Executive Summary

The concept of personalized health care has attracted considerable scientific, medical, commercial and policy interest for its potential to sharpen the focus of health care and improve its effectiveness and efficiency. It is intended to shift diagnostic and therapeutic interventions from more traditional, population-based, empirical approaches to those that are more scientifically-informed and tailored for individual patients. Personalized health care is intended to "deliver the right treatment to the right patient at the right time—every time."[1]

Personalized health care (PHC) draws from information about differences in individual genomes, molecular- and cellular-level disease processes, health states, behavioral and environmental determinants and response to interventions. It applies this to deliver patient-specific health care that reflects individual risks and benefits of particular treatments, to determine risks of particular conditions or diseases and to facilitate the discovery and validation of health care products and other interventions. PHC may involve genetic and molecular testing, functional imaging and other means to determine a patient's predisposition for particular health care responses and outcomes. Continued advances in health information technology should facilitate PHC research and delivery.

As part of a broader vision of advancing and leveraging medical research to improve and transform health care in the US, the Secretary of the US Department of Health and Human Services (DHHS) has identified PHC as one of the Department's top 10 priorities in the near- and long-term future.[2]

In order to advance the Secretary's vision for PHC, the Office of the Assistant Secretary for Planning and Evaluation (ASPE) commissioned The Lewin Group to convene the PHC Expert Panel for a one-day meeting on March 20, 2007, at the Hubert H. Humphrey building in Washington, DC.

The purpose of the PHC Expert Panel was to provide input to the Office of the Secretary, DHHS, toward realizing the integration of PHC into clinical and public health practice. Panelists' observations and findings from this facilitated discussion are intended to help inform and enable the Secretary, other policymakers and other stakeholders to chart important steps over the next 5 to 20 years for transforming current medical practice into a system of PHC.

The PHC Expert Panel was comprised to represent various key stakeholder perspectives involved in the integration of new technologies into clinical and public health care. Twenty-two experts representing the private sector (e.g., payers, industry representatives, advocacy representatives) and public sector (e.g., Food and Drug Administration, Centers for Medicare & Medicaid Services, National Institutes of Health) served on the Expert Panel.

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1 HHS Secretary Leavitt Announces Steps Toward A Future of "Personalized Health Care." Washington, DC: US Department of Health and Human Services. March 23, 2007. Accessed May 1, 2007. http://www.hhs.gov/news/press/2007pres/20070323a.html.

2 Secretary Mike Leavitt: DHHS priorities. Washington, DC: US Department of Health and Human Services. Accessed April 13, 2007. http://www.hhs.gov/secretary/priorities/.

Prior to convening, panel members were provided with background information to help them prepare for the meeting. This included an environmental scan of issues relevant to PHC and a brief discussion guide (both prepared by The Lewin Group) that outlined the main issues, a set of forward-oriented assumptions and a set of questions intended to prompt and focus discussion on each main issue.

During the Expert Panel meeting, panelists considered and discussed the following five main issues pertaining to the integration of PHC into clinical and public health practice:

- Demonstrating clinical validity and utility of PHC
- Demonstrating value/cost-effectiveness of PHC
- Identifying the role of PHC in reducing health disparities
- Educating and engaging providers and patients about PHC
- Using databases to build evidence and inform decisions in PHC

The Expert Panel was not charged with providing recommendations to the Office of the Secretary. However, the Expert Panel was asked to express "what the Office of the Secretary needs to know" toward realizing the DHHS initiative in PHC. In particular, panel members were asked to comment on current and potential enablers and barriers to PHC, incentives and disincentives, the pre- and post-marketing environments, the integration of PHC with health information technology and the potential view of PHC as being "disruptive" to the current health care system. Panel members also were asked to comment on potential stakeholder perspectives (e.g., patients/consumers, providers, payers, policymakers, employers) concerning these issues.

The Expert Panel's main observations and findings for each of the five main issues are as follows.

