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21st Century Cures: The future of product innovation and approval

by

After almost three years of public meetings, debate, and consensus building, the 21st Century Cures Act (Cures Act) became law on December 13, 2016. The bipartisan legislation allocates a total of $6.3 billion to advance biomedical innovation by funding basic science research at the National Institutes of Health (NIH) ($4.8 billion) and allowing for innovation and flexibility for product regulation at the Food and Drug Administration (FDA) ($500 million).

Other provisions target health information technology (HIT) and public health priorities. The major Cures Act provisions that pertain to biopharmaceutical and medical technology product development, regulation, and approval include:

- Striving for faster drug approvals using new classes of evidence and adaptive frameworks. Streamlines clinical trials using new drug development tools and frameworks; requires FDA to evaluate the use of real-world evidence (RWE) and ensure that patient experience is reflected in assessments of benefit/risk
- Communicating health care economic information among stakeholders. Expands the dialogue and provides flexibility on the economic evidence that biopharma and health care stakeholders can share
- Advancing medical device innovation. Adds clarity and updates to existing regulations; introduces regulatory flexibility for advancing devices that treat life-threatening conditions or small populations
- Creating regulatory clarity for combination products. Provides clarity on assigning regulatory oversight to products that involve a combination of drugs, biologics, and devices
- Regulating medical software and HIT. Provides some boundaries on the types of software that will be excluded from FDA regulation; and advances interoperability of electronic health

records (EHRs)

- Establishing a pathway for regenerative medicine. Updates the regulatory pathway for regenerative medicine products, bringing more products under FDA oversight and providing flexibility for more complex products

The Cures Act creates an opportunity for the FDA to apply recent advances in technology and analytics and scientific and evidentiary models to continue evolving regulatory programs. Moreover, the new administration's focus on reducing regulatory burden could spur agencies to move towards a more collaborative, adaptive approach to regulating therapies, and create regulatory flexibility that also supports patient access and public safety.

In many ways, the drug, device, and diagnostic development and approval process of yesterday is over. Life sciences companies (biopharma, medical device, and diagnostics companies) may risk being out of date and competitively disadvantaged if they are not pursuing the newer breakthrough, priority, or accelerated pathways included in the Cures Act and in some of the initiatives the FDA has developed in the past several years. As the industry strives to meet the evolving needs of stakeholders—patients, providers, and health plans— this regulatory flexibility will likely be imperative to drive both regulatory approval and market access.

To take advantage of the evolving regulatory landscape, our research and discussions with industry stakeholders suggest that life sciences companies should consider:

- Engaging in early discussions with the FDA to design clinical trials that incorporate surrogate endpoints and other tools to shorten drug development timelines
- Expanding capabilities to access, collect, and analyze RWE and patient experience data
- Continuing to work with the FDA, patient advocacy groups, and provider organizations to delineate pathways for patient and caregiver involvement
- Expanding the dialogue on economic evidence between biopharma and medical device companies and health care stakeholders, including payers
- Taking advantage of additional regulatory clarity by investing in breakthrough devices, point-of-care (POC) diagnostics, drug-device combination products, and regenerative medicine
- Advancing the conversation on the regulation of medical software in collaboration with the FDA and other industry stakeholders

The Cures Act builds on previous FDA initiatives to modernize the regulatory process

In an effort to modernize the regulatory process, the Cures Act builds on FDA initiatives already

underway, including activities such as expedited review programs; working with life sciences companies to design efficient, flexible clinical trials; and helping speed development of potential treatments for rare diseases (see sidebar below). The Cures Act calls for collaboration among government agencies such as the FDA, NIH, the Department of Health and Human Services (HHS), the Office of Management and Budget (OMB), and the Reagan Udall Foundation to innovate processes and advance therapies.

The FDA has worked to implement six main expedited development and approval pathways

1. Orphan Drug Act (1983) Diseases affecting <200,000 people per year
2. Fast Track (1988) Potential to address unmet medical need; one Phase 2 trial sufficient
3. Accelerated Approval (1992) “Meaningful advantage” over existing therapy; approval based on surrogate or intermediate endpoint “reasonably likely to predict clinical benefit”
4. Priority Review (1992) “Significantly improve” safety or effectiveness; shorter FDA review (six months vs. 10 month standard)
5. Breakthrough Therapy (2012) Preliminary clinical evidence with clinically significant endpoint(s); "substantial improvement” over existing therapy; benefits include intensive guidance to expedite development
6. The Food and Drug Administration Safety and Innovation Act (FDASIA) (2012) Expands the FDA’s authorities and strengthens the agency’s abilities to safeguard and advance public health in a number of ways, including collecting user fees, promoting innovation, increasing stakeholder involvement in FDA processes, and enhancing the safety of the drug supply chain

The Cures Act allocates $6.3 billion in funding for the NIH and FDA over the next 10 years, beginning in fiscal year 2017. The Act establishes the FDA Innovation Account and provides the agency with $500 million to implement the initiatives laid out for faster drug approvals and updated guidance. The Act also creates the NIH Innovation Account and appropriates $4.8 billion for:

- Cancer research ($1.8 billion)
- Brain research ($1.5 billion)
- Precision medicine ($1.4 billion)
- Regenerative medicine ($30 million)

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Advancing federally-funded research

The Cures Act encourages the HHS Secretary to develop a network of scientists and public-private partnerships to come up with new approaches for addressing scientific, medical, public health, and regulatory science issues. The Act includes provisions that encourage translational medicine through greater data sharing among NIH-funded research and industry, and expands the scope of research that NIH can support. Further, it formalizes several key initiatives—such as the Cancer Moonshot and the Brain Research through Advancing Innovative Neurotechnologies® (BRAIN) initiative—by providing dedicated funding for each area. The Cures Act also contains provisions specifically aimed at advancing the Precision Medicine initiative.

Among questions for life sciences companies: How should they invest their R&D dollars across platforms, capabilities, and programs to keep up with scientific advances?

Many health care stakeholders have voiced concerns around funding commitments, as the administration's blueprint budget released in March 2017 proposed $5.8 billion in NIH funding cuts for fiscal year 2018. Many in the scientific community have warned that such cuts would blunt progress in improving the nation's health. The dollars that the Cures Act authorizes for NIH research initiatives represent an increase from NIH's current budget, after years of decline. Many had hoped that the funding included in Cures would, among other things, provide talented scientists and students with more opportunities in biomedical research and discovery in the US. However, Congress will still need to appropriate NIH funding every year and some stakeholders have expressed concern that competing priorities may slow the funding stream that the Cures Act provides.

Among questions for life sciences companies: How should they invest their research and development (R&D) dollars across platforms, capabilities, and programs to keep up with scientific advances? How can they better collaborate and share data with NIH, the broader research community, and patient advocacy groups to advance the scientific understanding of disease? Biopharma and medical device companies increasingly are partnering with other health care stakeholders to address scientific and technological challenges, create greater efficiencies in R&D, and accelerate the development and delivery of new treatments. The Cures Act encourages more of these collaborations and creates an opportunity to expand the nature of these relationships.

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