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RANKING MEMBER

ONE HUNDRED FOURTEENTH CONGRESS

Congress of the United States
House of Representatives

COMMITTEE ON ENERGY AND COMMERCE
2125 RAYBURN HOUSE OFFICE BUILDING
WASHINGTON, DC 20515-6115

Majority (202) 225-2927
Minority (202) 225-3641

MEMORANDUM

May 19, 2015

To: Committee on Energy and Commerce Democratic Members and Staff

Fr: Committee on Energy and Commerce Democratic Staff

Re: Full Committee Markup of H.R. __, the "21st Century Cures Act"

On Tuesday, May 19, 2015, at 5:00 p.m. in room 2123 of the Rayburn House Office Building the Committee on Energy and Commerce will convene for opening statements for a markup of H.R. __, the 21st Century Cures Act.". The Committee will reconvene on Wednesday, May 20, 2015, at 10:00 a.m. in room 2123 of the Rayburn House Office Building.

This memorandum supplements information provided in the memoranda from the previous legislative hearing and the Subcommittee on Health markup on the 21st Century Cures Act.

I. SUMMARY OF CHANGES TO H.R. __, THE "21st CENTURY CURES ACT"

A bipartisan draft of the "21st Century Cures Act" was reported out of the subcommittee by voice vote. Below is a summary of the changes included in the amendment in the nature of a substitute (AINS) to the "21st Century Cures Act" released this morning. We expect that additional technical edits to specific provisions will be considered by the Committee in a manager's amendment when it reconvenes on Wednesday at 10:00 a.m. to consider the AINS.

II. TITLE I—DISCOVERY

A. Subtitle A—National Institutes of Health (NIH) Funding

Section 1002. NIH Innovation Fund

In the previous draft, Section 1002 contained an appropriations trigger that required NIH's annual appropriations be at least as much as the amount appropriated in a previous fiscal year in order for NIH to receive the $2 billion in mandatory funding each year. That trigger has been replaced with a requirement that the mandatory funding be unlocked each year as part of the annual appropriations process. Therefore, there is no discretionary minimum that must be

met for NIH to receive the $2 billion in mandatory funding each year. Additionally, because the mandatory funding will be unlocked as part of the annual appropriations process, any restrictions applied to the funding will be debated as part of the annual appropriations process.

B. Subtitle B—National Institutes of Health Planning on Administration

Section 1023. Reducing Administration Burdens of Researchers

In the previous draft, this provision would have created a new Biomedical Research Working Group. That group would have been charged with making recommendations on how to reduce administrative burdens of researchers funded by NIH. After hearing from NIH about all the efforts that have been completed and are ongoing to examine this issue, we determined that there is no need for this group or for its report. Therefore, the Working Group has been deleted from this provision. Now, the NIH Director would have to consult with certain groups and existing reports about this issue in order to implement a plan to reduce administrative burdens.

III. TITLE II—DEVELOPMENT

A. Subtitle B—Qualification and Use of Drug Development Tools

Section 2021. Biomarkers, surrogate endpoints, and other drug development tools; and Section 2022. Accelerated Approval Development Plan

This section would codify FDA's current qualification process for biomarkers and other drug development tools, require FDA to issue guidance establishing a framework for qualification of biomarkers and other drug development tools, and provide for greater transparency and collaboration throughout the guidance development and qualification process.

The AINS makes clarifying changes in Section 2021 to the definition of biomedical research consortia, and transparency requirements for drug development tool qualification submissions. Technical changes have also been made to Section 2022 regarding the accelerated approval development plan to clarify that the Secretary's determination shall be in regard to whether the drug meets the criteria to use the accelerated approval pathway.

B. Subtitle C – FDA Advancement of Precision Medicine

Subtitle 2041. Precision Medicine Guidance and Other Programs of Food and Drug Administration

Section 2041 of the AINS seeks to build on the announcement of the President's Precision Medicine Initiative by requiring FDA to issue guidance defining a "precision drug or biological product", as well as issue and periodically update guidance that would help with the development of such products. Technical changes have been made to Section 2041 regarding the sponsor of a precision drug or biological product's ability to obtain a right of reference to another sponsor's data and information for purposes of expediting development.

C. Subtitle D – Modern Trial Design and Evidence Development

Section 2062. Utilizing Evidence from Clinical Experience

Section 2062 requires FDA to establish a program to evaluate the potential use of evidence from clinical experience to help support the approval of a new indication for a drug and to help support or satisfy post-approval study requirements. The AINS makes clarifying changes to Section 2062 regarding the consultation process when developing the clinical experience program framework.

Section 2063. Streamlined Data Review Program

Section 2063 would require FDA to establish a streamlined data review program that allows for the submission of clinical data summaries to support the approval or licensure of specified new indications of drugs and biologics, provided that certain qualifying criteria are met. Section 2063 has been revised to require FDA to make certain information about the program publicly available annually. Such information shall include: the number of applications reviewed under the streamlined data review program; the average time for completion of the review under the program versus review of other applications for new indications; and the number of applications reviewed under the program for which FDA used full data sets in addition to the qualified data summary. In addition, the revised language in this section includes a *Sense of Congress* expressing that the program should enable FDA to make approval decisions based on qualified data summaries.