A. Demonstrating Clinical Validity and Utility

- The great share of population disease burden arises from complex disease processes involving often inadequately-understood genomic, environmental, behavioral and other factors.
- Although usually preferred where feasible, randomized controlled trials (RCTs) are not the only means of generating needed evidence on the clinical utility (risks and benefits) of tests and other interventions used in PHC.
- Observational studies can augment the evidence base for PHC, including to assess clinical validity of tests. However, they generally are less useful for assessing clinical utility and are not adequate substitutes for RCTs in establishing cause-and-effect relationships between PHC interventions and health outcomes.
- New study designs and methods should facilitate evaluation of PHC technologies.
- Genetic/genomic tests are of little or no clinical value without availability of validated, associated interventions—whether prevention strategies, treatments, behavior changes, life planning alternatives or others—whose use is informed by those test results.

- Clinical utility of genomic testing and other PHC interventions must be supported with data generated in real health care settings.
- Integrated data collection, spanning pre- and post-market phases, is needed to demonstrate clinical validity and utility of PHC.
- Coverage of PHC interventions by public and private sector payers should be subject to data collection throughout their lifecycles.
- Better alignment of requirements and processes of DHHS agencies and other organizations responsible for regulation and reimbursement would improve the generation of evidence for clinical validity and utility.
- Standards are needed to establish robust evidence requirements and methods for assessing the validity and utility of PHC interventions.

B. Demonstrating Value

- While its impact on aggregate health care spending remains to be determined, PHC has considerable potential to improve the return on health care investment.
- Despite its promise, the evidence base for demonstrating the value of PHC on population health outcomes still is sparse.
- The federal government can influence the adoption of PHC by sponsoring comparative effectiveness and cost-effectiveness research.
- To justify payment, a diagnostic test should be demonstrated to alter the prevention or management of a disease or disorder, or inform behavioral or life planning decisions, and to achieve benefits that could not have occurred otherwise as cost-effectively.
- Data sources and methods for determining value and allocating resources for PHC interventions should account for their use, and health and economic impacts, in practice.
- Value assessment of PHC products should be considered within broader economic and social impacts, as with any new genomic application or other health technology.
- Realization of the value of PHC in screening and primary disease prevention in the Medicare population is subject to the limitation of the Medicare statute.

C. Reducing Health Disparities

- More research is needed to understand the causes of health care disparities and means for preventing or reducing them.
- Development and introduction of PHC provide opportunities to learn about factors that may contribute to disparities and ways to prevent or reduce them.
- Reducing disparities will require representing all populations in biomedical research and related data collection.
- The history of health care disparities, and prevailing factors that continue to contribute to them, may raise barriers to adoption of PHC by affected population groups.

- Although PHC has the potential to reduce health care disparities, it also has the potential to create or widen them if its benefits are inequitably directed or accessible.

D. Educating and Engaging Providers and Consumers

- The potential for realizing large-scale benefits of PHC depends on overcoming misconceptions about the role of genetics in disease, e.g., genetic determinism.
- The success of PHC will depend on translating evidence-based research into appropriate use in routine medical practice. This will require modernizing education of health care providers, including physicians, nurses, pharmacists, genetic counselors and others, regarding the benefits, risks and costs associated with PHC.
- The benefits and risks of validated PHC technologies must be communicated to the patient and the consumer. Patients must remain the focus of PHC.
- Patients need to be assured of protections of privacy and against discrimination based on personal genomic data.
- Providers need to be aware of the diverse sources and great volume of health information encountered by patients and other consumers, and to be prepared to share accurate, useful information about PHC and reduce its misuse and potential harm. The federal government can support efforts to create such information sources and make them readily available.

E. Using Databases to Build Evidence, Inform Decisions

- Realizing the potential of PHC, including to accelerate the discovery, development and delivery of diagnostics and therapeutics, will require linking and analyzing large magnitudes of data on genomics, biomarkers, health care interventions, outcomes and costs.
- Before widespread and integrated use of databases in PHC can occur, standards for their design and use are needed.
- Prospectively-generated databases and related studies are needed to further PHC.
- The federal government could facilitate and support efforts to enhance or develop PHC databases in the public and private sectors.
- The federal government could facilitate and support efforts to design and standardize databases and decision-support platforms for incorporation into health care practice.
- Large government and private sector investment in electronic health records, personal health records and other health information technology will be required to validate, implement and track the impact of PHC.
- The protection of privacy and confidentiality will be essential in the development of databases for PHC purposes.