U.S. HOUSE OF REPRESENTATIVES
COMMITTEE ON ENERGY AND COMMERCE

November 28, 2017

TO: Members, Subcommittee on Health

FROM: Committee Majority Staff

RE: Hearing entitled "Implementing the 21st Century Cures Act: An Update from FDA and NIH"

I. INTRODUCTION

The Subcommittee on Health will hold a hearing on Thursday, November 30, 2017, at 10:00 a.m. in 2123 Rayburn House Office Building. The hearing is entitled "Implementing the 21st Century Cures Act: An Update from FDA and NIH."

II. WITNESSES

- Francis S. Collins, MD, PhD, Director, National Institutes of Health; and
- Scott Gottlieb, MD, Commissioner, Food and Drug Administration.

III. BACKGROUND

The *21st Century Cures Act* (Cures) was signed into law on December 13, 2016.[1] Enactment of Cures was the culmination of a bipartisan, multi-year effort by Congress to modernize the cycle of discovery, development, and delivery of innovative medical products.

This hearing will provide a status update on Cures, giving members an opportunity to hear from the agency officials at the helm of implementing the drug development and biomedical research provisions included in the law.

A. National Institutes of Health

The *21st Century Cures Act* authorized resources for the National Institutes of Health (NIH) to support biomedical research through the funding of basic, translational, and clinical research. Cures gave NIH the tools and flexibility to alleviate administrative burdens that can hamper biomedical innovation, increase data sharing among NIH-supported researchers, authorize innovation prize competitions, support high-risk, high-reward research, increase funding opportunities for young investigators, improve privacy protections for research volunteers, and encourage the inclusion of diverse populations in clinical research. In addition,

[1] 21st Century Cures Act, Pub. L. No. 114-255.

Cures provided multiyear funding to support four innovative initiatives, which NIH describes in the agency's implementation plan:[2]

- **The Precision Medicine Initiative –** Cures authorized $1.455 billion over 10 years for the Precision Medicine Initiative. NIH, through the *All of Us Research Program*, is building a national resource—one of the world's largest, most diverse biomedical data sets in history—to accelerate health research and medical breakthroughs, enabling individualized prevention, treatment, and care across many health conditions impacting patients today.

- **The Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative –** Cures authorized $1.511 billion over 10 years for the BRAIN Initiative. This Initiative was announced in April 2013 to better understand how the brain stores and retrieves information, in order to improve the ability to diagnose and treat neurological/mental disorders. The Initiative coordinates across multiple NIH Institutes, Federal agencies, and private groups to spur multiple brain research efforts. As outlined in the planning document, *BRAIN 2025: A Scientific Vision*,[3] the NIH plans to advance seven high-level research goals that focus on monitoring and mapping the brain to further understand how neural activity is turned into cognition, emotion, perception, and action.

- **The Cancer Moonshot –** Cures authorized of $1.8 billion over 7 years to accelerate progress in cancer prevention, diagnosis, treatment, and care through the Beau Biden Cancer Moonshot. The National Cancer Institute intends to initiate a large amount of new research and provide a scientific roadmap for the cancer research community to follow in order to take full advantage of the project funding. NIH and the cancer research community are working towards a goal in which:

    - Patients contribute information about their cancer, obtain genomic profiles, and are matched to treatments and clinical trials that best fit their profiles.
    - Health providers and researchers share, access, and analyze information that improves the understanding of tumor evolution, treatment outcomes, and patient symptoms and side effects.
    - Researchers identify possible targets for the development of new cancer treatments and preventive interventions, and learn more about how to avert or overcome cancer drug resistance in patients.

- **The Regenerative Medicine Innovation Project –** Cures authorized $30 million over 4 years for the NIH, in coordination with the Food and Drug Administration (FDA) to support clinical research using adult stem cells to further the field of regenerative medicine. The initiative will address fundamental knowledge gaps and stimulate a

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[2] NATIONAL INSTITUTES OF HEALTH, IMPLEMENTATION OF FUNDING PLAN FOR THE NIH INNOVATION PROJECTS UNDER THE 21ST CENTURY CURES ACT (2017), *available at* https://www.nih.gov/sites/default/files/research-training/initiatives/nih-cures-innovation-plan.pdf.

