

Experts confident of Congressional funding for US Cures Act

The landmark 21st Century Cures Act should receive continued financial support from US Congress, say experts. Susan Jaffe, *The Lancet's* Washington correspondent, reports.

Just 5 weeks before his presidency ended, Barack Obama signed the 21st Century Cures Act, a law that will sustain several of his signature biomedical research initiatives, streamline the US drug and medical device approval process, improve the nation's mental health-care system, and combat the country's opioid misuse epidemic.

The legislation won nearly unanimous approval in both houses of Congress, a bipartisan consensus that might be sufficient to protect it from the looming political storms as Washington lawmakers begin to upend Obama's Affordable Care Act (ACA). Opponents of that effort, which fulfils a long-standing Republican campaign pledge, fear it could push new medical discoveries beyond the reach of patients who desperately need them.

The 21st Century Cures Act will provide US\$6.3 billion over the next 10 years, including \$4.8 billion for the National Institutes of Health (NIH) to support Obama's Cancer Moonshot project exploring new cancer treatments, the Precision Medicine Initiative investigating how drugs can be genetically tailored to patients, the BRAIN Initiative developing new tools to understand the human brain, and a regenerative medicine programme using adult stem cells.

Another \$500 million is earmarked for the Food and Drug Administration (FDA) to hire more staff scientists at competitive salaries, and help get pharmaceutical products to market sooner. The Cures Act also provides \$1 billion for Americans seeking drug addiction treatment. About half of the funding for the law comes from the ACA's Prevention and Public Health Fund, with most of the remaining support coming from the sale of oil from the nation's strategic petroleum reserve.

Obama praised the law at a White House signing ceremony in December, and then paused to remember his mother, who died of cancer. "She was two and a half years younger than I am today when she passed away", said Obama, who is 55 years old. "And so it's not always easy to remember, but being able to honour those we've lost in this way and to know that we may be able to prevent other families from feeling that same loss, that makes it a good day", he said.

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But the funding to implement the law is not guaranteed. Congress will have to vote each year whether to allow the money to be spent, a prospect that doesn't worry Representative Fred Upton, a Michigan Republican who last year headed the House of Representatives Committee on Energy and Commerce, which produced the legislation. "It's a huge win for everybody", and the overwhelming approval should provide the momentum to sustain support in the coming years, he said.

Upton and the committee's then-senior Democrat Diana DeGette of Colorado worked for nearly 3 years to build that consensus. The committee held eight hearings and two dozen round-table discussions around the country to hear from the biomedical research, patient, and health-care communities, including FDA and NIH officials, and industry groups.

In addition to strong bipartisan support, DeGette cited two more reasons why she is confident that funding is reliable. The law says the money must be spent on the targeted programmes and cannot

be applied towards other projects, and it is exempt from federal budget spending limits.

"This looks pretty solid", said Francis Collins, NIH director. NIH has already received the first year of Cures Act funding—about \$352 million—which has enabled the National Cancer Institute to issue almost ten grant opportunities for researchers, Collins said, with about eight more expected. These grants would not have been possible without the Cures Act, he said. (As *The Lancet* went to press, Collins was scheduled to leave his post when Donald Trump becomes president on Jan 20. Collins said he would stay on, if asked.)

The law also makes some other changes at NIH to assist biomedical researchers. It gives them more freedom to attend scientific meetings, creates a contest to encourage new research, and raises the limit on an educational loan repayment programme, among other reforms. Helping with school loans could attract "more people into research who otherwise couldn't see how to make it work financially, and that's particularly going to be important for under-represented groups", Collins said.



Barack Obama signs the 21st Century Cures Act into law, Dec 13, 2016

He also pointed out that the law provides benefits that go beyond the specific research areas receiving special funding. "Numerous economic analyses have indicated the return on investment of dollars that NIH gives out in grants is at least twofold...in the local community where the grant is awarded and that's all 50 states whose economies are growing as a consequence of these investments", he said.

Accelerating drug approval

The Cures Act "tackles one of the most critical issues—the fact that it takes more than 10 years and \$2 billion to get a drug to market, and seeks to cut the red tape without lowering the bar, the gold standard for safety", said Janet Marchibroda, director of the health innovation initiative at the Bipartisan Policy Center, a Washington, DC, think tank.

The law enables the FDA to hire more staff at competitive salaries (addressing an estimated 700 vacancies), promotes patients' input into FDA drug reviews, improves clinical trial designs, and protects patients' health data.

But some critics argue that certain provisions could eventually weaken the FDA's drug reviews. For example, the agency will be allowed to assess the value of "real world evidence" such as observational data or medical insurance claims when considering drugs seeking approval for new indications other than their original approved use. "That kind of evidence is much lower than the gold standard of randomised controlled clinical trials", said Michael Carome, director of Public Citizen's Health Research Group, a consumer advocacy group. Although the law does not require the FDA to rely on these data, he said the law provides "a stepping stone in that direction".

Upton said that two former FDA commissioners, Margaret Hamburg and Andrew von Eschenbach, reviewed the legislation "page by page and they told us what we missed". Their recommendations were incorporated

to ensure that the safety and efficacy standards were not eroded.

The Pharmaceutical Research and Manufacturers of America, which represents many of the leading US brand name drug makers, praised the law, saying it will "enhance the competitive market for biopharmaceuticals and drive greater efficiency in drug development".

If brand name prescription drugs reach the market sooner, their generic versions will follow more quickly, which can increase competition and reduce prices, said Michael Brzica, senior director of federal affairs at the Generic Pharmaceutical Association, a trade association for manufacturers of generic drugs.

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But the few Democrats who voted against the law said Congress missed an opportunity to address sky-rocketing drug prices. Massachusetts Senator Elizabeth Warren said the law contained "special giveaways and favours" for drug companies and only "a tiny fig leaf of funding for NIH...and most of the money won't really be there unless future Congresses pass future bills in future years to spend those dollars".

Mental health-care reform

As the bill headed toward final approval in the last days of the legislative term, congressional negotiators folded into it a package of mental health reforms and measures addressing the opioid misuse crisis. "The focus was on services for people with serious mental health illness, who are commonly associated with high rates of emergency room utilisation, hospitalisation, homelessness, and criminal justice involvement", said Ron Honberg, senior policy adviser at the National Alliance on Mental Illness, a nationwide advocacy organisation

for people with mental illness and their families.

The law creates a new assistant secretary for mental health in the Department of Health and Human Services that Honberg says will "elevate the visibility and priority of mental health and substance abuse services" within the federal government. It also establishes several grant programmes to help states and municipalities provide community-based mental health treatment, as well as crisis response, including tracking the availability of beds in psychiatric hospitals. A new federal committee will help housing, health, education, and other agencies coordinate services for people with serious mental illness. And a new policy group for mental health and substance use disorders will help translate mental health research into practice, "which is always a challenge", said Honberg.

Other provisions aim to set up alternatives to prison for people with mental illness, fund special mental health courts and re-entry services after incarceration, and train police to respond effectively to people in crisis by de-escalating a confrontation.

But Honberg and other patients' advocates worry that these positive steps could be undermined by the repeal of the ACA, and particularly the loss of its Medicaid expansion providing health insurance for millions of low-income people. "The provisions in the Cures Act are quite significant but if you erode the underlying system of care for people, you are going to have more people experiencing crises", he said and limited resources won't be able to keep pace with the increasing need.

"The whole point of this bill and this investment is to benefit real patients", said Ellen Sigal, the chair and founder of Friends of Cancer Research, an advocacy group. "If people don't have access and they don't have the benefit of this investment, it would not only be sad, it would be tragic."

Susan Jaffe