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Personalized Medicine: Innovation, Threats and Opportunities

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Personalized medicine seeks to individualize health care by identifying the most effective or most comfortable treatment for each individual. According to a report published by the Personalized Medicine Coalition, the use of this technology should increase structural efficiencies in healthcare by identifying an individual's disease risk so that a healthcare strategy can be implemented that focuses on prevention or early intervention rather than a reaction to an advanced stage of disease.[1]

The Promise and Potential

Many of the applications of personalized medicine use an individual's unique genetic code and new methods of molecular analysis. For example, it is now possible to determine if an individual is more likely than not to succumb to a certain disease such as breast cancer or run the risk of an adverse reaction to a therapy or drug. Another current application is the linking of an individual's genetic identity to the most effective cancer therapy. Present technology exists to determine which colon cancer patients are more likely to respond to several available cancer drugs by analyzing a patient's genetic code at a preselected location. As a result, a patient can be matched with the most effective treatment earlier rather than later and without unnecessary and costly trial and error.

In late 2009, PricewaterhouseCoopers estimated the U.S. market for personalized medicine at about $232 billion and projected an annual 11 percent growth rate, nearly doubling in size by 2015 to over $450 billion.[2] The group also reported that the core diagnostic and therapeutic segment of the market presently was around $24 billion with an estimated 10 percent annual growth, reaching $42 billion by 2015.

While the market potential is large, much work remains to be done. A correlation between a genetic variation and a potential disease or successful therapy requires identification of the gene, a method or test to identify the gene, and studies to validate the correlation. The risk of developing a disease such as cancer may be multi-factorial, which further increases the complexity of establishing and validating the correlation between an individual's genetic make-up and predisposition to disease.

Research and development in the pharmaceutical industry has typically relied on strong patent protection and the period of exclusivity a patent provides to recoup the investment in bringing a drug or diagnostic to the public. Strong patent protection for this emerging field is at risk on numerous fronts which can limit investment in this technology just as the benefits are moving from the laboratory to the marketplace.

Challenges in the Courts

The American Civil Liberties Union ("ACLU") and others joined the Association for Medical Pathology in challenging several patents on forms of two human genes linked to breast or ovarian cancer. The patents are owned by Myriad Genetics and the University of Utah Research Foundation. Because of the patents, no competitive, alternative tests are commercially available. This fact and the price of the test, over $3,000 which is not covered by all insurance policies, may have motivated the plaintiffs to seek invalidation of the patents.

The U.S. District Court for the Southern District of New York recently invalidated patent claims relating to descriptions of certain genes by nucleic acid sequence and their use to identify patients who might be at risk for breast or ovarian cancer.[3] Surprisingly, the patent claims were not challenged and invalidated on so called prior art grounds, i.e., for failing to provide a novel and nonobvious invention over known technology. Rather, the plaintiffs argued and the court agreed that the U.S. Patent and Trademark Office should not have granted the patents because information relating to genes and their use should not be patentable *per se*. The court reasoned in part that the challenged claims were unconstitutional and invalid because the subject matter was a product of nature and should not be patented. If the grounds for invalidating the patents were limited to failing to describe an invention over the prior art, the effect of the decision would have been limited to the challenged patents. However, the plaintiffs' successful strategy in challenging the patents on the basis of nonstatutory subject matter has raised questions as to whether or not patents will continue to be granted on information related to genetics. If the district court's decision is sustained on appeal, granted and issued patent claims that are the basis for personalized genetic tests could be invalidated and no further patents of this kind would be granted in the United States.[4]

Challenges in Congress

In February 2007, Representatives Becerra of California and Weldon of Florida introduced in Congress H.R. 977 or the "Genomic Research and Accessibility Act" that sought to prohibit patent claims similar to those challenged in the Southern District of New York. The bill as introduced would amend the U.S. patent law to add a section titled "Prohibition on patent of human genetic material." The proposed language of the bill prohibits the patenting of "a nucleotide sequence, or its functions or correlations, or the naturally occurring products it specifies."

If enacted in its present form the bill would effectively cease the ability to patent any technology directed to genes and gene fragments as well as other inventions based on a nucleotide sequence, such as interfering RNA, plasmids, viral vectors, and vaccines as long as the invention is based on or described by a nucleotide sequence.

It also would preclude the patenting of proteins and antibodies or conceivably any product of a nucleotide sequence such as a stem cell which is a "naturally occurring product it [the nucleotide] specifies." Genetic tests would also be unpatented as they are based on correlations of a nucleotide sequence.

Whether or not such far reaching implications of the bill were intentional or the result of poor drafting is unknown. The bill was cleared from the books since it did not pass during the session of Congress in which it was introduced. However, a member of Congress can reintroduce the bill in a subsequent session.

