The 21st Century Cures Act: A gift to the US pharmaceutical industry

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The 21st Century Cures Act is an early Christmas gift from the US Congress to the pharmaceutical and medical device industries. The bill guts the regulatory powers of the Food and Drug Administration (FDA), weakening the standards used to judge the safety and efficacy of drugs and medical devices. Notably, the bill does nothing to address skyrocketing drug prices.

As part of its public relations cover for the corporate giveaway, Congress included in the bill some limited funding for biomedical research and other health initiatives. This funding, however, is not mandatory and will be subject to the annual appropriations battles over discretionary spending. It is likely that these funds will never materialize.

Crafted over the course of two years with the input of more than 1,400 lobbyists, the Cures Act received overwhelming bipartisan support. The House voted 392 to 96 in favor of the nearly 1,000-page bill, and last week the Senate passed the bill in a vote of 94 to 5. President Obama says he looks forward to signing the bill when it reaches his desk.

The widely publicized portion of the bill contains $6.3 billion in funding for biomedical research and health initiatives. The FDA will receive $500 million through 2026 to pay for the provisions in the act, but nothing to address the agency's other longstanding problems. The bill also grants states $1 billion in funding to combat the opioid epidemic, and support for mental health initiatives.

The National Institutes of Health (NIH), which has seen its budget decline 22 percent since 2003, will receive $4.8 billion (an earlier House version called for $8.75 billion), but spread out in equal portions over 10 years. This includes the $1.8 billion reserved for the "cancer moonshot" being pushed by Vice President Joe Biden, and another $1.6 billion earmarked for research on brain diseases such as Alzheimer's.

"While the bill authorizes $4.8 billion to the NIH over the next 10 years—on average, a mere $480 million a year—this is barely a quarter per year of what the House passed last year," Representative Rosa DeLauro of Connecticut, the senior Democrat on the Appropriations subcommittee on health and human services, told the *New York Times.*

"There is also no guarantee that the appropriators will follow through and provide funding each year," said DeLauro.

"When American voters say Congress is owned by big companies, this bill is exactly what they are talking about," Democratic Senator Elizabeth Warren said in a speech from the floor last week.

"Why bother with a fig leaf in the Cures bill? Why pretend to give any money to NIH or opioids? Because this funding is political cover for huge giveaways to giant drug companies," said Warren.

Moreover, the Cures Act actually cuts $3.5 billion from the Prevention and Public Health Fund established under the Affordable Care Act (ACA), according to *The Hill*. The fund was established to promote the prevention of Alzheimer's disease, hospital-acquired infections and chronic illnesses such as cancer, heart disease and diabetes.

"Cutting the Prevention Fund will limit the nation's ability to improve health and quality of life and prevent disease," Rich Hamburg, interim president and CEO of The Trust for America's Health, told NBC News. "This is the nation's first and only substantial investment in moving from our current 'sick care' system to a true preventive health system."

The Cures Act represents a significant rollback of the regulatory authority of the FDA and the guidelines used to approve new drugs for the past half century.

The modern system of drug regulation was established by the 1962 Kefauver-Harris Amendments to the Federal Food, Drug, and Cosmetic Act of 1938 in response to the thalidomide tragedy. Thalidomide was approved for sale in Europe as an over-the-counter treatment for morning sickness in pregnant women in the late 1950s, but resulted in thousands of babies being born with crippling and deadly birth defects.

Famously, FDA reviewer Francis Oldham Kelsey withheld approval of the drug, insisting that it first be fully tested. Her decision minimized the fallout from the tragedy in the US.

Previously, a drug would be automatically approved if the FDA failed to act on an application within a certain time period. The Kefauver-Harris Amendments transformed the FDA into an actual gatekeeper. Drugs could no longer be marketed unless both their safety and efficacy had first been demonstrated through a series of carefully designed clinical trials. Standards were issued for the manufacture, packaging and labeling of drugs, along with a system for reporting adverse events.

Congress has made a number of modifications to the modern system since then—such as creating an expedited approval process, and requiring that pharmaceutical companies fund agency drug reviews—but the Cures Act marks a qualitative development.

Instead of relying primarily on randomized clinical trials, the

gold standard for determining drug safety and efficacy, the Act requires the FDA to consider "real world evidence," such as anecdotal observations of patient outcomes.

