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RxLegal
21st Century Cures Act

Michael Gabay, PharmD, JD, BCPS[*]

On December 13, 2016, President Barack Obama signed into law the 21st Century Cures Act.[1] Broadly, this $6.3 billion law provides funding for accelerating cancer research through the so-called Cancer Moonshot, increasing the understanding of the human brain through the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) initiative, and extending precision medicine to all diseases through the Precision Medicine Initiative (PMI).[1,2] Funding is also provided for combating mental health illnesses and to aid states in battling the national opioid epidemic. Plus, the Act allocates substantial support to the US Food and Drug Administration (FDA) in order to streamline the process for drug and medical device approvals, promote increased use of electronic health records, eliminate bureaucratic red tape, and advance the implementation of telehealth services.[1-4] A variety of organizations, companies, and groups expended hundreds of millions of dollars to lobby Congress and advance their own interests prior to passage of the Act.[5] These interests included pharmaceutical and medical device companies, medical schools, hospitals and doctors, mental health and substance abuse treatment advocates, health information technology and software companies, and consumer and patient safety groups.

Despite generally favorable views regarding the enactment of this landmark bill into law, concerns have been raised about certain aspects of the legislation. One topic of discussion has been changes regarding the types of clinical data that pharmaceutical and device manufacturers must provide to the FDA in order to gain initial approval or additional indications.[6] Specifically, the Act discusses the use of biomarkers, surrogate measures, patient experience information, and observational data from routine clinical use or "real world evidence" to facilitate more rapid drug and device approval. Although the use of biomarkers and surrogate measures is not a novel approach when conducting clinical drug studies, overreliance may mislead clinicians and potentially expose patients to unsafe or ineffective treatments. Additionally, the scientific merit of the submitted clinical data is not a mandatory, but rather voluntary, component that is one of many factors considered during review of a drug or device submission per the Act. The language within the Act states that the Secretary of Health and Human Services "shall determine whether to accept a qualification submission based on factors which **may** [emphasis added] include the scientific merit" of the submission.[1,6] Although this language may expedite the FDA approval process, some clinicians worry that this approach could result in approvals based upon lower quality data.

What is meant by the phrase "real world evidence" is also a subject of continuing debate.[6,7] Researchers often define real world evidence as health care–related information that is derived from various sources including electronic health records, claims and billing data, registries, and health applications.[7] The 21st Century Cures Act defines the phrase broadly and includes "any data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than randomized clinical trials."[1,6] This broad approach to the definition, and its inclusion in the Act, may result in FDA reviewers feeling the necessity to lean more heavily on this type of data when considering new approval submissions rather than objective endpoints from properly designed clinical trials.

Another potentially concerning issue is that additional wording in the Act allows for the approval of new indications for existing medications to be based only on "data summaries" submitted by manufacturers.[6] Currently, the FDA assesses all of the submitted data for new drug approvals and supplemental indications. This process is time intensive; however, it may be necessary as unfortunately some manufacturers have been found to overestimate the benefits and underestimate the risks of their medication when summarizing their own clinical data in the past.

[*]Director, Drug Information Group and Prior Authorization Services, Clinical Associate Professor, Department of Pharmacy Practice, University of Illinois at Chicago College of Pharmacy, Chicago, Illinois

In summary, the 21st Century Cures Act is a landmark piece of legislation that enjoyed broad bipartisan support in Congress. The main goals of the Act are impactful and should transform future cancer, neurologic, and precision medicine research as well as aid individuals with mental health illnesses and opioid dependence. However, some of the wording within the Act regarding the drug and device approval process may bring pause to health care providers including pharmacists. Although this wording is intended to facilitate the prompt approval of new agents and devices, clinicians should be aware of the types of data behind an approval and take this into consideration when developing and implementing care plans and counseling patients.

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