

Personalized Medicine Coalition Mission and Principles

The Personalized Medicine Coalition (PMC), representing a broad spectrum of academic, industrial, patient, provider, and payer communities, seeks to advance the understanding and adoption of personalized medicine concepts and products for the benefit of patients.

I. The Personalized Medicine Coalition

The Personalized Medicine Coalition exists to advance the understanding and adoption of personalized medicine concepts and approaches for the ultimate benefit of the patients who need them.

The Coalition seeks to serve as a source of information on public policy matters that will affect the realization of the promise of personalized medicine.

The membership of the PMC is open, with the goal of attracting universities and academic medical centers, non-profit research entities, relevant trade associations, patient organizations, government officials (ex-officio), healthcare organizations, healthcare providers, information technology companies, and research-based commercial companies that offer an array of products and services including research tools, diagnostic technologies and products, screening services, and therapeutic interventions.

II. Operational Guidelines

The Personalized Medicine Coalition is guided by the following operational principles:

- The PMC serves as an umbrella organization for stakeholders who are conducting personalized medicine education in both the public and private sectors;
- The PMC offers stakeholders a forum to discuss public policy and regulatory issues affecting personalized medicine — and, where possible, to develop consensus positions on key issues;
- The PMC collaborates with member organizations and outside experts in identifying, analyzing and addressing ethical, legal and social implications of personalized medicine; and
- The PMC provides networking opportunities for the many and varied stakeholders in personalized medicine.

III. Vision

The vision of personalized medicine is to better use characteristics of the individual (and his or her disease) to help identify which of the many treatment alternatives to use in addressing a medical problem¹. Rapid advances in biology and information technology, as well as progress in sequencing the human genome and the genomes of viruses and bacteria, offer new building blocks and tools to advance medicine in both its scientific and clinical domains.

- **Research and development:** A personalized medicine approach may allow the expanded use of biomarkers for more focused and targeted drug development. More sophisticated knowledge about the role of individuals' genetic and biological characteristics in disease may help expedite development of drugs that more effectively treat diseases in specific populations.
- **Clinical practice:** Personalized medicine also may improve diagnostic and prognostic processes through screening methodologies that identify patients in particular disease subgroups and/or those predisposed to a particular disease. Better use of biomarkers also could result in more informed selection and dosing of medicines, thus reducing side effects and improving efficacy. Through improved determination of likely outcomes of drug treatments — which in turn may help physicians decide when, whether and how to treat patients — personalized medicine may help improve the quality and cost effectiveness of medical interventions.

¹ The concept of tailoring a patient's treatment is not new. The wide variation in innovator medicines available to treat a specific condition today already allows a certain level of personalized care. Doctors and patients often engage in a process of trial and error to find the right medication to alleviate a particular patient's condition, with the minimum of undesirable side effects. Numerous factors — genetic differences, environmental influences, diet, competing co-morbidities — affect which drug works best in a specific patient. Sequencing of the human genome now allows us to investigate in more detail the genetic differences among patients and populations, and the effects of those differences on the metabolism and response to various drugs. In an era of personalized medicine, patients will still be assessed and treated based on information gleaned from studies of groups or populations, but through better definition and understanding of more precisely defined groups it is hoped that clinical predictability will be improved for individuals in those groups. In effect, we believe that the personalization of medicine as it exists today will be practiced more precisely and uniformly in the future. However, we think it is unlikely that science will afford us a one-to-one prediction between the genetics of the individual and the selection of the "right" drug — a physician will still need to consider the improved diagnostic information regarding the specific patient and exercise his or her discretion in selecting the medication he or she feels is most appropriate.

IV. Definition of Personalized Medicine

Personalized medicine means different things to different people. Some have suggested that personalized medicine is the application of genomic data to better target the delivery of medical interventions. Others have suggested that it is a crucial tool in the discovery and clinical testing of new products. And others have suggested that it involves the application of sophisticated, clinically useful diagnostic tools that may help determine a patient's predisposition to a particular disease or condition. In fact, personalized medicine can encompass all of those concepts.

In theory, personalized medicine is the management of a patient's disease or disease predisposition, by using molecular analysis² to achieve the optimal medical outcomes for that individual — thereby improving the quality of life and health, and potentially reducing overall healthcare costs.

In practice, personalized medicine is a comprehensive approach utilizing:

- Molecular analysis of both patients and healthy individuals to guide decisions throughout all stages of the discovery and development of pharmaceuticals and diagnostics; and
- Applying this knowledge in clinical practice for a more efficient delivery of accurate and quality healthcare through improved prevention, diagnosis, treatment, and monitoring methods.

