits and risks of drugs and drug combinations in the "real world"-at a time when there is "a tremendous potential to use new sciences like genomics and information technology to do a much better job to match the drugs to patients who will really benefit and who will not have serious complications." PMC encourages the Centers for Medicare and Medicaid Services to ensure that its policies support the development of PM to improve the safety, quality and value of care for its beneficiaries. This is especially important given the implementation of the prescription drug benefit in 2006.

For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 provides numerous opportunities to improve the quality, effectiveness and efficiency of health care delivered to Medicare beneficiaries through its Medication Therapy Management programs, quality reporting initiatives and increased focus on preventive strategies. Personalized medicine approaches can offer new methods to help realize these goals. Similarly, PMC believes that PM advances will play an increasingly important role in helping realize the goals of CMS' quality improvement initiatives such as "Pay for Performance" programs targeting hospitals, physicians, integrated health systems and disease management organizations.

d. AHRQ: PMC supports AHRQ's efforts to implement the principles of evidence-based medicine (see section 1 and 2) to improve the quality of health care in the US. We are encouraged by the transparency and collaborative approach being taken in their implementation of Section 1013 of the Medicare Modernization Act through the Effectiveness Program.

PMC understands the importance of both the disease-based research on outcomes, comparative clinical effectiveness and appropriateness of health care items and services under Section 1013, as well as the methodological research being conducted under this program. Such research can support the appropriate use of health interventions, as well as the approaches to care management and delivery that encourage appropriate use of interventions and improve patient outcomes.

PMC also believes that AHRQ should recognize a key role for personalized medicine approaches in increasing the effectiveness of therapeutic interventions through identification of those at high risk for side effects and/or those with a high probability of a positive response to targeted therapy.

4. Support the development of new policies and legislation to expand payer coverage and reimbursement of PM products and services focused on disease prevention.

a. Individuals who lack current signs, symptoms or personal histories of illness stand to benefit clinically from predictive and predispositional genetic tests and services. Currently, many products for prevention do not reach the market because of significant regulatory, reimbursement and other barriers.

b. HHS and its components should help spur innovative research into diagnostics and treatments for primary prevention by clarifying and streamlining the regulatory requirements for approval. For example, FDA should seek to make better use of surrogate endpoints, since this will shorten development times and get needed medicines to patients in a timely manner.

c. HHS should ensure that preventive treatments and diagnostics are reimbursed at appropriate levels by public payers and government programs.

5. Pursue greater transparency and predictability of payment policy decisions by using a range of established evaluation methods.

PMC will work with public and private stakeholders to develop both a methodology-focused and implementation-focused research agenda to determine how best to apply these evaluation methods (e.g., systematic reviews, patient-reported outcomes, meta-analyses, clinical trials, observational research, cost-effectiveness analysis, willingness-to-pay and other types of scientific evaluations).

6. Pursue changes in in vitro diagnostic coding and payment systems to remove reimbursement as a barrier to innovation.

a. Personalized medicine is an emerging paradigm dependent on innovative diagnostic tools.

b. Current coding and payment systems for in vitro diagnostics inadequately reflect the technological, clinical and health economic impact of novel tests.

c. PMC supports the development and implementation of a payment system for diagnostics that better recognizes the clinical, economic and other benefits of improved patient outcomes.

d. PMC supports the creation of a new process for diagnostic tests that is open, systematic, accountable and which includes public stakeholder input into pricing and coding decisions.

7. Work to ensure that no individual or group is discriminated against or stigmatized by payers on the grounds of a personalized medicine assessment.

a. PMC believes that it is essential for patients and research study volunteers to have the assurance that their genetic information, gathered for medical care and research, will be treated with the utmost discretion and will not be used for discrimination in health insurance coverage or employment.

b. Nation-wide assurance that payers will not use genetic information to discriminate against individuals or groups will only be achieved through federal legislation. The PMC supports passage of the Genetic Information Non-Discrimination Act and will continue to work with other coalitions toward this end.

c. The PMC also supports IRB efforts to ensure that results of research-related genetic testing are kept separate from the individual's medical record. Given the unconfirmed and preliminary nature of personalized medicine

assessments conducted as part of research, it is critical that these study results not be available to payers through their inclusion in medical records.