Benefits of Personalized Medicine

1. Shifting the emphasis in medicine from reaction to prevention
2. Directing targeted therapy and reducing trial-and-error prescribing
3. Reducing adverse drug reaction
4. Revealing additional targeted uses for medicines and drug candidates
5. Increasing patients adherence to treatment
6. Reducing high-risk invasive testing procedures
7. Helping to control the overall cost of health care
Scientific Advancement - Human Biology and Disease Susceptibility

- DNA: find associations between a given disease and genetic variation
- RNA: analyzing messenger RNA transcripts, the immediate downstream mediator of the genome, can sometimes detect gene expression
- Epigenetics: These “epigenetic” changes can occur in response to environmental factors, and influence whether certain genes are turned “on” or “off.”
- Proteins: Entirely new approaches to protein biomarker detection are promising to make proteomics as “simple” as genetic analysis

Scientific Advancement - Immunotherapy

- Some provide a general boost to the body’s immune system
- Others help train the immune system to attack specific cancer cells (e.g. immune checkpoint inhibitors like Keytruda® and Opdivo® inhibiting a tumor’s ability to use a substance called PD-L1 to bind with its receptor, PD-1, which normally acts as a type of “off-switch” that helps keep a patient’s immune system from attacking cancer cells.)
Scientific Advancement

Gene Therapy
- replace a mutated gene that causes illness, or to introduce a healthy copy of a gene to restore the function of a needed protein.
- Alipogene tiparvovec (Glybera®), and several gene therapies have advanced to phase III trials in the U.S

CRISPR/Cas9 Gene Editing
- has allowed for the development of efficient and reliable ways to make precise changes to the genomes of living cells

Regulatory Policy

- Regulatory oversight of laboratory-developed tests (LDTs): FDA’s non-binding discussion paper published in January 2017.
- Regulatory Oversight of Next-Generation-Sequencing (NGS) Based Diagnostic Tests: NGS technology can examine millions of DNA variants at a time, and therefore require a more flexible oversight approach
- Codevelopment: In 2014, FDA released its final In Vitro Companion Diagnostic Devices Guidance, which helped clarify its method for conducting simultaneous reviews of a drug and its companion diagnostic
Coverage and Payment Policy

- Evidence Requirements: payers look for convincing evidence of their clinical and economic impact, which is difficult unless the products and services in question are covered by insurance policies.
- Value Assessment Frameworks: lack of consideration of the clinical benefit of a drug to certain patients.
- The Changing Reimbursement Landscape for Diagnostics: A number of coding and payment policy changes have led to significant drop in reimbursement for molecular diagnostic tests.
- Value-Based Payment Models: CMS and private payers are also proposing new, “value-based” payment models, which should encourage physicians to tailor care based on an individual’s genetics and other factors.

Clinical Adoption

- Health care providers, payers, employers, and policymakers, as well as patients and their families, need to have a better understanding of personalized medicine concepts and technologies.
- Policies and practices related to patient engagement, privacy, data protections, and other ethical, legal, and societal issues regarding the use of individual molecular information must ensure appropriate consent and be acceptable to patients.
- Best practices must be established for the collection and dissemination of evidence needed to demonstrate clinical utility of personalized medicine and ensure the recognition of its value to care.
Clinical Adoption

- Effective health care delivery infrastructure and data management systems should be developed and applied so that individual patient and clinical support information is comprehensive, useful, and user-friendly, and so that it can be used to guide clinical decisions.

Health Information Technology

- Government Support: Since 2015, hospitals and physicians face penalties for not using health IT.
- Electronic Health Records: more than 80 percent of U.S. physicians using EHRs.
- Mobile Technologies: The ubiquity of mobile information devices such as smart phones as well as advances in sensing technologies and self-management platforms may also provide important tools for personalized medicine.