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

May 15, 2015

TO: Members, Committee on Energy and Commerce

FROM: Committee Majority Staff

RE: Full Committee Markup

I. INTRODUCTION

The Committee on Energy and Commerce will meet in open markup session on May 19 and 20, 2015, in 2123 Rayburn House Office Building.

On Tuesday, May 19, 2015, 5:00 p.m., the Committee will convene for opening statements only. The Committee will reconvene on Wednesday, May 20, 2015, at 10:00 a.m. to consider the following:

- H.R. ____, 21st Century Cures Act.

In keeping with Chairman Upton's announced policy, Members must submit any amendments they may have two hours before they are offered during this markup. Members may submit amendments by email to peter.kielty@mail.house.gov. Any information with respect to an amendment's parliamentary standing (e.g., its germaneness) should be submitted at this time as well.

II. H.R. ____, 21ST CENTURY CURES ACT

In April 2014, Chairman Fred Upton (R-MI) and Rep. Diana DeGette (D-CO) launched the 21st Century Cures initiative to conduct a comprehensive look at the cycle of cures – from discovery to development to delivery.

Over the course of the last year, the 21st Century Cures initiative involved a nationwide conversation with patients, providers, innovators, regulators, and researchers. During the conversation, the Committee received countless ideas in response to the Committee white papers, held eight hearings convened by Subcommittee on Health, and held over a dozen roundtables hosted both at the Committee and by members in their districts all across the country.

In January, the Committee launched the legislative phase of the 21st Century Cures initiative by circulating a discussion document, which included a number of ideas proposed by both Republicans and Democrats. The Subcommittee on Health held a hearing on the discussion document on April 30, 2015, and on May 14, 2015, the Subcommittee met in open markup session to consider the discussion draft and forwarded the bill, as amended, to the full Committee by a voice vote.

A summary of the draft follows:

Title I - Discovery

Subtitle A - National Institutes of Health Funding

- **Section 1001. National Institutes of Health Reauthorization**: This section would reauthorize the National Institutes of Health (NIH) for three years: Fiscal Year (FY) 2016 through FY2018.

- **Section 1002. NIH Innovation Fund**: This section would establish an innovation fund at NIH for five years, FY2016 through FY2020, to support biomedical research through the funding of basic, translational, and clinical research.

Subtitle B – National Institutes of Health Planning and Administration

- **Section 1021. NIH Research Strategic Plan**: This section would require NIH to issue a strategic plan.

- **Section 1022. Increasing Accountability at the National Institutes of Health**: This section would increase accountability at NIH, including with respect to the award of grants.

- **Section 1023. Biomedical Research Working Group**: This section would establish a working group to provide recommendations on how to streamline the grant process for researchers.

- **Section 1024. Exemption for the National Institutes of Health from the Paperwork Reduction Act Requirements**: This section would exempt certain NIH research activities from the Paperwork Reduction Act.

- **Section 1025. NIH Travel**: This section would set forth a sense of Congress to reiterate the importance of scientific conferences and meetings to the mission of NIH.

- **Section 1026. Other Transactions Authority**: This section would provide the National Center for Advancing Translational Science (NCATS) at NIH with more flexibility on the use of Other Transaction Authority (OTA) so it can operate like the Defense Advanced Research Projects Agency (DARPA).

- **Section 1027. NCATS Phase IIB Restriction**: This section would remove a restriction on NCATS' conduct of, or grants for, phase II and III clinical trials.

- **Section 1028. High-Risk, High Reward Research**: This section would support research that pursues innovative approaches to major challenges in biomedical research that involve inherent high risk, but have the potential to lead to breakthroughs.

Subtitle C – Supporting Young Emerging Scientists

- **Section 1041. Improvement of Loan Repayment Programs of National Institutes of Health**: This section would improve loan repayment programs for NIH researchers.

- **Section 1042. Report**: This section would require the NIH Director to submit a report to Congress on programs for young emerging scientists at NIH.

Subtitle D – Capstone Grant Program

- **Section 1061. Capstone Award**: This section would create a capstone grant program to support outstanding scientists who have been funded by NIH.

