Inciting Innovation In Drug And Medical Device Development

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Law360, New York (December 14, 2016, 1:58 PM EST) -- On Dec. 13, 2016, President Barack Obama signed into law the 21st Century Cures Act, one of the most important pieces of health care and life sciences legislation in several decades. The act is intended to spur the rapid discovery, development and delivery of innovative drugs and devices to treat a broad range of diseases, and will impact research institutions, pharmaceutical companies, device manufacturers, hospitals and health systems. For life sciences companies, the act:

- Directs the U.S. Food and Drug Administration to consider a broader range of real-world evidence when approving new indications for a drug;
- Grants accelerated approval for regenerative therapies and certain antibiotics;
- Permits broader sharing of health care economic information (HCEI);
- Establishes a "breakthrough" approval pathway for cutting-edge medical devices; and
- Exempts certain software or apps from regulation as devices.

The act touches many other areas of the health care economy as well. It strengthens privacy protections for participants in clinical trials; directs the National Institute of Health to streamline regulations for funding recipients; offers further incentive for providers to make health information technology and electronic records accessible; amends certain provisions of the Medicare Part A and B statutes; dedicates new resources to mental health issues; and allocates billions of dollars for medical research, as well as for treatment for opioid addiction.

As is often the case, implementation of the act will depend heavily on yet-to-be-issued FDA and U.S. Department of Health and Human Services regulations. The act vests agencies with broad discretion over whether and how to decrease regulatory burdens and speed innovation. Health care, pharmaceutical and medical device companies will want to carefully follow the implementation of this sweeping legislation.

Key Takeaways

- The act simplifies the process to gain premarket approval for many drugs and devices. For drugs, the act permits the use of "real-world" data and data summaries to approve new indications for drugs already in use; allows certain antibiotics and antifungals to be approved after limited clinical trials; and generally permits more flexibility in the clinical trial process.
- For devices, the act creates an expedited review process for "breakthrough" technologies; accelerates the approval of certain regenerative therapies; and exempts certain tracking and reporting software from regulation as a medical device.
- The act allows HCEI to be shared not only with formulary committees, but also with payors and other expert entities that select drugs for coverage or reimbursement. This information conveys the economic consequences of using a drug and may be based on sources other than on-label clinical trials.
- The act promises $4.3 billion dollars over 10 years for cancer research, precision medicine and brain research, as well as $1 billion for the treatment of opioid addiction.
- The act directs new resources toward the research and treatment of mental health issues, including evidence-based therapies.

Spurring Innovation in Pharmaceuticals and Medical Devices

One of the act's main goals is to facilitate the swift development of new drugs and medical devices, particularly those that successfully treat debilitating or life-threatening conditions.

Drug Development Tools

The act permits the HHS secretary to approve certain "drug development tools" for use in clinical trials and the new drug approval process. The additional tools are intended to shorten drug development time and reduce the failure rate for drugs in development. The act contemplates broader use of biomarkers, surrogate endpoints and patient-reported outcomes in clinical trials. Rather than relying on morbidity rates to test the safety and efficacy of a drug, developers might rely on some "surrogate" or biological indication that the drug works — for example, whether a tumor shrank or a patient's T-cell count increased.

To facilitate the development of drugs for rare or life-threatening diseases, the act also allows sponsors of genetically targeted or variant protein targeted drugs to rely on data from previously approved applications to demonstrate safety and efficacy. The FDA will now, in addition, have the authority to approve antibacterial and antifungal drugs based on a limited trial population if the drug treats a life-threatening infection.

Use of Patient Experience Data

The act also provides for the use of "patient experience data" — that is, information concerning the qualitative impact of a disease and related therapies on patients' lives. Patient experience data would also encapsulate patient preferences with respect to disease

treatment. The act envisions this data playing a role in the new drug approval process, although the secretary has discretion to determine what that role might be. When the FDA approves a drug, it will be required to comment on the patient experience data it used, if any.

Flexible Trial Design

The act seeks to inject more flexibility into the clinical trial design process. For example, the act requires the FDA to consider using real-world evidence to support the approval of a new use for a drug that has already been cleared for marketing, or to study drugs post-approval. Real-world evidence is considered to be evidence that comes from sources other than randomized clinical trials, such as data from insurance claims or observational trials. The act also requires the FDA to issue guidance addressing the use of novel, complex and adaptive trial designs in the development and approval of drugs.

Improving Patient Access to Therapies

In addition, the act seeks to expedite patient access to drugs that have already been proven safe, certain regenerative therapies and vaccines. If a drug has been approved for use, the FDA may now rely on data summaries, as opposed to full data sets, to authorize the drug for other indications. For example, if a drug already is approved for the treatment of anxiety, it may now be simpler to clear it for the treatment of insomnia. The act also creates a path for the accelerated approval of regenerative therapeutic products, such as cell therapy treatments or products used to regenerate damaged tissue. In addition, the act urges the Advisory Committee on Immunization Practices to consider the use of a new vaccine immediately after the vaccine is licensed.

