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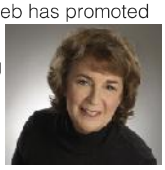
Gottlieb Seeks More Efficient Clinical Research Strategies to Lower Drug Costs

May 01, 2018 By Jill Wechsler
Applied Clinical Trials
Volume 27, Issue 5

In his first year leading the FDA, Commissioner Scott Gottlieb has promoted multiple initiatives to streamline drug development and clinical research as part of his campaign to moderate drug prices and achieve "a good balance between innovation and access," he told *Applied Clinical Trials* magazine in a recent interview. The commissioner has blasted brand manufacturer "shenanigans" for blocking generic drug approvals, while also supporting the development of more effective pain medicines and addiction treatments to help combat the lethal opioid epidemic plaguing the nation.


Jill Wechsler

In tackling contentious issues, Gottlieb has quieted critics on all sides. Democrats initially feared an industry bias, but have been impressed by his efforts to advance public health and challenge drug prices. Republicans hoping for a free-market deregulator support his campaign against opioid abuse and efforts to speed innovative drugs, devices, and diagnostics to patients.

A clear sign of achievement for Gottlieb is the $400 million boost in FDA's budget plan for 2019, a notable shift from earlier administration proposals to sharply cut agency appropriations. To convince the legislators to approve the requested funds, FDA has outlined how the added resources will advance biomedical innovation (see bit.ly/2JXjcum). A prime initiative is to develop data and analytical tools to better utilize real-world evidence in accelerating medical product development. An expanded "knowledge management system" will evaluate new drugs more rapidly and consistently. Additional funds will support FDA's Oncology Center of Excellence and advance new treatments for rare diseases.

FDA also faces numerous deadlines for implementing the 21st Century Cures legislation, including provisions to support regenerative medicine and to speed the development of new cancer treatments, personalized medicines, and gene therapies. At the other end of the spectrum is a proposal for new user fees to support more efficient oversight and approval of over-the-counter drugs.

More guidance

FDA will be hard pressed in the coming months to realize last year's gains in drug development and approvals, while also tackling a number of hot issues, such as nicotine levels in cigarettes, oversight of independent testing labs, and food contamination outbreaks. A main strategy for the commissioner is to take some of the innovative research approaches devised for the oncology

setting and implement them across additional new drug review divisions, Gottlieb told ACT.

This involves developing guidance documents on strategies such as designing master protocols and shorter advisories on applying different clinical trial approaches to additional disease settings. In February, for example, FDA issued several guidances on addressing serious, complex neurological conditions, such as Alzheimer's disease (see bit.ly/2qKfzjJ). Gottlieb also unveiled in March an updated Benefit-Risk Assessment plan for incorporating patient perspectives into regulatory decision-making (see bit.ly/2HE9sHr). More recently, FDA published draft guidance on including pregnant women in clinical trials (see bit.ly/2J8dKnd) and an update on international standards for pediatric drug development (see bit.ly/2JarpdA). A final guidance clarifies FDA's process for sponsors to gain FDA agreement on development programs under the special protocol assessment (SPA) process (see bit.ly/2HLrUvh). And the agency proposed in April a model-informed drug development (MIDD) pilot program, with a series of workshops, to encourage sponsor use of exposure-based, biological and statistical models in drug development programs (see bit.ly/2qLado4).

Gottlieb anticipates that more streamlined clinical research and disease-specific guidance, along with initiatives to speed more generic drugs and biosimilars to market, should translate into lower drug costs. While the commissioner acknowledges that new drugs are priced at what the market will bear, he believes that more predictable R&D pathways can help "de-risk" drug development, which would reduce the cost of capital and permit a lower price to justify initial R&D investment. Such efficiencies may be even more important in bringing a second or third branded product to market, which Gottlieb considers important for achieving a good balance between innovation and access.

A related intriguing issue for Gottlieb is how current policies and practices encourage global "free riding" on U.S. biopharmaceutical R&D. New FDA data indicates that other industrial nations pay more for generic drugs than in the U.S., and less for innovator therapies—"but not a lot less" when adjusted for net price, he points out. The payments should be reversed, Gottlieb says, as the current situation is a "recipe for destroying innovation."

Gottlieb's concerns about the high cost of medicines reflect his own experience as a physician and seeing ill patients "struggling very hard at the worst moments in their lives" to try to afford drugs that are "absolutely indicated for their disease." He wants to be sure "that in my time here at FDA, I do something to address that."

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