Legal Genome

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hange and uncertainty accompany any political transition, and as the new administration was taking shape back in the early part of the year, FDA-regulated stakeholders had many questions and concerns about what regulatory changes might be in store for them in the next few years. While drug pricing rhetoric and the brief floating of a couple unconventional names as potential FDA Commissioners caught industry’s attention early on, with the confirmation of Dr. Gottlieb as FDA Commissioner and political attention focused on Congressional efforts to repeal the Affordable Care Act, the FDA has been a relatively quiet haven from the usual Washington politics thus far. In fact, one of the key policy priorities being pursued at FDA is the incentivization of innovation, and that goal seems to have broad political support (at least as an overarching aspirational goal). And of all areas of FDA’s vast regulatory jurisdiction, Precision Medicine is perhaps the most logical and well-positioned to benefit from policies to promote innovation.

We have, in fact, seen several concrete milestones in FDA’s oversight of Precision Medicine that are noteworthy and encouraging. In May, FDA approved expanded labeling for Keytruda (pembrolizumab), for the treatment of patients whose cancer has a specific genetic biomarker, regardless of the location of the tumor in the body. Now, the drug is approved for any unresectable or metastatic solid tumor identified as having microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) biomarkers. This is the first cancer drug approved based solely on the genetic characteristics of the tumor and not on the tumor’s location. Also in May, FDA approved expanded labeling for Kalydeco (ivacaftor), to include treatment of cystic fibrosis patients with one of 23 additional rare genetic mutations, raising the total number of labeled mutations to 33, up from 10 previously.

As CDER Director Dr. Janet Woodcock has noted, these advances are the result of, and dependent on, the development of high-quality substantiated biomarkers by industry, elaborating that “the ability to identify useful biomarkers depends on how well scientists understand the disease they are seeking to treat. In some areas, such as cancer and infectious diseases, we have made real progress in understanding how these diseases develop and how to treat them with drug therapy. FDA continues to encourage drug developers to use strategies based on biomarkers.”

However, FDA and other arms of the federal government have been taking a more proactive and controlling role in what has traditionally been primarily the private sector domain of drug research and development. In the case of biomarker development, FDA has established a Biomarker Qualification Program “to develop biomarkers that aid in the drug development process.” While that too sounds like a laudable effort, government intervention and assistance now comes with a price.

As Dr. Woodcock has also pointed out, “once qualified, these biomarkers may be used in the specified manner by any drug sponsor.” In other words, FDA is willing to work closely with industry to facilitate development of biomarker innovations that the agency will then be willing to bless in the context of product development and approvals, but such regulatory collaboration arguably requires innovators to forego opportunities for intellectual property and related regulatory process protections that the traditional models afford.

To date there are only 6 submissions listed under FDA’s Qualified Biomarker Program web page, so it may be too early to tell if this will in fact be an engine of innovation in Precision Medicine. It is also too early to tell whether the general trajectory of innovation policy will bend more toward private incentives, or innovation through governmental partnerships and interventions. The ability of FDA to wield both carrots and sticks in pursuit of innovation is worthy of attention, respect, and caution as Precision Medicine advances further into critically important yet uncharted medical — and regulatory — territories.

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