

Personalized Medicine

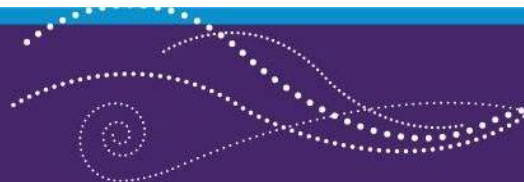
“Personalized medicine” is a vision of medical practice in which the unique medical attributes of patients, especially their genetic make-up — but also key biomarkers, prior treatment history, environmental factors, and behavioral preference — are taken into account to guide treatment planning for the patient. Personalized medicine does not mean creating medicines or medical devices unique to a patient, but rather the consideration of patient characteristics to optimize pharmaceutical treatment and/or overall care. This includes the ability to segment individuals into subpopulations that differ in their susceptibility to a particular disease, their response to a specific treatment, or the nature or origin of their disease. Personalized medicine allows preventive or therapeutic interventions to be focused on those most likely to benefit, sparing expense and side effects for those who are not.¹ To advance the practice of personalized medicine, Pfizer has adopted a “precision medicine” paradigm for biopharmaceutical research and development, to enable the development of therapies capable of delivering more meaningful treatment effects based on a deeper understanding of disease mechanisms and the ability to target therapies to defined patient subpopulations.²

Background

Personalized medicine is fundamentally transforming the way we view disease — from a catalog of clinical symptoms to a category defined by its underlying cause. Over the past century, medical care has generally focused on selecting medicines that are “standard of care” based on clinical signs/symptoms and that are successful in the “average” patient. However, we now know that complex diseases, such as diabetes, cancer, and Alzheimer’s, are usually caused by a combination of genetic and environmental factors, not by a single gene or event. The personalized medicine approach allows physicians to profile genetic variation as a basis for understanding disease drivers in each patient, while considering other factors, such as treatment history and environmental factors, in order to select the medicine or treatment that will potentially ensure a more successful outcome with a more favorable safety profile. The development of tests to guide dose selection, or to help predict which patients are likely to respond to treatment or have high risk for adverse events, have allowed products that treat cardiovascular disease, HIV/AIDS, cancer, multiple sclerosis, and other diseases to be marketed and used more safely. In a growing number of cases, a test is developed together with a medicine in order to support the safe and effective use of the medicine. Such a test is called a companion diagnostic because it provides essential information for determining whether a patient is eligible to receive the associated therapy.³

Guided by knowledge of an individual’s genetic and genomic data, as well as a deeper understanding of disease, personalized medicine can benefit individual patients and the entire health care system in many ways.

- **Get to optimal therapy more quickly.** The use of diagnostic tests can help the health care provider to select a treatment option with the greatest probability of success at the outset, helping to reduce inefficient “trial and error” prescribing.
- **Use drugs more safely.** Screening tests can identify patients who have an elevated risk for an adverse reaction to specific medicines. Also, individuals with uncommon variants of drug metabolizing enzymes and transporters may need a different dose, or may not benefit sufficiently or at all from certain medicines.
- **Increase patient compliance.** The failure of patients to adhere to prescribed treatment plans exacerbates their medical condition and increases medical costs resulting from nontreatment. When a therapy proves more effective or has a more favorable safety profile for a patient, the patient is more likely to adhere to the treatment.
- **Leverage “precision medicine” principles to increase the probability of success in R&D.** Biopharmaceutical research and development focused on narrower, well-defined patient subpopulations has the potential to increase the speed of clinical trials and increase the probability of demonstrating clear clinical benefit.
- **Reduce inefficiencies in health care.** Optimal practice of personalized medicine can help reduce many of the inefficiencies in the current health care system. Awareness of genetic risk factors encourages preventive care and early diagnosis. Much of its value will come from the prevention of advanced disease states, reduction of ineffective treatment, and avoidance of additional care resulting from adverse drug reactions.



