

POSITION STATEMENT

Advancing Personalized/Precision Medicine

June 2015

Introduction

The scientific and healthcare communities have made significant progress in understanding the physiological, genetic and biochemical composition of the healthy human body, including detailing the human genome. These efforts have improved our ability to precisely define the characteristics of each patient's disease and have been instrumental in translating the concept of personalized, precision medicine into a reality. Increasingly healthcare professionals, with the use of laboratory tests, are able to identify and monitor precisely targeted, individualized therapeutic interventions that may result in the best patient outcome — helping to ensure the right therapy for the patient. Personalized medicine is rapidly becoming an integral component of patient care.

Background

Failed Treatments and Adverse Reactions

Many patients diagnosed with serious medical conditions do not receive effective treatment in a timely manner. This scenario is a byproduct of the traditional “trial and error” healthcare system, wherein clinicians must rely on probabilistic predictions resulting from clinical trial outcomes to determine a treatment plan for individual patients. In this system, physicians prescribe treatments and then wait to see if the individual patient will be among the percentage of patients who respond favorably to the prescribed treatment. Studies have shown that 43 percent of the population does not respond to commonly prescribed drugs for diabetes, 40 percent to drugs for cardiac arrhythmias, and 75 percent to therapeutics for cancer (1). In addition, studies indicate that 5.3 percent of all hospital admissions are associated with adverse drug reactions (2). Mortality from adverse drug reactions is a serious

AACC POSITION:

AACC strongly supports efforts within the healthcare community to personalize patient care and improve individual patient outcomes. Laboratory medicine plays a vital role in enabling the practice of precision medicine, including the identification of drugs or interventions that are best suited for each individual patient. We urge policymakers and payers to adopt policies that ensure access to and appropriate reimbursement for laboratory testing that will facilitate personalized therapeutic decisions.

concern (3). It is critical that the healthcare community move away from a one-size-fits-all approach to patient care by incorporating medicine that tailors therapeutic interventions to biomarker and genetic test results that identify the optimum care decisions for individual patients.

Advances in Care

Although healthcare professionals have always strived to customize treatment plans to meet patient needs, recent technological and scientific advances in laboratory medicine, including the decoding of the human genome, have provided new tools for personalizing medical treatments (4). Clinicians are now able to diagnose and treat medical conditions earlier, even identifying the presence of risk for a disease before clinical symptoms or signs appear and with greater accuracy

(1). In parallel with laboratory efforts, the Food and Drug Administration (FDA) has taken steps to facilitate personalized or precision medicine. The Agency has added pharmacogenomics information

to the product labels of more than 150 drugs, which enables clinicians to target those patients who will respond better to a drug based on their genetic makeup. Similarly, the FDA has developed a new regulatory pathway for companion diagnostics that will further assist healthcare providers to prescribe individual therapeutic interventions based on the alignment of the drug with the individual's laboratory test results that predict successful treatment outcomes (2).

Value of Laboratory Medicine

Laboratory testing is central to all facets of personalized medicine. From the most basic traditional biomarkers to those newly discovered, test results are used to diagnose the patient, predict and monitor drug response, stratify individuals for comparative effectiveness research and provide the informatics data needed for complex predictive algorithms (5). Clinical laboratories support the delivery of companion diagnostics that guide clinicians in making decisions about drug and dose selection for an individual. Laboratory practitioners have a wealth of knowledge and expertise pertaining to the myriad of tests used to personalize medical diagnosis and treatment; this knowledge is critical to assisting clinicians accurately interpret the intricate panels of tests used in precision medicine.

Considerations

Regulation

Test Development and Application

The FDA has been engaged in promoting personalized medicine for more than a decade, conducting stakeholder meetings, publishing reports, providing guidance, and issuing rules to advance the field. The agency has taken an active role in facilitating the availability of companion diagnostics, in which diagnostic tests and therapeutics are developed and reviewed simultaneously. To date, the FDA has approved or cleared 19 companion diagnostic products through the premarket approval or 510(k) process (2). FDA not only regulates the initial availability of these commercial products, but also provides oversight in the post-market environment by way of facilities inspections and medical device reporting regulations.

Testing Oversight

Laboratory testing processes are regulated by the Centers for Medicare and Medicaid Services (CMS) under the Clinical Laboratory Improvement Amendments of 1988 (CLIA'88). CLIA oversight applies when a laboratory uses FDA approved/cleared commercial products; it also applies when a laboratory develops innovative tests to meet medical needs in the absence of appropriate FDA approved/cleared commercial products. These laboratory developed tests, or LDTs, are an integral component in precision/personalized medicine, as they allow the clinical team to tailor innovation to immediate clinical needs. Regulations should promote continued innovation while ensuring patient safety (6).