The act also simplifies the approval process for some medical devices. The act allows FDA to expedite the approval of "breakthrough" technologies that either effectively treat life-threatening or debilitating diseases, or that represent a significant improvement over the status quo. A request for designation as a "breakthrough" technology can be made before a premarket approval application, premarket notification or petition for classification is filed. Additionally, the act clarifies that software used for hospital administrative support, to encourage a healthy lifestyle, for patient records, or to display or summarize test results will not be regulated as a medical device.

Expanded Safe Harbor for Health Care Economic Information

The act broadens a safe harbor that protects drug manufacturers from allegations of misbranding when they share certain HCEI. HCEI consists of data that demonstrates the economic impact of taking a drug. That information may include clinical data, inputs or assumptions, and it may be based on separate or aggregated clinical consequences. Currently, the safe harbor protects drug manufacturers who share information with formularies if that information "directly relates" to an on-label use and is based on competent and reliable scientific evidence.

The act expands the safe harbor to cover the disclosure of information to payors and other experts who select drugs for coverage or reimbursement. The information need only "relate" to an on-label use; however, if the information relates only to an off-label use, then sharing it with payors or formularies is still prohibited. In addition, the HCEI must be accompanied by a "conspicuous and prominent statement describing any material differences" between the HCEI and a product's label. The information must still be based on competent and reliable scientific evidence.

Reducing Administrative Burden for Researchers

In addition, the act aims to reduce the administrative burden on medical researchers.

Under the act, the HHS secretary must take steps to reduce the burdens of conflict-of-interest regulations, laboratory animal regulations and requirements for documentation of personnel expenses from federal funding recipients. The act also establishes a Research Policy Board that must review all regulations governing medical research and recommend policies to reduce the overall administrative burden imposed on researchers by the federal government.

Enhanced Privacy Protection for Human Research Subjects

The act includes enhanced privacy protections for participants in medical research or clinical trials. The act mandates that the HHS secretary offer researchers who receive funding from the agency a "certificate of confidentiality" to protect the privacy of individuals who are the subjects of their research. The act also permits the secretary to issue certificates of confidentiality to privately funded researchers. The certificates require researchers to keep all personally identifiable information and biospecimens confidential, absent an exception specified in the act. It also immunizes personal information and biological materials from being subject to legal process, absent the consent of the research participant.

Delivery of Medical Care

The act contains provisions designed to improve the delivery of medical care to patients. In particular, the act requires the secretary to ease the electronic documentation burden on doctors, so they can spend a higher percentage of their time on patient care. The act also supports the interoperability of health care information systems, which will better facilitate the secure exchange of health care information between providers. The act calls for a voluntary framework and model agreement for the secure transfer of electronic documentation. Moreover, the act supports the development of patient-centered electronic health records that will provide better patient access to understandable, up-to-date health information.

New Civil Monetary Penalties for False or Fraudulent Claims

The act allows HHS to impose new civil monetary penalties for false claims. If HHS determines that a party has presented or caused to be presented a false or fraudulent claim for payment under a grant or contract, that party can be subject to steep monetary penalties as well as liability for up to three times the amount of the claim. These penalties are separate and apart from those that may be sought under the False Claims Act.

Funding for Cancer, Precision Therapy, Brain Research and Addiction Treatment

The act provides billions of dollars in funding for research and treatment. It provides NIH with $4.8 billion in funding over 10 years for cancer research; precision treatments for diseases that vary based on genes, lifestyle or environment; and research into diseases that affect the brain. The act also provides $1 billion over two years for grants to states for the prevention and treatment of opioid addiction and abuse. Those grants can be used to better monitor prescription drugs, otherwise prevent addiction, train medical practitioners or provide access to treatment services for those suffering from opioid addiction.

Other provisions of the act aim to improve the treatment of mental health issues. For example, the act creates a new assistant secretary for mental health and substance abuse to coordinate mental health programs across the federal government. The act also contains provisions encouraging the development and deployment of evidence-based mental health treatments.

The act created some controversy based on the source of its funding. Around $3.5 billion in

funding will be rescinded from the Prevention and Public Health Fund, which was created by the Affordable Care Act to finance community and clinical prevention initiatives. $464 million will be taken from Affordable Care Act funds that had been allocated to U.S. territories. Additional funding will be provided by the sale of a portion of the U.S. strategic petroleum reserves. Moreover, while the act authorizes massive new spending and creates commensurate cuts in other programs, much of the money spent will still need to be appropriated each year through future legislation.

Conclusion

The 21st Century Cures Act is breathtaking in scope and promises to affect researchers, pharmaceutical companies, device manufacturers and health care providers in profound ways.

For researchers, the act provides ample new funding and may streamline onerous regulations. For drug companies and medical device manufacturers, the act will offer expedited approval pathways for new drug indications, regenerative therapies and disruptive technologies. For health care providers, the act aims to make electronic records simpler and more user-friendly. While the legislation holds great promise to spur innovation in the treatment of debilitating and life-threatening medical conditions, much remains in the air, as implementation of the new law and the appropriation of actual funding will be in the hands of the incoming administration and Congress, respectively.

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