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Memorandum

President Obama Signs the 21st Century Cures Act, Accelerating Review of Drugs and Medical Devices

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Yesterday afternoon, President Obama signed the 21st Century Cures Act into law. The Act, passed by overwhelming bi-partisan margins in both the House and Senate contains provisions on diverse topics, including the Beau Biden Cancer Moonshot, funding for mental health and substance abuse programs, and reauthorizing the National Institutes of Health (NIH). This memo does not attempt an exhaustive summary of the legislation. Instead, it highlights certain provisions related to accelerated Food and Drug Administration (FDA) review, changes to clinical trial design, expanded access for patients to drugs and devices, and increased data sharing among researchers.

Changes to the FDA Review Process

The far-reaching legislation targets procedural improvements to the FDA's approval process for certain medical devices and drugs:

- **Least Burdensome Means Approach.** Under Section 3058 of the Act, the FDA will use the "least burdensome appropriate means" approach in requesting additional information in connection with premarket applications for Class III medical devices. The FDA will consider the minimum required information supporting a determination that an application provides reasonable assurance of safety and effectiveness of a device. This is not intended to change the threshold for approval; rather, it aims to optimize efficiencies in the approval process.
- **Priority Review for Breakthrough Devices.** The Act provides a fast-track to approval for certain medical devices. Under Section 3051, a sponsor of a device may request that it be designated a "breakthrough device," for priority review. The FDA is tasked with applying efficient and flexible approaches to expedite the development and review of breakthrough devices. The FDA will assign the device sponsor a team to provide timely and interactive communication with the sponsor during the

development and review process. The FDA may also agree with the sponsor in writing on certain clinical protocols and an early data development plan to ensure that design of clinical trials is as efficient and flexible as practicable. The intent is to expedite the availability of new device technologies without compromising the integrity of the approval process.

- **Facilitating Drug Development for Rare Diseases.** The Act reauthorizes the priority review voucher program to encourage treatments for rare pediatric diseases under Section 3013. Under Section 3012, the Act aims to facilitate the development, review and approval of genetically targeted drugs and variant protein targeted drugs for treatment of rare diseases by allowing sponsors to rely on data and information submitted in previously approved applications that incorporate or utilize the same or similar genetically targeted technology, or the same variant protein targeted drug. Under Section 3033, the Act also provides for accelerated approval of certain regenerative advanced therapies, defined to include cell therapy, therapeutic tissue engineering products, human cell and tissue products and combinations.
- **Easing Regulatory Burdens for Medical Devices.** The Act eases regulatory burdens for Class I and Class II medical devices and requires the FDA to allow for expedited recognition of standards established by nationally or internationally recognized standard organizations. According to Section 3053 of the Act, the FDA must determine whether or not to recognize standards within 60 days of a recognition request. The approach is intended to make it easier for device manufacturers to incorporate established standards in product development. Additionally, under Section 3054, the FDA will identify certain types of Class I and Class II medical devices that will no longer require the submission of a Section 510(k) report.
- **Certain Medical Software is Exempted from FDA Regulation.** Under Section 3060 of the Act, certain medical software is excluded from the term "device" as used by the FDA, thereby exempting the software from regulation. The following software is exempted from FDA regulation based on its low level of risk to patients: administrative support software (*e.g.*, appointment schedules); wellness software (*e.g.*, apps for tracking exercise); electronic patient records; software for transferring, storing, or displaying medical device data such as lab data; and clinical decisions support software.

Changes to Clinical Trial Design and Evidence Development

The legislation implements changes to the requirements for clinical trial design and evidence development. This changes are intended to speed the approval process for certain products by allowing increased flexibility in clinical trial design.

- **Qualification of Drug Development Tools.** Under Section 3011, the FDA will establish a process for the qualification of drug development tools. This aims to accelerate the FDA approval process by endorsing tools used in drug development before researchers begin clinical trials. This section identifies biomarkers and surrogate endpoints as potential tools. The Act notes that surrogate endpoints could be used to support the accelerated approval of a drug.

- **Improved Clinical Trial Designs.** Under Section 3021, the Department of Health and Human Services will assist sponsors in incorporating complex adaptive and other novel trial designs into clinical protocols and the regulatory approval process for drugs and biological products.
- **Possible Expansion of Types of Evidence Considered in Approval Process.** Sections 3001 and 3002 allow the FDA to expand the type of evidence used in the regulatory approval process to include patient experience data. The Act requires the FDA to provide guidance on how patient experience data, including the assessment of desired benefits and tolerable risks associated with new treatments, will be incorporated into the regulatory decision-making process.

Expanded Access for Patients

Under Section 3032, the legislation requires manufacturers and distributors of investigational drugs for serious conditions to publish their policies on expanded access (also known as compassionate use). This is intended to provide greater patient access to potentially lifesaving drugs outside of an ongoing clinical trial. The Act also allows the FDA to exempt certain medical devices intended to benefit fewer than 8,000 individuals from effectiveness requirements, under Section 3052, expediting patients' access to these devices.

Increases in Data Sharing and Collaboration

The Act aims to increase the amount and ease of data sharing among researchers, to promote advancement of new medical research and discovery. Under Section 2011, the Act encourages the establishment of the Precisions Medicine Initiative to augment efforts to address disease prevention, diagnosis, and treatment. The Initiative proposes to create a network of scientists and develop new approaches for addressing public health issues. Section 2063 aims to expand researcher's remote access to health information by streamlining authorization for use and disclosure of health information. Additionally, Section 2014 states that the NIH may require recipients of NIH awards to share scientific data generated from award-supported research with other researchers to promote advancement in the field, while continuing to protect the privacy of human research subjects under Section 2012.

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