"A homeopath would love this provision, and I'm sure, so would drug companies," oncologist David Gorski commented in 2015 on the Science-Based Medicine web site. "Why bother with the time, bother, and expense of those pesky clinical trials to get your drug approved for additional indications, when you can rely on clinical experiences based on therapeutic use, uncontrolled observational studies, or registries instead?"

The bill creates a new expedited pathway for "regenerative medicine" products that rely on surrogate or intermediate endpoints, instead of clinical endpoints such as patient survival. Expedited approvals require a rigorous collection of post-approval data to confirm the safety and efficacy of the drug, but a report released last year by the Government Accountability Office (GAO) found that the FDA was not fulfilling its post-market oversight obligations.

The Cures Act allows pharmaceutical companies to promote off-label uses for drugs (uses not indicated by the FDA-approved label) to insurance providers. In recent years, the pharmaceutical industry has faced major fines for off-label promotion, and it has sought to roll back restrictions on promotion of off-label uses by suing the FDA, claiming that such restrictions violate free speech. The new rule will discourage companies from funding clinical trials for new indications of a drug.

The limited population pathway provision of the Act pressures the FDA to approve new antibiotics, antifungals and possibly other drugs based on smaller clinical trials, which will be less likely to detect safety risks or establish efficacy.

The bill reauthorizes and expands priority review voucher programs. Intended to promote the development of drugs that treat rare or neglected diseases, the FDA has nonetheless awarded priority vouchers, which can be sold to other companies for large sums of money, for treatments already widely used. Furthermore, there is no provision guaranteeing that these treatments will be made available or affordable.

The Cures Act also weakens the already lax regulations governing medical devices. It requires FDA employees to only ask for the minimum possible amount of information when approving new medical devices, eases the FDA's authority to regulate combination drug/device products, and provides an overly broad category of "breakthrough" devices.

The watchdog group Public Citizen has detailed these and other problematic provisions contained in the Cures Act.

"These provisions would unravel the FDA, turning it from the treatment watchdog it is today into a puppet of the pharmaceutical and medical device industry," said Johns Hopkins medical doctors Reshma Ramachandran and Zackary Berger in an opinion piece for *STAT News* earlier this month .

"If the 21st Century Cures Act is passed as written, clinicians could be given potentially deadly drugs and devices to prescribe to their patients, blessed by this new version of FDA approval," they wrote.

The Cures Act was one of the most heavily lobbied bills proposed by the 114th Congress, with over 1,455 lobbyists representing 400 companies and other organizations, according to Kaiser Health News, which analyzed lobbying data compiled by the Center for Responsive Politics. This includes 78 pharmaceutical companies, 24 device companies and 26 biotech companies, which reported more than $192 million in lobbying expenses on the Cures Act and other legislative priorities.

The Pharmaceutical Researchers and Manufacturers of America (PhRMA), the trade association representing the drug industry, spent $24.7 million of its overall $30.3 million in spending on the bill.

The Cures Act was also supported by a number of patient advocacy groups. While claiming to give voice to patients, these groups are often heavily tied to the pharmaceutical industry.

A study published this month by the Project on Government Oversight found that at least 93 percent (39 out of 42) of the "patient advocacy groups" included in stakeholder discussions with the FDA in late 2015 and early 2016 received funding from the pharmaceutical industry. More than a third of these organizations (15), had executives, directors or other personnel from the pharmaceutical or biotech industry on their governing boards.

The premise behind the bill—that the FDA holds up and delays the approval of potentially life-saving drugs—has no basis in reality. Last year the FDA approved 45 novel drugs, the highest number since the record-setting 53 approvals in 1996.

"The emphasis has been on getting drugs and devices on the market quickly, not on making sure that they are safe," Dr. Rita Redberg, a cardiologist at the University of California San Francisco Medical Center and critic of the Cures Act, told *Health News Review* last year.

An analysis by *Forbes* this past August found that this year the FDA has rejected only 11 percent of the new uses for new molecular entities it has reviewed (3 out of a total of 28). In 2008, the rejection rate stood at 66 percent.

"The evidence is that we're living in a golden age of drug approvals, at least from a drug company's perspective," writes *Forbes* reporter Matthew Herper.

Groups such as the ultra-conservative Goldwater Institute and Manhattan Institute have long been pushing for "reforms" to the FDA, a cause that was taken up by Michigan Republican Fred Upton, chairman of the House Energy and Commerce Committee. Upton was the major sponsor of the act and in the course of the past two election cycles received $536,650 in campaign donations from pharmaceutical and health products groups, according to data from the Center for Responsive Politics.