Reimbursement

Coverage

CMS determines whether adequate evidence exists for covering a test under the Medicare program and sets the amount to be paid for the service. Personalized medicine tests are developed to determine the efficacy of drugs or treatments within narrowly defined subpopulations with low disease prevalence; therefore, evidence for the effectiveness of these tests might not be extensive. CMS has sought to address this issue through its Coverage with Evidence Development program, although the level of evidence required under this initiative may still be unachievable in practical terms. Only when CMS and other insurers adapt their evidentiary requirements for coverage of these types of very low-volume tests will the full potential of personalized/precision medicine be realized (7).

Payment

Medicare and other insurers are adopting payment reforms that encourage health care providers to coordinate patient care across settings. Rather than reimbursing for individual services, insurers are paying a capitated fee to participating organizations and practices. Personalized medicine can assist these healthcare entities by selecting the most appropriate drug therapy for the patient, thereby leading to earlier interventions and improved patient outcomes. Better patient care can also result in a reduction in healthcare costs by eliminating the purchase of ineffective drugs and avoiding any complications that may arise from their inappropriate

use. While payers might encounter higher up-front costs associated with patient-specific testing, they will realize ultimate savings as a result of earlier, more effective care.

Informatics

The adoption of health information technology (HIT) by healthcare providers is vital to the success of personalized medicine. Capturing patient health data through electronic health records (EHRs) provides researchers with valuable information for evaluating health status and the effectiveness of treatment interventions in individual patients. In addition, the use of clinical decision support systems can promote personalized medicine by incorporating a variety of tools such as clinical guidelines and patient-specific recommendations.

Positions

AACC is encouraged by the continued and recently reinvigorated focus on personalized/precision medicine. To ensure the full benefits of this clinical approach can be realized, AACC supports the following positions:

Congress

- Congress should continue to promote the adoption of health information technology by healthcare providers through financial incentives that encourage the use of electronic health records.
- Lawmakers should fund efforts to create health data repositories that can be used by researchers to advance the science of personalized medicine.
- Congress should increase funding for basic and clinical research in the field of personalized medicine tests, including efforts to translate these new tests from laboratories into clinical practice.

Food and Drug Administration

- FDA should expand its efforts to include information in the product labeling for relevant drugs or other pharmaceutical products that urge healthcare providers to test for certain genetic or other biomarkers prior to prescribing the drug or other pharmaceutical product.
- FDA should not adopt policies that hinder test innovation.

CMS and Private Payers

- CMS should expand use of the Coverage with Evidence Development program for promising personalized medicine tests.
- Public and private payers should develop clear and consistent evidence criteria for coverage of companion diagnostics that would account for the relatively smaller affected patient and population subsets. Public and private payers should ensure that personalized medicine testing is appropriately reimbursed.

Laboratory Professionals and Other Medical Stakeholders

- The experts in clinical laboratory science and medicine should continue to work with clinicians to create LDTs that identify the most effective treatments for patients when an FDA-approved companion diagnostic is not available.
- Laboratory professionals should educate clinicians about the availability, advantages and limitations of personalized medicine tests and assist with the interpretation of the test results.
- Laboratory, clinical, and pharmaceutical professionals should partner to author and promote guidelines and standards for the development and implementation of personalized medicine algorithms and practices.

References

1. Food and Drug Administration. "Paving the Way for Personalized Medicine: FDA's Role in a New Era of Medical Product Development." October 2013 <http://www.fda.gov/downloads/ScienceResearch/SpecialTopics/PersonalizedMedicine/UCM372421.pdf> (Accessed April 2, 2015)
2. Personalized Medicine Coalition. "The Case for Personalized Medicine." 2014 4th Edition http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc_case_for_personalized_medicine.pdf (Accessed April 2, 2015)
3. Lazaro J, et al. "Incidence of Adverse Drug Reactions in Hospitalized Patients." JAMA. April 15, 1998; 279:1200-1204 (Accessed April 2, 2015)
4. Managed Care Magazine. "The History and Future of Personalized Medicine." August 2011 <http://www.managedcaremag.com/content/history-and-future-personalized-medicine> (Access April 2, 2015)
5. Valdes, Jr R & Linder M. "Labs are the central providers of patient-specific information that will enable personalized medicine." ADVANCE for Administrations of the Laboratory. July 2011 <http://laboratory-manager.advanceweb.com/features/articles/clinical-laboratory-driving-personalized-medicine.aspx> (Accessed April 2, 2015)
6. AACC Position Statement. "Oversight of Laboratory Developed Tests." November 2014 <https://www.aacc.org/~media/files/position-statementslaboratorydevelopedtestspositionstatement2014.pdf?la=en> (Accessed May 31, 2015)
7. Hresko, Andrew & Haga, Susanne B. "Insurance Coverage Policies for Personalized Medicine." Journal of Personalized Medicine. October 30, 2012, 2, 201-216 <http://www.mdpi.com/2075-4426/2/4/201> (Accessed April 9, 2015)