Patient Protection and Affordable Care Act (H.R. 3590)

Should any one or more of the above challenges in the courts or Congress succeed, the pharmaceutical and medical device industry may need to rely on more than patent exclusivity to support continued research and development. The U.S. government may provide some assistance in the form of the recently enacted "Patient Protection and Affordable Care Act" (H.R. 3590) (the "Act") signed into law on March 23, 2010 by President Obama. While the Act does not directly provide grants to support specific research and development, several provisions of the Act recognize the importance and promise of personalized medicine and promote its prophylactic application to disease prevention and mitigation.

For example, Section 3011 of the Act prioritizes the establishment of a national strategy to improve the delivery of health care services, patient health care outcomes, and population health. The national strategy must, in part, enhance the use of health care data to improve quality and efficiency and address gaps in quality, efficiency, comparative effectiveness information, and health outcome measures and the collection of data.

Section 3013, which amends the Public Health Service Act [5] authorizes the Secretary of Health and Human Services ("Secretary") to give funding priority to the development of quality measures that allow the assessment of the efficiency of care as well as the safety, effectiveness, patient-centeredness, and appropriateness of patient care as part of the national strategy to improve health care quality. "Quality measure" means a standard for measuring the performance and improvement of population health or of health plans, providers of services, and other clinicians in the delivery of health care. In awarding funding (the Act authorizes the appropriation of $75 million for each of fiscal years 2010 through 2014, with amounts appropriated to remain available until expended), the Act requires that the Secretary give priority to the development of quality measures that allow the assessment of several factors. As relevant here, those factors include health outcomes and the functional status of patients, and the experience, quality, and use of information provided about and used to inform decision-making about treatment options.

The most significant recognition of personalized medicine appears in Section 3113 of the Act. This section authorizes the Secretary to conduct a demonstration project to allow separate payment under Medicare Part B, at rates to be determined by the Secretary, for covered complex diagnostic tests (as defined by the Act) that link a patient's genetic makeup to a cancer chemotherapy where no alternative test is available having equivalent performance characteristics, under certain limited

circumstances. While the demonstration project will be limited to tests on patient samples collected during hospitalization but performed after hospitalization, it will ultimately result in a report to Congress with an assessment of the project's impact on access to care, quality of care, health outcomes, and Medicare expenditures, including savings.

Individualized preventive care is supported as well. For example, Section 4103 supports Medicare coverage for an annual "wellness visit," to include a personalized prevention plan for an individual that takes into account the results of a health risk assessment. In addition, the prevention plan should provide personalized health advice aimed at reducing identified risk factors and improving self-management of an individual's health care and treatment. The focus on individualized medical risks is further evidence that Congress may provide support for Medicare to expand coverage for medical care and planning, which can help to avoid or reduce disease, rather than limiting Medicare coverage to the diagnosis or treatment of existing medical conditions.

Studies in comparative effectiveness also are supported. Section 6301 establishes a nonprofit corporation to be known as the Patient-Centered Outcomes Research Institute to assist in the analysis of health outcomes and the clinical effectiveness, risks, and benefits of more medical treatments such as therapies, diagnostic tools, and pharmaceuticals (e.g., drugs and biologics). The research funded under this section must take into account, as appropriate, the potential for differences in the effectiveness of health care treatments in various subpopulations; for example, individuals with different genetic and molecular sub-types. Results of the studies are to be published in a format that is comprehensible to patients and providers, with safeguards to protect patient privacy and confidentiality of study subjects.

Conclusion

Although it is difficult to imagine that patent protection for therapies and diagnostics that service the growing field of personalized medicine would no longer be permitted either by an act of Congress or a judicial ruling, the aforementioned challenges do create uncertainty. The parties in *Ass'n for Molecular Pathology* will likely appeal the decision all the way to the U.S. Supreme Court. Ultimate resolution is likely years away.

If patent protection is lost or significantly curtailed, the public is likely to ultimately lose, even if the U.S. government can step in and provide incentives to develop products and therapies in this area. In order to obtain a patent, an applicant must disclose the invention in the patent document in a manner that places the invention in the hands of the public upon patent expiration. If companies are prohibited from protecting and financing inventions through patenting, trade secret protection for inventions becomes one of the few alternative options to protect research investment, and a relatively unattractive one at best. Accordingly, the technology and science supporting personalized medicine would not be disseminated to the public forcing many to "re-invent the wheel" rather than advancing science and technological progress to the benefit of the public.

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[1] The Case for Personalized Medicine, available at http://personalizedmedicinecoalition.org/communications/TheCaseforPersonalizedMedicine_11_13.pdf.

[2] The new science of personalized medicine, PriceWaterhouseCoopers, available at http://www.pwc.com.

[3] *Ass'n for Molecular Pathology v. U.S. Patent and Trademark Office*, No. 09-CV-04515, 2010 BL 112357 (S.D.N.Y. Apr. 2, 2010).

[4] For a general discussion of other pending lawsuits that seek to limit patent protection on inventions in this technology, See Tu, S. et al. A Perfect Storm Is Brewing Against Personalized Medicine. Bloomberg Law Reports - Intellectual Property, Vol. 4, No. 8 (Feb. 22, 2010).

[5] 42 U.S.C. § 299 et seq.