Subtitle E – Promoting Pediatric Research through the National Institutes of Health

- **Section 1081. National Pediatric Research Network**: This section would require NIH to establish a national pediatric research network. It would be composed of research institutions that would operate as a consortium in order to pool resources and coordinate activities related to pediatric rare diseases or birth defects.

- **Section 1082. Global Pediatric Clinical Trial Network Sense of Congress**: This section would set forth a sense of Congress that NIH and the Food and Drug Administration (FDA) should work with the European Union, industry, and others to establish a global pediatric clinical trial network.

- **Section 1083. Appropriate Age Groupings in Clinical Research**: This section would help ensure appropriate age groupings are included in research studies involving human subjects.

Subtitle F – Advancement of National Institutes of Health Research and Data Access

- **Section 1101. Sharing of Data Generated Through NIH-Funded Research**: This section would allow the NIH Director to require those whose research is supported by NIH to share their data.

- **Section 1102. Standardization of Data In Clinical Trial Registry Data Bank on Eligibility for Clinical Trials**: This section would enhance patient searches for ongoing trials by requiring NIH to standardize certain patient inclusion and exclusion information across all trials housed in ClinicalTrials.gov.

Subtitle G – Facilitating Collaborative Research

- **Section 1121. Clinical Trial Data System**: This section would create a third-party scientific research sharing system for trials solely funded by the Federal government in order to allow the use and analysis of data beyond each individual research project.

- **Section 1122. National Neurological Diseases Surveillance System**: This section would require the Centers for Disease Control and Prevention (CDC) to set up a surveillance system for neurological diseases like Parkinson's disease and Multiple Sclerosis.

- **Section 1123. Data on Natural History of Diseases**: The section would establish a public-private partnership to establish or enhance and support an information technology system, including staffing, to collect, maintain, analyze, and interpret data on the natural history of diseases, with a particular focus on rare diseases.

- **Section 1124. Accessing, Sharing, and Using Health Data for Research Purposes**: This section would require the secretary to revise several provisions in the Health Insurance Portability and Accountability Act (HIPAA) Privacy Rule to clarify certain permissible uses of protected health information.

Subtitle H – Council for 21st Century Cures

- **Section 1141. Council for 21st Century Cures**: This section would establish a public-private partnership in the United States to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients.

Title II - Development

Subtitle A – Patient-Focused Drug Development

- **Section 2001. Development and Use of Patient Experience Data to Enhance Structured Risk-Benefit Assessment Framework**: Because no one understands a particular condition or disease better than patients living with it, this section would require FDA to establish a structured framework for the meaningful incorporation of patient experience data into the regulatory decision-making process, including the assessment of desired benefits and tolerable risks associated with new treatments.

Subtitle B – Qualification and Use of Drug Development Tools

- **Section 2021 – Qualification and Use of Drug Development Tools**: This section would require FDA to issue guidance that would assist in the development of biomarkers, with input from public-private partnerships, and establish a codified process for qualification of biomarkers and other drug development tools. This section would also provide for transparency and collaboration throughout the guidance development and qualification process.

- **Section 2022. Accelerated Approval Development Plan**: This section would facilitate early interactions and agreement between sponsors and FDA on designing studies to generate evidence for purposes of accelerated approval.

Subtitle C – FDA Advancement of Precision Medicine

- **Section 2041. Precision Medicine Guidance and Other Programs of Food and Drug Administration**: This section would require FDA to issue, and periodically update, guidance documents intended to assist sponsors in the development of precision drugs and biological products.

Subtitle D – Modern Trial Design and Evidence Development

- **Section 2061. Broader Application of Bayesian Statistics and Adaptive Trial Designs**: This section would require FDA to hold a public meeting and issue guidance documents that would assist sponsors in incorporating adaptive designs and Bayesian statistical modeling into their clinical protocols and new drug applications.

- **Section 2062. Utilizing Evidence from Clinical Experience**: This section would require 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. In parallel, FDA would identify and execute pilot demonstrations to extend existing use of the Sentinel System to support these efforts.

- **Section 2063. Streamlined Data Review Program**: This section would require FDA to establish a streamlined data review program that would make use of submitted clinical data summaries to support the approval or licensure of specified new indications of drugs and biologics if certain qualifying criteria are met.

