

This is the final installment of the Pharmacy Cost Trends and System Impacts fact sheet series. Prior installments have explored pharmacy cost trends, drug supply chains, and specialty drugs, and this fact sheet discusses the 21st Century Cures Act (Act) and some of its major policy implications. Lauded as the most comprehensive piece of federal healthcare legislation since the passage of the Patient Protection and Affordable Care Act in 2010, the Act incorporates a wide range of policy objectives including modifications to the Food and Drug Administration (FDA) drug approval process, research and funding opportunities for the National Institutes of Health (NIH), and measures to address the country's escalating opioid crisis. While the Act passed with bipartisan support, some have underscored the Act's shortcomings in addressing high drug costs, patient safety concerns, and unease about annual NIH funding reauthorization. This fact sheet summarizes major components of the Act while also addressing areas of concern as implementation of its major provisions moves forward.

BACKGROUND

The 21st Century Cures Act (Act) was the result of almost two years of deliberation within Congress. Initial legislation was introduced in the House of Representatives in January 2015. The final bill was signed into law on December 13, 2016.¹ Totalling over 900 pages, the Act includes provisions that will have significant impact on health policy at both the federal and state levels. Although the Act's expansive language includes notable provisions intended to enhance electronic health record interoperability and family member and caregiver access to health information to inform treatment options, the following sections focus on three major areas impacted by the Act—changes to the FDA drug approval process, NIH funding for research and new initiatives, and substance abuse and mental health reforms.

FDA DRUG APPROVAL CHANGES

The Food and Drug Administration (FDA) is the federal agency responsible for the review and approval of new drugs and devices. Historically, the FDA's approval process has included multiple review phases before market entry, including a discovery and screening phase, multiple clinical trials, drug application review, and ongoing post-marketing assessment once a drug becomes available to the public.^{1,2}

Sections of the Act will affect the standards previously required for drug and device approval. While these changes are intended to expedite how soon certain products can reach the market to aid consumers in need of alternative therapies, there are concerns about the impact of these provisions on consumer safety and the regulatory authority of the agency itself. Four of the provisions are summarized below.¹

- Section 3022 allows the FDA to consider “real-world evidence” in place of evidence derived from randomized clinical trials, historically considered the gold standard for evidenced-based review. This modification requires the Secretary of Health and Human Services (HHS) to determine whether real-world evidence could support drug approval for a new clinical indication.
- Section 3011 creates additional pathways to potentially shorten time in drug development and decrease drug failure rates. This section allows use of biomarkers and patient-reported outcomes, even though these may not be reflective of the specific clinical outcome of interest.
- Section 3042 allows for faster approval of antimicrobial drugs. Expedited approval is intended for limited populations if a drug has been demonstrated to treat a serious or life-threatening infection, even if it has not been tested within that particular population. Interpretation of the groups qualifying as “limited populations” has raised concerns about patient safety and increased proliferation of antibiotic use.
- Section 3014 reauthorizes and expands the scope of the “Priority Review Voucher” program. These vouchers were originally intended to provide additional incentives to manufacturers to develop drugs for neglected diseases by expediting their review by the FDA.

RESEARCH AND FUNDING

The Act also contains provisions related to funding and other support for the National Institutes of Health (NIH), the country's medical research agency housed within HHS. Under the Act, the NIH is projected to receive approximately \$4.8 billion to fund targeted disease programs, including the newly-formed Precision Medicine Initiative and the Cancer "Moonshot" Program (summarized below in Table 2).^{3,4} However, the recent budget proposal released by the Trump Administration calls for a reduction in NIH funding of \$5.8 billion in 2018, along with a \$1.2 billion cut for the 2017 fiscal year.⁵ In late April 2017, Congress allocated an additional \$2 billion to the NIH over the next five months under a bipartisan spending agreement.^{6,7}

Table 2: Summary of Newly-Funded NIH Programs

Precision Medicine Initiative (PMI)	<ul style="list-style-type: none">• PMI's objectives are to utilize advances in genomics and availability of large datasets and to leverage health information technology to advance biomedical discoveries• Establishes the "All of Us" Research Program, which aims to provide research for a range of diseases (both common and rare)• Provides funding to establish a cohort of one million or more volunteers to advance precision medicine
Cancer "Moonshot" Program	<ul style="list-style-type: none">• Enables the National Cancer Institute to begin implementing cancer research objectives outlined in the Cancer Moonshot Blue Ribbon Panel report, such as development of a network for direct involvement of patients and a national cancer data ecosystem• Authorizes \$1.8 billion in funding over 7 years for funding of program, with \$300 million available for use in fiscal year 2017

The \$4.8 billion allocated to the NIH is less than the amount requested in previous versions of the legislation, with the original legislation containing a request for approximately \$10 billion. Additionally, the amount is less than the 2 percent average annual increase that the agency has received since 2004.^{8,9}

SUBSTANCE ABUSE AND MENTAL HEALTH REFORMS

The nation's opioid epidemic has also prompted inclusion of funding within the Act to address the ongoing crisis. One billion dollars will be made available to states through HHS for treatment, prevention, and prescription drug monitoring programs.^{10,11}

Provisions in the Act impacting mental health include the following:¹⁰

- Establishment of an Assistant Secretary for Mental Health and Substance Abuse and a Chief Medical Officer with "real-world experience" providing mental health or substance abuse treatment within the Substance Abuse and Mental Health Administration (SAMHSA)
- Development of a strategic plan every four years to determine agency priorities, specifically relating to improvements and retention within the mental health workforce
- Formation of the National Mental Health and Substance Use Policy Laboratory for state innovations in mental health service delivery
- Reauthorization of the Substance Abuse Prevention and Treatment Block Grant for states
- Establishment of demonstration program within the Health Resources and Services Administration to develop five-year grants to medical students who practice addiction medicine in underserved areas

In April 2017, HHS Secretary Tom Price, M.D. announced the first round of funding provided through the State Targeted Response to the Opioid Crisis Grants, totaling \$485 million.¹² Arkansas received \$3.9 million during the first funding round, which will be used to expand statewide availability of Medication-Assisted Treatment and a comprehensive needs assessment to identify high-risk communities, along with other activities.¹³

CONCLUSION

The 21st Century Cures Act is the result of over two years of Congressional debate. The Act's FDA drug approval provisions have been met with mixed reception, with supporters emphasizing the need for expedited review and critics raising concerns about proper vetting before market entry. Whether the regulatory flexibility granted by the Act addresses what the public views as the main problem with the drug industry—escalating price increases—will need continued monitoring. The implementation of new research programs created by the Act and funding allocated to the NIH will be key and could be impacted by the new administration's funding priorities. Finally, although the new administration has demonstrated a commitment to funding for states to target responses to the opioid crisis, states must continue to commit their own resources, adopt tested responses from other states, and develop state-specific policies for prevention and treatment.

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