

Personalized Medicine

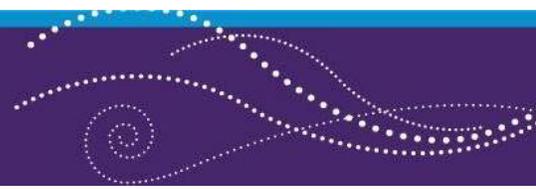
“Personalized medicine” is a form of medical practice in which the unique medical attributes of patients, especially their genetic make-up — but also key biomarkers, prior treatment history, environmental factors, and behavioral preference — are taken into account to more precisely guide treatment planning. Personalized medicine does not mean creating medicines or medical devices unique to a single patient, but rather the consideration of non-obvious 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 R&D, to help enable development of therapies capable of delivering more meaningful benefits based on deeper understanding of disease mechanisms and the ability to target therapies to defined patient populations.²

Background

Since its emergence, personalized medicine has shifted the way we view disease — from a set of clinical symptoms to a condition defined by its underlying cause. Over the past century, medical care has often focused on selecting medicines that are “standard of care” based on clinical signs/symptoms and that are successful in a large group of patients. 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 and other non-obvious patient characteristics as a basis for understanding disease drivers in each patient, in order to select the medicine or treatment that will increase the likelihood of a successful outcome with a more favorable safety profile. “Precision medicine” approaches – involving the use of tests to guide dose selection, or to help predict which patients are likely to respond to drug treatment or have high risk for adverse events – has 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, genomic and other biomarker data, as well as a deeper understanding of disease, personalized medicine can help 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 non-treatment. When a therapy proves effective for a particular group or subset of patients, 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 health care costs.** Optimal practice of personalized medicine can help reduce unnecessary costs 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, efficient use of physician resources and avoidance of additional care resulting from adverse drug reactions.



Key Facts and Figures

- With advances in medicine and genetics, approximately 132 drugs have been developed that include biomarker information on their drug labels.^{4,5}
- More than 25% of medicines approved by the FDA in 2016 were personalized medicines and 42% of medicines in the pipeline have the potential to become personalized medicines.⁶
- 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.⁷ At current, as much as 73% of drugs in development for cancer are personalized medicine products, compared to 42% for all disease areas.⁸
- 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 medicine⁹.
- While there have been growing concerns about the cost of targeted therapies, it has been shown that precision medicines can drive down overall costs and produce health system savings. For example, stratification of women over the age of 50 based on family history and genetic testing has been demonstrated 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.¹⁰

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 continue to be amended in order to foster continued innovation for personalized medicine and to unlock the full value of targeted medicines and companion diagnostics. A key component here is support of FDA implementation of 21st Century Cures provisions related to precision medicine such as adaptive clinical trials and real world evidence, as well as efforts to streamline approval/qualification pathways for biomarkers, diagnostics and targeted therapeutics and patient focused drug development. Continued education is also 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 efficacy 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,” Nov, 2011.

³ 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 (eds. 1-4 2008-14)

⁵ Personalized Medicine Coalition: Applications: Therapies; <http://www.personalizedmedicinecoalition.org/Education/Therapies>; Oct 31, 2016

⁶ Personalized Medicine Coalition. The Personalized Medicine Report, 2017: Opportunity, challenges and the Future

⁷ IMS Institute for Healthcare Informatics, “Innovation in Cancer Care and Implications for Health Systems: Global Oncology Trend Report,” May 2014.

⁸ Tufts Center for the Study of Drug Development: Personalized medicine gains traction but still faces multiple challenges: Impact Report. 2015;17(3)

⁹ Epstein RS, Moyer TP, Aubert RE, et al. Warfarin genotyping reduces hospitalization rates. Results from the MM-WES. *J Am Coll Cardiol.* 2010; 55:2804-12.

¹⁰ Van Dyck W et al. “Unlocking the value of personalized healthcare in Europe—breast cancer stratification”. *Health Policy and Technology* 2012; 1: 63–68.