Subtitle E – Expediting Patient Access

- **Section 2081. Sense of Congress**: This section would express the support of Congress for FDA's expedited approval of breakthrough therapies.

- **Section 2082. Expanded Access Policy**
  Section 2083. Finalizing Draft Guidance on Expanded Access: These sections would place transparency requirements on certain drug companies regarding their expanded access programs (programs for patients to access drugs before they are approved) and require FDA to finalize guidance regarding how it interprets and uses adverse drug event data resulting from drug use under such expanded access programs.

Subtitle F – Facilitating Responsible Manufacturer Communications

- **Section 2101. Facilitating Dissemination of Health Care Economic Information**: This section would add clarity and facilitate dissemination of health care economic

information, as defined in the section, to payers, formulary committees, or other similar entities.

- **Section 2102. Facilitating Responsible Communication of Scientific and Medical Developments**: This section would require FDA to issue guidance on facilitating responsible, truthful, and non-misleading scientific and medical information not included in the label of drugs.

Subtitle G – Antibiotic Drug Development

- **Section 2121. Approval of Certain Drugs for Use in a Limited Population of Patients**: This section builds off of the progress Congress made with the passage of the GAIN Act as a part of the Food and Drug Safety and Innovation Act (FDASIA) in 2012 by facilitating the development of new antibacterial or antifungal drugs through a tailored FDA approval pathway.

- **Section 2122. Susceptibility Test Interpretive Criteria for Microorganisms**: This section streamlines the process by which FDA can clear or approve updates to antimicrobial susceptibility testing devices.

- **Section 2123. Encouraging the Development and Responsible and Use of New Antimicrobial Drugs**: This section provides for higher Medicare payments for certain antimicrobial drugs.

Subtitle H – Vaccine Access, Certainty, and Innovation

- **Section 2141. Timely Review of Vaccines by the Advisory Committee on Immunization Practices**:
  Section 2142. Review of Processes and Consistency of ACIP Recommendations:
  Section 2143. Meeting Between CDC and Vaccine Developers: These sections would create and formalize processes for the making of vaccination scheduling recommendations by the advisory committee on immunization practices (ACIP), for CDC review of the ACIP recommendations, and for meetings between CDC and vaccine developers.

Subtitle I – Orphan Product Extensions 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 provision would provide incentives for drug companies to conduct research to repurpose approved drugs for rare diseases and seek approval from FDA for a rare disease indication on its label.

- **Section 2152. Reauthorization of Rare Pediatric Disease Priority Review Voucher Incentive Program**: This section would reauthorize the rare pediatric disease priority review voucher (PRV) program.

Subtitle J – Domestic Manufacturing and Export Efficiencies

- **Section 2161. Grants for Studying the Process of Continuous Drug Manufacturing**: This section would allow FDA to award grants to higher education and non-profit organizations to study and recommend improvements to the process of continuous manufacturing, and other similar innovative monitoring and control techniques, of drugs and biologics.

- **Section 2162. Re-Exportation among Members for the European Economic Area**: This section would allow U.S. pharmaceutical manufacturers to export controlled substances from a second country as European pharmaceutical companies are allowed to do. Currently, U.S. pharmaceutical manufacturers are prohibited from re-exporting controlled substances from any second country.

Subtitle K – Enhancing Combination Products Review

- **Section 2181. Enhancing Combination Products Review**: This section requires FDA to issue a final guidance describing the role of all agency centers when reviewing a combination product.

Subtitle L – Priority Review for Breakthrough Devices

- **Section 2201. Priority Review for Breakthrough Devices**: In order to provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions, this section would require FDA to establish a priority review program for certain breakthrough medical devices technologies.

Subtitle M – Medical Device Regulatory Process Improvements

- **Section 2221. Third-Party Quality System Assessment**: This section would establish a voluntary third-party quality system assessment program where device companies could have their quality system certified by an FDA-authorized third party and, once certified, gain certain efficiencies in the FDA pre-market review process.

- **Section 2222. Valid-Scientific Evidence**: This section would clarify that, in the context of FDA evaluation of medical devices, valid scientific evidence may include registry data, studies published in peer-review journals, and data collected in countries other than the United States so long as certain criteria are met.