V. Public Policy Issues Impacting Personalized Medicine

Several clusters of significant public policy issues mark the pathway to the growth and acceptance of personalized medicine. While none of these issues is unique to personalized medicine, government regulation of clinical trials, intellectual property rights, licensing practices, healthcare reimbursement, and privacy are among the areas that may need to be re-examined.

Currently, public policy decisions in the healthcare arena are made in a fragmented and uncoordinated way, without consideration of the impact one decision could have on other policy issues. For providers of products and services to be able to deliver on the promise of personalized medicine, policymakers must intelligently and comprehensively review the array of issues that affect the science and clinical practice of personalized medicine. The PMC exists because there is real risk that public health and medical practice will suffer unintended consequences unless policymakers coordinate their efforts. The next generation of medical practice — personalized medicine — demands a coherent, integrated approach to the legal, financial, social and professional issues that will shape its development and application.

Intellectual Property

A strong intellectual property system is necessary to stimulate investment in innovation. It is essential that government patent systems offer protection for innovations relating to personalized medicine, as well as high quality patent examination that allows patents of appropriate scope and quality.

Regulatory Oversight

The development of personalized medicine may require that regulatory bodies adopt some new approaches to product approval. This will entail new guidance about the processes for obtaining approval to commercialize new therapeutics, including when and under what circumstances the use of a new drug must be preceded and/or accompanied by the use of a diagnostic or screening test. These clinical trial rules will influence the drug, biotechnology, diagnostic and device industries.

Public and Private Sector Reimbursement

If the healthcare system is to secure the full benefits of personalized medicine, it must provide full and fair reimbursement for new technologies, products and services, based on market principles to the extent possible. The unit cost of new products could be greater than the products they are replacing; yet these new products may offer superior results for the smaller pool of patients for whom they are appropriate. The reimbursement system — both governmental and private payers — must have coverage and payment policies that support the timely adoption of new personalized medicine technologies, including both diagnostics and therapeutics.

² In addition to genes, environmental factors — such as diet, exposure to toxins, viruses, etc. — are also involved in disease processes.

Moreover, the issue of whether the government should encourage or subsidize — though targeted grant programs — the development of the infrastructure to permit the application of personalized medicine presents its own policy dilemma. For example, both medical education about genetics and information technology (IT) systems in healthcare institutions will need to be improved to permit the use of more complex pharmacogenomic data and more detailed and accurate health history data in research and in clinical practice.

Privacy, Confidentiality and Patients' Rights

The protection of patient privacy and other rights is critical to creating public confidence in the collection of the data necessary for the practice of personalized medicine. Patients may be less willing to provide tissue and blood samples, or to disclose medical history information, if they fear that their data will be misused or their privacy will be violated. Appropriate informed consent for both research and treatment also is critical. Thus, assuring effective protection of sensitive information is a necessary prerequisite to the collection of the individually identifiable data that allow personalized medicine research and practice. Specific issues that may need to be addressed include: the potential impact on insurability and/or employment of “orphan patients” or “non-responders”; the potential for our society to use genetic characteristics in discriminatory ways; the implications for an ethnic group of genetic testing results that are common to a significant percentage of its members; the effects of predictive genetic test results on the personal responsibility and psychological well-being of the individual who is tested; and the rights and well-being of “third-party” non-consenting family members of an individual who undergoes a predictive genetic test.

VI. Goals

The goals for the PMC are:

- To provide opinion leadership with respect to the evolving discussion of public policy issues that affect personalized medicine;
- To work, with others as appropriate, to help educate the public, policymakers, government officials, and private sector healthcare leaders regarding the public and personal health benefits of personalized medicine;
- To 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; and
- To work toward creation of a structure for achieving consensus on those public policy issues, if any, for which changes are needed to further the public interest in personalized medicine.

VII. Strategy

The PMC's strategy for meeting its goals includes:

- Working with existing organizations, including relevant professional and trade associations, to improve the understanding of personalized medicine and the interrelated issues it raises;
- Developing consensus on the principles that ought to govern the development of public policy affecting personalized medicine, including policies regarding intellectual property, regulatory approval, reimbursement, patient privacy and dignity, confidentiality, and protections against misappropriation of personalized data; and
- Educating policymakers in the federal government, state officials when appropriate, and private sector healthcare leaders about the importance of personalized medicine and identifying concrete steps that they can take to facilitate the positive evolution of personalized medicine.

In order to implement its strategy and achieve its goals, the PMC may undertake tactics to advance the concepts of personalized medicine. These may include activities internal to the participating industries, the think tank community, the medical community, and the reimbursement community. Specific tactics may also include external steps relative to government policymakers, public education, and press-related measures.