Three Pressing Challenges for Personalized Medicine

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Personalized medicine can be described as the science of targeted therapies. Advances in diagnostic and molecular medicine have made it possible to more precisely identify alternative treatment options for patients based on their unique genetic or clinical profiles. According to an article published by the FDA in the spring of 2015 (FDA Continues to Lead in Precision Medicine), targeted, personalized therapies have been a priority for the agency since the 1990s when Herceptin (trastuzumab) was approved for treating breast cancer patients expressing high levels of the HER-2 biomarker. The FDA article also notes that 30-targeted therapies were approved since 2012 and in 2014 alone, eight of the 41 novel drugs approved by the Agency were targeted, including:

1. Lynparza (olaparib) for the treatment of advanced ovarian cancer.
2. Blincyto (blinatumomab) for the treatment of B-cell precursor acute lymphoblastic leukemia (ALL).
3. Harvoni (ledipasvir and sofosbuvir) to treat patients with chronic hepatitis C infection.
4. Viekira Pak (ombitasvir, paritaprevir, dasabuvir and ritonavir) for the treatment of chronic hepatitis C infection.
5. Cardelga (eliglustat) for the long-term treatment of Gaucher disease type 1.
7. Zykadia (ceritinib) to treat patients with non-small cell lung cancer (NSCLC).
8. Vimizim (elosulfase alpha) for the treatment of Mucopolysaccharidosis Type IV (Morquio Syndrome).

The increase in Agency-approved therapies is evidence of the technical and clinical progress that has been made in personalized medicine. However, technical and clinical proficiency without supportive policies by insurers and payors can impede patient access to these improved therapies.

The lack of adequate support by payors and in particular Medicaid is addressed in the Personalized Medicine Coalition (“PMC”) in its report – “The Future of Coverage and Payment for Personalized Medicine Diagnostics” (“Report”). In addition to the lack of adequate incentives to support targeted therapies, the Report notes that the current payment model and American’s aging population is a

“stimulus for a longer-term and more progressive approach to decision in medical care. Personalized medicine can control costs and improve outcomes, and the increased attention on the future of Medicare will demand creative approaches to accomplishing these goals.”

Three major challenges were identified by the PMC:
1. Imminent federal pricing of highly innovative molecular tests;
2. Inconsistent standards and paradigms for evaluating diagnostic, prognostic, and predictive genomic tests; and
3. Lack of incentives for genomic medicine.

Imminent Federal Pricing

As noted by the Report, new personalized therapies are efficient therapies that can reduce the cost of health care. If a patient is treated with the best medicine from the start, the use of multiple failed or ineffective therapies, supported by repeated medical support, is avoided. In addition, personalized medicine is not limited to
branded drugs but can also include cost-saving generic medicine. Advanced diagnostics that use liquid tissue samples (such as blood or needle aspiration) for initial diagnosis or monitoring can avoid the use of surgical biopsies that can also reduce costs.

However, the PMC’s Report notes that there have been recent changes in the “coding and pricing” paradigms for genomic tests between 2012 and 2014. As explained by the Report, coding and administrative pricing for laboratory diagnostic tests are handled in a “unique way by the Centers for Medicare & Medicaid Services (CMS)’ Medicare program” and “[r]ecent changes to molecular diagnostic reimbursements in ... 2013 were often rocky, and extended as far as a nearly complete cessation of federal payments for genomic tests in the first quarter of 2013.”

The issue for the industry, as noted by the Report, is that reimbursement for diagnostic tests (that have been unilaterally set in the past by policymakers), must not only “ensure access to high quality tests but also continue to allow for the development of a pipeline of innovative tests that require substantial risk-based research.” Pricing payments too close to the marginal cost of the provider will not allow for innovators to provide the product and recoup the cost to develop it.

**Inconsistent Standards for Genomic Tests**

Current diagnostic tests are to be evaluated for their “clinical utility” a standard that is neither clear nor predictable. As noted by the Report, “[m]ore objective and reliable standards for these evaluation processes need to become broadly accepted. Since many argue that reimbursement should be tied to value, value (utility for the patient) needs to be acceptably defined.”

**Lack of Incentives for Genomic Medicine**

Most approved personalized therapies are related to the diagnosis and treatment of cancer. However, personalized medicine’s impact need not be so limited but as noted by the Report, lack of funding to support the science and implementation (for example, lack of funding to educate genetic counselors and physicians) limits adoption and innovation. The Report indicates that the lack of protection, either by patents or other means, has created uncertainty as to how to attract sufficient “investment capital and research and development funds to permit their commercialization.”

**Marginal Cost is Not Value**

In sum, the PMC in this Report advocates for a “reimbursement system that encourages the improvement of patient care through medical innovation.” The current system determines reimbursement based on the marginal cost of the benchtop chemistry to offer the test. However, it fails to consider the significant research and development costs (which can include regulatory approval or oversight) that were expended by the innovator and the improvement in the quality of, and extension of, life that is the value of personalized medicine. The Report states that the current system fails the public in failing to consider the factors described in the Report that first to market innovators must bear.

**Paying and Supporting Diagnostic Innovation**

Lack of access to the BRCA1/2 diagnostic test was one of the policy reasons advocated by the patient-plaintiffs in the Myriad Genetics patent suit. Some insurers would not pay for the test and patients who wanted it could not afford the out-of-pocket cost. After the US Supreme Court invalidated Myriad Genetics’ patents covering the BRCA1/2 test, other laboratories entered the market with their own tests, and the cost dropped. However, lowering the cost did not translate into universal coverage for the test and access is therefore still limited.

Whether one agrees with Myriad’s business model around its BRCA1/2 genetic test, it is difficult to dispute that Myriad’s patent position allowed it to develop the science and technology that other labs now rely on in offering their tests. Without the ability to protect and develop the technology through patent protection, innovators need to rely on other means to recoup the cost of research and development. Current payment models, as noted by the PMC Report, however, currently fail as viable alternatives for recouping such costs.

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