

# Editorial Board Member's Commentary: Commissioner Scott Gottlieb's Predictable Imaginarium

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Dr Scott Gottlieb's still-nascent tenure as US Food and Drug Administration (FDA) Commissioner has, to date, been defined by two terms not generally associated with the agency that regulates more than a quarter of the US economy—predictability and imagination.

Let's start with predictability. Generally speaking, regulators love ambiguity. It gives them almost limitless boundaries. But there are significant unintended consequences, not the least of which is the resultant lack of confidence by innovators to invest in new and more complicated development programs writ large, and specifically those for non-biologic complex drugs and biologics. Minus a brightly lit regulatory pathway, the costs associated with these products will not come down anytime soon. The Gottlieb FDA has made bringing first-to-market generics and biosimilars a priority. Bravo. When people ask how the FDA can impact drug costs, this issue is at the top of the list, and it's due in no small part to Dr Gottlieb's understanding that a predictable FDA process facilitates speedier marketplace competition.

"Imagination" and "Regulatory Policy" are not often used in the same sentence. But, the winds of change are blowing. Dr Gottlieb recognizes that the agency must advance its relationships with the industries it regulates. That doesn't mean fewer in-depth reviews or strident actions. Rather, it means that the FDA is doing a better job being a first-among-equals intramural partner. From patient-focused drug development, to biomarker vetting, responsible off-label communications, new approaches to pain management, and the regulatory science required for validating real world evidence, Gottlieb is steadily moving the agency away from its traditional angst in "not invented here" regulatory conversations. He's not only imagining an FDA that is both regulator of and colleague with industry, he's working to make it a reality.

Speaking to the Senate Appropriations Committee, Gottlieb announced that the FDA is launching a Medical Innovation Access Plan designed to facilitate biomedical innovation.<sup>1</sup> He positioned the plan as a tool to lower health care costs by reducing expenditures on costly diseases, noting that "ultimately, the most tangible way we're going to reduce healthcare costs is by finding new and better treatments for vexing diseases like diabetes and cancer and neurodegenerative ailments like Alzheimer's."<sup>1</sup>

He also suggested that the initiative could lead to lower drug prices by reducing regulatory uncertainty and eliminating unnecessary regulatory costs. According to an article in *BioCentury*, he believes that drugs "are priced to some measure of the cost of the capital—including the investment capital—that's required to discover and develop them. And the risk and time and cost of the regulatory process are a big part of that equation."<sup>2</sup> His plan will include a "broad range of steps we'll take to make sure that our own regulatory tools and policies are modern and risk based—and designed to facilitate the development of potentially breakthrough new treatments."<sup>2</sup> One focus of the plan will be targeted drugs for rare diseases or disorders for which there is currently no effective therapy. Within 6 months, FDA will issue new guidance on the clinical evaluation of targeted therapies for rare disease subsets.

Per the Commissioner, "This new policy will address the issue of targeted drugs, and how we simplify the development of drugs targeted to rare disorders that are driven by genetic variations, and where diseases all have a similar genetic fingerprint, even if they have a slightly different clinical expressions."<sup>2</sup> The guidance will clarify circumstances in which FDA may approve a cancer drug based on its molecular mechanism of action rather than the specific tissue or organ where tumors occur. It will also help sponsors develop drugs for rare subsets grouped by laboratory testing, so they can be studied in a single clinical trial.

Gottlieb also committed FDA to clearing its backlog of 200 pending Orphan Drug designation requests. He said that the agency would respond to all future requests for the designation within 90 days of the request. As part of the plan, FDA will update guidance documents on clinical trial enrichment strategies and adaptive trial designs. The agency will also take a fresh look at policies to support the qualification of biomarkers

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and the use of pharmacogenomics in early phases of clinical trials.

To help address immediate concerns about drug prices, Gottlieb has committed FDA to identify off-patent drugs that have little or no generic competition to highlight market opportunities, and to put Abbreviated New Drug Applications for generic versions of these drugs at the front of review queues. In addition, one of his first public initiatives will seek to identify and close legal loopholes that allow branded manufacturers to stymie generic competition. Problems he has cited include the use of risk evaluation and mitigation strategies (REMS) to prevent generic and biosimilar companies from obtaining product samples, as well as refusal to share REMS programs that restrict distribution or access.

Gottlieb also understands that regulatory transparency cannot be a *for-thee-but-not-for-me* proposition. Per the Commissioner, “We should be making sure that we try to provide as much information back into the market of ideas as possible. There are places across this agency where we bottle up too much information.”<sup>2</sup> He singled out complete response letters as a “place where we should ask hard questions because there’s some very important information in those communications.” Complete response letters indicate why FDA has decided that an application is not ready for approval at a specific time, and often they specify the kinds of data needed to gain approval in the future. Releasing this information could advance public health, for example, by publicizing a safety threat, or by helping other companies avoid known pitfalls and thereby get drugs onto the market more quickly.

“There might be other products on the market or other products in development that are affected by the agency’s

judgment,” Gottlieb said. “If there are ways to redact those letters from commercial competition information, to make that sort of bottom-line important information available, I think that we should look at that.”<sup>2</sup>

Gottlieb’s intimate knowledge of the health industry psyche, viewed by some as his greatest flaw, is actually a unique and important asset. He recognizes that predictability is power in pursuit of the public health and, in imagining an FDA that can (indeed must!) color outside the lines, pays heed to Bertrand Russell’s maxim that “science may set limits to knowledge, but should not set limits to imagination.”<sup>3</sup>

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