



A coalition to drive personalized medicine forward

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The widespread adoption of personalized medicine will require the harmonization of many components: advances in technology; changes in healthcare infrastructure and medical practice convention; improvements in the efficiency and quality of healthcare delivery; diagnostic and therapeutics business models for genetically defined markets; attempts by government and private payers to justify a new genre of tests and drugs; a different approach to regulatory oversight; and, of course, the ethical and legal issues that go along with the extensive use of genetic information in medical records. Personalized medicine is, in fact, a systems-level challenge, in which no single component or stakeholder can hope to push through a coherent and encompassing policy for adoption on its own. The need for a nationwide, multi-industry policy consensus for the next generation of medicine has led to the creation of the Personalized Medicine Coalition – an independent, non-profit organization of over 20 leading pharmaceutical, diagnostic, biotechnology and information technology companies, as well as major academic institutions and governmental agencies. The Coalition provides a structure for achieving consensus positions on crucial public policy issues and serves as a forum for debate and education in personalized medicine.

The goal of the pharmacogenomic approach is to deliver personalized healthcare to the patient based on knowledge of that individual's genetic make-up as well as an understanding of how a drug is working at the molecular level, in order to administer therapies that will convey maximum effectiveness and safety. While this appears to be a very simple concept, in the context of current healthcare systems and commercial markets, it is not so simple to implement.

Personalized medicine may be described as a 'systems engineering' or 'industrial engineering' challenge. It will require diverse technologies to work together, collaboration between professional workforces, information technology (IT) infrastructure supporting the integration of data and clinical records, and appropriate incentives for insurance payers, physicians, hospitals, and pharmaceutical and diagnostics companies (Figure 1).

However, healthcare in the USA is highly decentralized and market driven. In many respects, this system is more effective in supporting the rapid dissemination of and access to innovative medical technologies. At the same time, problems such as fragmentation of care delivery and misalignment of payment incentives can discourage the adoption of innovative technologies or modes of healthcare delivery. Legislative and policy decisions on one issue, such as patient privacy or payer reimbursement, are often made

without full consideration of the impact such a decision might have in other areas. As a result, a system-wide perspective is necessary when seeking to address current and potential barriers to the adoption of innovative technologies, such as those embodied in personalized medicine.