D. Subtitle F – Facilitating Dissemination of Health Care Economics Information

Section 2101. Facilitating Dissemination of Health Care Economic Information

Section 2101 would facilitate the dissemination of healthcare economic information to payors, formulary committees, or other similar entities. This provision will provide manufacturers with the ability to provide information about the economic value of their product to payors, but ensures the continuance of safeguards to prevent the promotion of the product for uses that have not been approved by FDA as being safe and effective. Technical changes were made to Section 2101 to clarify what "health care economic information" means, as well as the ability of such information to include a statement describing material differences between health care economic information and the approved drug labeling.

E. Subtitle G—Antibiotic Drug Development

Section 2123. Encouraging the Development and Responsible Use of New Antimicrobial Drugs

This section provides an additional Medicare reimbursement bump for new "superbug" antibiotics that are developed as a last line of defense against highly-resistant infections. Antibiotic resistance has been named the top public health threat by the World Health Organization (WHO), and accordingly, there has been a worldwide effort to preserve the antibiotics we have today, and develop new antibiotics that are capable of treating so-called "superbugs" that are virtually impossible to treat. This provision has changed since the last draft to address technical concerns by CMS. In addition, the new language clarifies that a company can elect to take either the new incentive or a New Technology Add-on Payment (NTAP),

establishes an annual overall cap on the new incentive, and clarifies that a drug can receive the incentive for a period of four years.

F. Subtitle I—Orphan Products Extension Now; Incentives for Certain Products for Limited Populations

Section 2151. Extension of Exclusivity Periods for a Drug Approved for a New Indication for a Rare Disease or Condition

This draft has been changed so that it now does not require FDA to implement the program through rulemaking, reducing some unnecessary burden on the agency. However, the draft policy is not currently integrated into the existing FDA Orphan Drug program, which would support administrative efficiency and maintain FDA controls on eligibility for orphan drug designation. There are still ongoing discussions around making further improvements to this section.

Section 2152. Reauthorization of Rare Pediatric Disease Priority Review Voucher Incentive Program

This section is no longer in brackets. The program has been reauthorized until Dec 31, 2018. The section also now includes a GAO study on the effectiveness of the program in providing incentives for the development of rare pediatric disease drugs that would not have otherwise been developed. GAO is to report its findings by the end of 2017, in time for Congress to take it into account when considering whether to extend the program at the end of 2018.

G. Subtitle O—Streamlining Clinical Trials

Section 2261. Protection of Human Subjects in Research

This section has been extensively revised, and is no longer in brackets. It requires the Secretary to harmonize the differences between the HHS Human Subject Regulations and the FDA Human Subject Regulations to the extent possible. It also requires that the Secretary modernize these regulations in the context of multisite and cooperative research projects, while incorporating local considerations, community values and mechanisms to protect vulnerable populations.

IV. Title III—DELIVERY

A. Subtitle A—Interoperability

Section 3001. Interoperability

This section has been significantly changed since the last draft. It builds upon the work from the HITECH Act by requiring that health information technology (HIT) be interoperable. To be interoperable, HIT must allow for the secure transfer of all available patient data without special effort, and it must not be configured, set up, or implemented to block the sharing of information.

This section gives the Secretary contract authority, and requires her to contract with accredited Standards Development Organizations to set standards for HIT in order to achieve interoperability.

All electronic health records (EHRs) will need to be interoperable to be certified for the meaningful use program or face decertification. Additionally, EHRs will require more vigorous testing prior to certification, including testing in the field. EHR vendors will attest that their products are interoperable and do not engage in information blocking. This section includes a hardship exemption from the meaningful use program for providers that purchased an EHR that was subsequently decertified through no fault of the provider. The Medicare/Medicaid meaningful use program, established in the HITECH Act, requires that providers use EHRs in a meaningful way to improve quality of health care in order to receive incentive payments (initially) and not be financially penalized (starting in 2015). EHR products must be certified in order for providers to meet meaningful use.

This section also gives the HHS Inspector General the authority to investigate vendors in violation of their meaningful use attestations and vendors, providers, health information system providers who engage in information blocking. The IG will also be authorized to levy fines on those found to knowingly block the sharing of information.

The Office of the National Coordinator will create a portal to make EHR vendor practices more transparent, and allow providers to compare products and pricing structures.

Last, in order to facilitate patient engagement, this section includes a *Sense of Congress* indicating that patients have the right to access their medical information and clarifies HIPAA privacy regulations.

The Committee intends to continue its bipartisan work on this policy.

B. Subtitle H—Medicare Part D Patient Safety and Drug Abuse Prevention

Section 3141. Establishing PDP Safety Program to Prevent Fraud and Abuse in Medicare Prescription Drug Plans

Additional changes have been made to this section since the subcommittee markup to include a provision to ensure that prescribers and pharmacies are available to at-risk beneficiaries in the case of emergency or multiple residencies. The new language also addresses stakeholder concerns regarding beneficiaries in long-term care settings, and explicitly lists retail pharmacies, physicians, and pharmacists as part of the stakeholder group referenced in the legislation.