Key Facts and Figures

- With advances in medicine and genetics, over 100 drugs have been developed that include genetic (pharmacogenomic) information in their labels.⁴ Personalized medicine will continue to play a leading role in health care, with estimates of a 69 percent increase in the number of therapies developed by the year 2020.⁵
- Cancer remains at the vanguard of personalized medicine. In the past 10 years, cancer patients have seen a four-fold increase in their personalized medicine treatment options.⁶ This past year alone, over half (5 out of 9) of identified personalized medicines approved by the FDA were for cancer indications.⁷
- A recent study by the Mayo Clinic found a 30 percent decrease in hospitalizations when personalized, genetic information was available to doctors prior to prescribing a medicine.
- While there have been growing concerns about the cost of targeted therapies, it has been shown that precision medicines have driven down overall costs and produced health system savings. For example, stratification of women over the age of 50 based on family history and genetic testing has been predicted to reduce the cost of breast cancer treatment by 37 percent with no loss of efficacy, due to a 60 percent drop in incidence of metastatic disease.⁸

Pfizer's Position

Pfizer is committed to the discovery and development of innovative treatments through “precision medicine” approaches. New medicines based on detailed molecular knowledge of disease mechanisms play an increasingly critical role in helping health care professionals provide the right medicine, to the right patient, at the right dose, at the right time. Pfizer supports the development of companion diagnostics when they improve a medicine’s benefit/risk profile. Targeted medicines and companion diagnostics developed through precision medicine approaches can enhance the practice of personalized medicine, improve patient outcomes, and make more efficient use of resources throughout the health care system.

Current regulatory, reimbursement, and health care delivery policies should be amended in order to foster continued innovation for personalized medicine and to unlock the full value of targeted medicines and companion diagnostics. Continued education is needed to help health care providers and patients understand and adopt the latest advances in personalized medicine.

How Patients and Health Care Professionals Benefit

Medical products tailored to specific patient populations can improve the chances that an individual patient will get optimal treatment more quickly and be more likely to achieve a positive medical outcome. Personalized medicine can enhance trust between patients and their treating physicians by increasing confidence in the effectiveness of the treatment approach.

How the Health Care System Benefits

Appropriate use of personalized medicine in clinical practice may make health care systems more efficient in several ways: by optimizing preventive care for high-risk patients, by minimizing ineffective care, by reducing serious adverse drug reactions, and by limiting the need for additional care due to the progression of poorly treated disease.

What It Means for Pfizer

Pfizer is committed to continuous investment in precision medicine to deliver more meaningful treatment effects to defined patient populations, increase the success rate of our research and development, and create a more compelling value proposition for our products.

¹ President’s Council of Advisors on Science and Technology (PCAST) “Priorities for Personalized Medicine,” September 2008.

² National Research Council “Toward Precision Medicine: Building a Knowledge Network for Biomedical Research and a New Taxonomy of Disease,” November 2011.

³ Food and Drug Administration (FDA) “Draft Guidance for Industry and Food and Drug Administration Staff: In Vitro Companion Diagnostic Devices,” August 2014 [according to FDA website].

⁴ Personalized Medicine Coalition. The Case for Personalized Medicine - 4th Edition. 2014. Website: http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc_case_for_personalized_medicine.pdf.

⁵ Tufts Center for the Study of Drug Development, “Impact Report,” Volume 17, No.3, May/June 2015.

⁶ IMS Institute for Healthcare Informatics, “Innovation in Cancer Care and Implications for Health Systems: Global Oncology Trend Report,” May 2014 (accessed September 2015).

⁷ Personalized Medicine Coalition. “More than 20 percent of the Novel New Drugs Approved by FDA’s Center for Drug Evaluation and Research in 2014 are Personalized Medicines” <<http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/2014-fda-approvals-personalized-medicine2.pdf>> Accessed September 2015.

⁸ Van Dyck W et al. *Unlocking the value of personalized healthcare in Europe—breast cancer stratification*. Health Policy and Technology 2012; 1: 63–68.