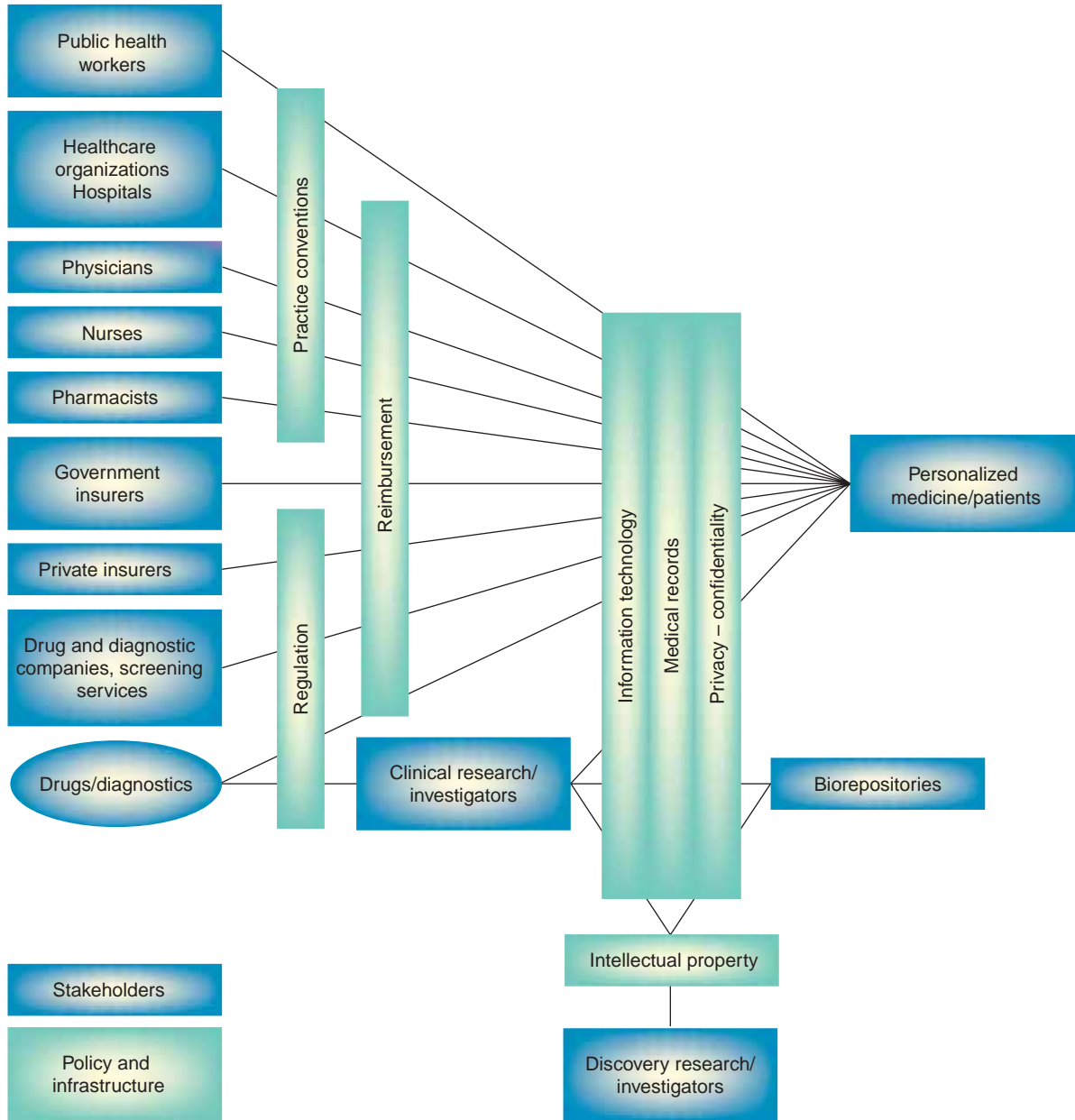
The fact that a comprehensive policy landscape for personalized medicine has not yet emerged is not surprising given its recent origins, rapid progress, and the multi-disciplinary nature of translational (bench-to-bedside) research. A broad array of government agencies, healthcare organizations and companies must work together to implement personalized medicine, including those focused on IT, medical imaging, diagnostics, medical devices, laboratory services, biotechnology, and pharmaceutical research.

To meet this challenge, the Personalized Medicine Coalition (PMC), based in Washington DC, USA, was funded and launched in 2003 by over 20 organizations (Box 1) to foster a better understanding of personalized medicine across industries. Today, more than 50 organizations have participated in coalition educational forums. As a neutral meeting ground for discussing policy among all players, the PMC will provide a resource of knowledge and expertise to legislators, physicians, hospitals and others seeking to restructure or realign their goals and incentives for the next generation of medicine.

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future
medicine

Figure 1. Multiple components of a personalized medicine system.



A diagram illustrating the interdependent network of stakeholders and the policies and infrastructure that cut across their interactions. A coherent set of policies and infrastructure must be coordinated for personalized medicine to move forward.

Personalized Medicine Coalition mission and goals

The primary mission of the PMC is to ‘debate, educate, and communicate’ and, in the process, become a resource for thought leadership and consensus-building among interdependent industries and government agencies (Box 2). An important role of the coalition is to zero in on current policies and incentive structures that

work against gains in personalized medicine and support discussion leading to the development of policies and incentives that are more supportive of change.

In the coming months, the PMC will consider a number of key public policy issues critical to the adoption of personalized medicine. These include, but are not limited to, electronic medical record standards, payer reimbursement,

Box 1. Current Personalized Medicine Coalition members.