[3] BRAIN WORKING GROUP REPORT TO THE ADVISORY COMMITTEE TO THE DIRECTOR, NATIONAL INSTITUTES OF HEALTH, BRAIN 2025: A SCIENTIFIC VISION (2014)), *available at* https://www.braininitiative.nih.gov/pdf/BRAIN2025_508C.pdf

coordinated effort to foster major scientific advances and ensure that regenerative medicine clinical studies are standardized, reproducible, and generalizable. The NIH will specifically focus on two goals: identifying meritorious clinical research projects that will enhance the development of safe regenerative medical interventions, and boosting sustained development of this field by setting the foundation and infrastructure for product development, clinical testing, and data standards and sharing.

B. Food and Drug Administration

The *21st Century Cures Act* provided FDA with new authorities to modernize the regulation of medical products throughout the lifecycle of discovery, development, and delivery. Title III of Cures included a broad range of deliverables for FDA, across the spectrum of development, to get new treatments to patients and providers faster, and to keep America at the forefront of medical innovation.[4] The law established an "FDA Innovation Account" and authorized $500 million in funding to carry out Title III. FDA began implementation of Cures with the submission to Congress of a work plan on June 9, 2017.[5]

FDA proposes to dedicate resources in the Innovation Account toward a broad range of activities under Title III of Cures:

- **Patient Focused Drug Development –** To increase patient engagement in medical product development and review, Cures directed FDA to take several steps to establish a transparent and consistent mechanism for the collection and incorporation of patient perspective in regulatory decision making. FDA is allocating resources to development of a guidance document and report that will allow for systematic collection and consideration of patient perspectives on conditions and treatments.

- **Advancing New Drug Therapies –** Cures directed FDA to engage in activities to transform the way drugs are developed. FDA will work on a new qualification process for development tools, reduce duplicative requirements for development of genetically targeted drugs, encourage development of treatments for rare diseases, and facilitate utilization of continuous manufacturing.

- **Modern Trial Design and Evidence Development –** Cures also directed FDA to update the way medical products are reviewed and approved. Clinical protocols and trial designs will be updated to include novel approaches to development, and FDA will evaluate the use of real world evidence to increase efficiency of research in an era of big data.

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[4] FOOD & DRUG ADMIN., 21ST CENTURY CURES ACT DELIVERABLES (2017), https://www.fda.gov/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFDCAct/21stCenturyCuresAct/ucm562475.htm.

[5] FOOD & DRUG ADMIN., WORK PLAN AND PROPOSED FUNDING ALLOCATIONS OF FDA INNOVATION ACCOUNT (2017), *available at* https://www.fda.gov/downloads/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFDCAct/21stCenturyCuresAct/UCM562852.pdf.

- **Patient Access to Therapies and Information –** Cures sought to facilitate access to the emerging field of regenerative therapy by requiring FDA to establish the standards and definitions necessary to develop these products. Cures also takes several steps to make combination product review consistent and efficient, and to advance FDA's reliance on qualified data summaries for supplemental applications.

- **Medical Device Innovation –** Cures established in law an expedited access program for the review of breakthrough devices and technologies in development for life-threating or debilitating conditions. Cures expanded access to devices for rare diseases or conditions by directing FDA to take several steps relating to Humanitarian Use Devices. Furthermore, Cures clarified FDA's role with respect to medical device software, and the agency will propose a system for surveillance of certain products and functionalities.

- **Improving Scientific Expertise and Outreach at FDA –** FDA has established the Oncology Center of Excellence pursuant to Cures' requirement that the agency set up intercenter institutes that will coordinate the activities undertaken in major disease areas. Increased integration of the centers for drugs, devices, and biologics is intended to streamline and expedite product review and approval.

IV. STAFF CONTACTS

If you have any questions regarding this hearing, please contact Kristen Shatynski, Danielle Steele, and Paul Edattel of the Committee staff at (202) 225-2927.