

The Age of Personalized Medicine

What Is Personalized Medicine?

Personalized medicine is the tailoring of medical treatment to the individual characteristics of each patient. The approach relies on scientific breakthroughs in our understanding of how a person's unique molecular and genetic profile makes them susceptible to certain diseases. This same research is increasing our ability to predict which medical treatments will be safe and effective for each patient, and which ones will not be.

Personalized medicine may be considered an extension of traditional approaches to understanding and treating disease. Equipped with tools that are more precise, physicians can select a therapy or treatment protocol based on a patient's molecular profile that may not only minimize harmful side effects and ensure a more successful outcome, but can also help contain costs compared with a "trial-and-error" approach to disease treatment.

Personalized medicine has the potential to change the way we think about, identify and manage health problems. It is already having an exciting impact on both clinical research and patient care, and this impact will grow as our understanding and technologies improve.

Traditional "One-Size-Fits-All" Approach
All patients with the same diagnosis receive same treatment



Personalized Medicine Approach
Treatment strategy based on patient's unique genetic profile



Genetic Profile A:
Targeted Therapy

Genetic Profile B:
Standard Therapy

Personalized Medicine Is Impacting Patient Care in Many Diseases. For Example...

...in Breast Cancer: One of the earliest and most common examples of personalized medicine came in trastuzumab. About 30% of patients with breast cancer have a form that over-expresses a protein called HER2, which is not responsive to standard therapy. Trastuzumab was approved for patients with HER2 positive tumors in 1998 and further research in 2005 showed that it reduced recurrence by 52% in combination with chemotherapy.¹

...in Melanoma: BRAF is the human gene responsible for the production of a protein called B-Raf, which is involved in sending signals inside cells to direct cell growth, and shown to be mutated in cancers. In 2011, a drug called vemurafenib, a B-Raf protein inhibitor, and the companion BRAF V600E Mutation Test were approved for the treatment of late stage melanoma. Vemurafenib only works in the treatment of patients whose cancer tests positive for the V600E BRAF mutation. Around 60% of patients with melanoma have a BRAF mutation, and approximately 90% of those are the BRAF V600E mutation.²

...in Cardiovascular Disease: Prior to the development of a gene expression profiling test to identify heart transplant recipients' probability of rejecting a transplanted organ, the primary method for managing heart transplant rejection was the invasive technique of endomyocardial biopsy – a heart biopsy. Today, a genetic diagnostic test is performed on a blood sample, providing a non-invasive test to help manage the care of patients post-transplant. New research suggests that ongoing testing may be useful in longer-term patient management by predicting risk of rejection and guiding more tailored immunosuppressive drug regimes.

Personalized Medicine Is...

Personalized medicine is a multi-faceted approach to patient care that not only improves our ability to diagnose and treat disease, but offers the potential to detect disease at an earlier stage, when it is easier to treat effectively. The full implementation of personalized medicine encompasses:



Risk Assessment:

Genetic testing to reveal predisposition to disease



Prevention:

Behavior/Lifestyle/
Treatment intervention
to prevent disease



Detection:

Early detection of disease
at the molecular level



Diagnosis:

Accurate disease diagnosis
enabling individualized
treatment strategy



Treatment:

Improved outcomes
through targeted
treatments and reduced
side effects



Management:

Active monitoring of
treatment response and
disease progression

Who Is Personalized Medicine?

The people and groups engaged in personalized medicine and helping to drive it forward

The realization of personalized medicine relies on the input and contributions of a broad community of stakeholders, all working together toward a shared goal of harnessing breakthroughs in science and technology to improve patient care.

Patients and Consumers

Participating in genetic testing and clinical trials and working with health care providers to proactively manage disease risk and/or treatment strategies

Health Care Providers

Employing an understanding of the patient's genetic profile and utilizing new technologies to individualize the approach to disease prevention, detection, diagnosis, treatment, and management

Biopharmaceutical Companies

Developing targeted therapies and conducting innovative research based upon an understanding of genetic variation and its effects on the safety and effectiveness of the candidate drug

Diagnostic Companies

Developing tools and tests to analyze and interpret genetic information, improving the understanding of disease at the molecular level and a patient's likelihood to respond to drug therapy

Academic Researchers

Conducting basic and clinical research to uncover new insights into human genetics and the molecular basis of disease, enabling greater precision in diagnosis and more targeted drug development

IT/Informatics Companies

Creating electronic tools and resources to collect and store patient health information, making it available to inform clinical decisions and improve safety while protecting patient privacy

Advocacy Groups

Advancing personalized medicine in patient care by educating consumers and providers, accelerating research, and supporting necessary changes in policy and regulation

Payors

Exploring new business models to incentivize the practice of personalized medicine through appropriate reimbursement of molecular diagnostics, targeted therapies, and other personalized treatment protocols

Accelerating the Adoption of Personalized Medicine

As the ecosystem of stakeholders works to advance personalized medicine, collaboration with government regulators and policymakers is necessary to encourage widespread use of these new tools and technologies. The regulatory process must evolve in response to advances that are targeted to smaller patient populations based on genetic profiles, and policies and legislation must be enacted that provide incentives for innovative research and adoption of new technologies. Together, progress in the research, clinical care, and policy enabling personalized medicine has great potential to improve the quality of patient care and to help contain health care costs.



The Age of Personalized Medicine

Personalized medicine is rapidly having an impact on how drugs are discovered and developed; how patients are diagnosed and treated; and how health care delivery is channeling its resources to maximize patient benefits. **The Age of Personalized Medicine** website is dedicated to highlighting the advances being made in the field, the individuals working to enable those advances, and the implications for health and health care policy.

Learn more: www.PersonalizedMedicineCoalition.org and www.AgeOfPersonalizedMedicine.org

Join the discussion: www.AgeOfPersonalizedMedicine.wordpress.com

¹ Personalized Medicine Coalition, "The Case for Personalized Medicine," May 2009, http://www.personalizedmedicinecoalition.org/sites/default/files/TheCaseforPersonalizedMedicine_5_5_09.pdf (Accessed 12 May 2011); Piccart-Gebhart MJ, Procter M, Leyland-Jones B, et al. Trastuzumab after adjuvant chemotherapy in her2-positive breast cancer. *N Eng J Med* 2005; 353:1659-72; Romond EH, Perez EA, Bryant J, et al. Trastuzumab plus adjuvant chemotherapy for operable HER2-positive breast cancer. *N Eng J Med* 2005; 353:1673-84.

² Chapman P, Hauschild A, et al. Improved survival with vemurafenib in melanoma with BRAF V600E mutation. *N Eng J Med* 2011; 364(26):2507-2516.