- Abbott Laboratories
- Affymetrix
- American Clinical Labs Association
- Amgen
- AstraZeneca
- BIO: The Biotechnology Industry Organization
- Center for Medicare and Medicaid Services (agency partner)
- Exagen Diagnostics
- Genentech
- Genetics and Public Policy Center
- Genomas
- Genomic Health
- Genzyme
- George Washington University
- IBM Life Sciences
- Millennium Pharmaceuticals
- National Cancer Institute (ex-officio)
- National Human Genome Research Institute (ex-officio)
- Pfizer
- PhRMA (the Pharmaceutical Research and Manufacturers of America)
- Roche
- Siemens
- The Genetics Alliance
- US Food & Drug Administration (agency partner)

global developments in biorepositories, patient use and acceptance of personalized medicine, and the impact of personalized medicine on intellectual property, ethics and privacy laws.

Electronic medical records

Electronic medical record standards and classification systems can facilitate the optimal use of clinical information in genomic medicine and eventually combine it with data from research laboratories. Current stakeholders include physicians who record the data and who are accustomed to paper-based systems with a level of detail that may be relevant for traditional medicine, but inadequate for genomics-based medicine. Patients will be concerned about confidentiality and the control of ownership of their medical data – who gets access to what part of their medical record, and when. Combining information from multiple sources into one data-bank is also a challenge, putting one up against the social and political forces that govern the way hospitals work, and the relationship among independent healthcare providers (e.g., physicians, surgeons, pharmacists, nurses, and specialists).

Box 2. Goals of the Personalized Medicine Coalition.

- Provide opinion leadership with respect to the evolving discussion of public policy issues that affect personalized medicine.
- Help educate the public, policy-makers, government officials and private sector healthcare leaders about the public and personal health benefits of personalized medicine.
- Serve as a forum for identifying and informing others of those public policies that may impede the ability to deliver the promise of personalized medicine.
- Create a structure for achieving consensus positions on crucial public policy issues and supporting changes needed to further the public interest in personalized medicine.

An ongoing dialog among these groups, as well as patient advocacy organizations and IT vendors in the context of the PMC, can lead to a mutual understanding, if not a consensus, on what needs to be done to move forward.

Equitable payer reimbursement

Equitable payer reimbursement will emerge only when the relevant government and private insurers begin to recognize the value that can come through more effective targeting of therapies to individual patients. However, recognition of this value may not be enough to change reimbursement patterns to support adoption, since many payers remain focused on short-term budget impacts and unit costs, a 'necessary' strategy in light of rapid subscriber turnover. Only a dialog among all parties involved can enable a policy that aligns patient welfare and payer incentives to support personalized medicine.

Biorepositories

Biorepositories represent a tremendous potential resource for the advancement of personalized medicine. The collection and storage of millions of human tissue and biofluid specimens for research can lead to the identification of genetically defined patient populations that respond better than others to a particular drug or experience fewer side effects. However, the hundreds of biorepositories in existence today vary greatly in their sample and data quality, and largely lack the ability to share information to facilitate searching and access to materials. Informed consent, privacy and other human subject policies are uneven and are not

always amenable to genetic investigation. Standard operating procedures, a shared IT infrastructure, and robust informed consent and other human subject practices for biorepositories must be supported as part of a coherent policy for technology advancement in personalized medicine.

Intellectual property issues

Intellectual property issues will need to be addressed. While much of the gene patenting debate has been worked out through the US Patent and Trade Office (USPTO) policy and litigation, the situation may become increasingly complex with multiple parties holding patents to multiple genetic markers. This possibility has led to pharmaceutical companies joining together in the SNP Consortium to put as many markers as possible into the public domain. New questions will arise that will require the USPTO, the courts and the companies involved to reconsider intellectual property policy once again:

- Will licensing become a barrier if multiple gene markers are required for a single product?
- What will the effects be on the branded drug lifecycle?
- What are the risks/rewards in the relationship between large pharmaceutical companies and smaller independent players developing pharmacogenetic tests for drugs already on the market?
- Will there be incentives for applying pharmacogenetic tests to drugs that are no longer covered by patent protection?

Regulatory policy

Regulatory policy has concerned the pharmaceutical industry since pharmacogenomics became a prominent topic in research. Pharmaceutical companies are now collecting large volumes of genetic data. There is a valid concern that if these exploratory data are submitted to the FDA, they could be inappropriately used to inform regulatory decisions, with significant economic implications. Such decisions could lead to requirements for the co-development/approval of diagnostic tests, new testing for marketed drugs, or labeling restrictions for new and marketed drugs. A number of potential regulatory issues have been addressed by the FDA's draft guidance on the submission of genomic data [1,101], but continued dialog is needed to address all these issues.

Ethical and privacy concerns

Ethical and privacy concerns in personalized medicine are being addressed by several other organizations (e.g., the Nuffield Council on Bioethics [2,102]), but will be considered by the PMC as well in the context of debate on all areas of policy. Issues to be addressed include the protection of medical records and privacy, the equitable distribution of benefits, the prevention of discrimination, and new ethical and legal issues facing the physician in the administration of personalized medicine.

Educate and communicate

Education and communication will be critical components of the PMC mission. Educational initiatives for healthcare practitioners, policy-makers, scientists and others will help to build an informed set of stakeholders, who will then contribute more productively to the policy debate. Communication efforts will be directed toward disseminating the consensus opinions of the Coalition.

Personalized medicine will pose significant challenges for a professional workforce unaccustomed to employing the concept of genetics in their practice, and educational programs will be crucial to its adoption. The FDA and the Centers for Disease Control and Prevention (CDC) have taken a lead in addressing this challenge.

In order to set new policy and procedures for companies submitting pharmacogenomic data and to establish credibility among the industry that it regulates, the FDA has implemented an education program for its own medical and scientific staff [3,4], established a format for dialog with industry through workshops and conferences, and published a draft guidance on pharmacogenomics submissions [1] to allay the industry's fears around the impact on clinical trials and product approval.

The CDC recognized that establishing the prevalence of human gene variants known to interact with environmental factors will be critical in assessing the impact of environmental and lifestyle interventions. In order to build a public health workforce capable of interpreting and using genomics in its practice, the agency has placed an emphasis on education [5,103]. In 2001, the CDC established three Centers for Genomics and Public Health [104] that were charged with developing genomics training programs for public health workers.

In contrast to the efforts of the FDA and the CDC, most medical schools have not implemented comprehensive educational programs to

Highlights

- The PMC has been established by over 20 leading companies, academic institutions and government agencies to facilitate education and consensus on matters critical to the adoption of personalized medicine.
- Personalized medicine is a systems-level challenge requiring multi-industry collaboration and the harmonization of technology, healthcare infrastructure, medical practice conventions, ethical and legal policy, intellectual property policy, regulatory policy and new business strategies.
- Healthcare in the USA is highly decentralized and market driven – a structure that can spawn innovation but can also inhibit the widespread adoption of innovative technologies and approaches, such as those embodied in personalized medicine.
- No single stakeholder can hope to push through a coherent and encompassing policy for personalized medicine adoption on its own, yet a coherent set of policies and infrastructure must be coordinated for personalized medicine to move forward.
- The PMC will provide a resource of knowledge and expertise to legislators, physicians, hospitals and others seeking to restructure or realign their goals, incentives and policies for the next generation of medicine.

prepare their students for the advent of personalized medicine [6]. These and other stakeholders have developed educational gaps that may lead to misunderstanding or resistance to personalized medicine concepts.

The PMC will help to identify areas where educational programs are required, and provide its own educational and communication initiatives to inform policy-makers and healthcare leaders about the importance of personalized medicine and the steps they can take to facilitate its positive evolution.

Call to action

The PMC will encompass a broad spectrum of academic, industrial, patient and healthcare provider constituencies, and the organization continues to seek additional members to join the fold and contribute their perspective. The organization

is calling for members from universities and academic medical centers, non-profit research entities, trade organizations, patient advocacy groups, government officials (ex-officio), healthcare organizations and providers, insurance payers, IT companies and research-based companies offering research tools, diagnostic products, screening services and therapeutic interventions. For more information, please visit the PMC website [105].

Conclusion

While personalized medicine seeks to reduce the burden of trial and error in healthcare, the PMC seeks to reduce the burden of trial and error in healthcare policy. It is only by convening all the key stakeholders for rational debate and formulation of consensus that a coherent strategy will take shape that will enable us to benefit sooner from the vision of 21st century medicine.

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