- **Section 2223. Training and Oversight in Least-Burdensome Appropriate Means Concept**: This section would ensure that each FDA employee involved in the review of medical device submissions receives adequate training in the meaning and implementation of the least burdensome means concept.

- **Section 2224. Recognition of Standards**: This section would establish a clear process at FDA for the submission, review, and recognition of standards established by a nationally or internationally recognized standard organization for purposes of medical device review.

- **Section 2225. Easing Regulatory Burden with Respect to Certain Class I and Class II Devices**: This section would allow FDA to focus its oversight on those devices that pose more risks to patients.

- **Section 2226. Advisory Committee Process**: This section would improve the medical device classification panel review process at FDA to ensure adequate expertise among panel members to assess the device and allow for presentation by the device sponsor to the panel, among other things.

- **Section 2227. Humanitarian Device Exemption Application**: This section would provide FDA with the authority to apply the humanitarian device exemption to diseases and conditions that affect up to 8,000 individuals in the U.S.

- **Section 2228. CLIA Waiver Study Design Guidance for In Vitro Diagnostics**: This section would require FDA to issue guidance clarifying CLIA waiver study design.

Subtitle N – Sensible Oversight for Technology Which Advances Regulatory Efficiency

- **Section 2241. Health Software**:
  Section 2242. Applicability and Inapplicability of Regulation:
  Section 2243. Exclusion from Definition of Device: Technological innovation around health software and wireless platforms such as smartphones hold great promise for our 21st century health care system. However, there are concerns related to the current regulatory approach for health technologies. Sections 2241-2243 would support further development in this field by updating the regulatory laws around software and creating clarity for developers and reviewers alike.

Subtitle O – Streamlining Clinical Trials

- **Section 2261. Protection of Human Subjects in Research; Applicability of Rules**: This section would exempt clinical trials that are subject to the comprehensive human subject regulations under the Federal Food Drug and Cosmetic Act (FFDCA) from requirements under the Common Rule. Further, this section would streamline the institutional review board (IRB) process for trials that are being conducted at multiple sites.

- **Section 2262. Use of Non-Local Institutional Review Boards for Review of Investigational Device Exemptions and Human Device Exemptions**: This section would remove the limitation on the use of central IRBs in medical device trials.

- **Section 2263. Alteration or Waiver of Informed Consent for Clinical Investigations**: This section would amend certain provisions governing the informed consent process for enrolling patients in medical device tests that poses no more than minimal risk and include appropriate safeguards to protect the rights, safety, and welfare of the participants.

Subtitle P – Improving Scientific Expertise and Outreach at FDA

- **Section 2281. Silvio O. Conte Senior Biomedical Research Service**: This section would enable FDA to hire more efficiently and ensure that the agency has the staff required to ensure they keep up with the pace of innovation.

- **Section 2282. Enabling FDA Scientific Engagement:** This section expresses congressional support for eliminating barriers that prevent agency staff from attending scientific conferences and meetings. Allowing staff travel for their continued training and education will help the agency keep pace with the latest scientific developments.

- **Section 2283. Reagan-Udall Foundation for the Food and Drug Administration**: This section would ensure that the Reagan-Udall Foundation has access to the expertise and human capital it needs to fulfill its statutory mission of advancing FDA's scientific priorities.

- **Section 2284. Collection of Certain Voluntary Information Exempted from Paperwork Reduction Act**: This section would exempt FDA from the Paperwork Reduction Act with respect to the collection from patients, industry, academia, and other stakeholders of voluntary information through voluntary surveys and questionnaires. This will enable FDA to more easily and efficiently receive patient input.

Title III – Delivery

Subtitle A – Interoperability

- **Section 3001. Ensuring Interoperability**: As evidenced by statements from numerous 21st Century Cures roundtable participants, the ability to share research and clinical data is a cornerstone of our drive for new cures. The Office of the National Coordinator (ONC) for Health Information Technology has led the charge but recently has identified barriers to nationwide interoperability of health technology. Section 3001 would refocus national efforts on making systems interoperable and holding individuals responsible for blocking or otherwise inhibiting the flow of patient information throughout our healthcare system.

Subtitle B – Telehealth

- **Section 3021. Telehealth Service Under the Medicare Program**: The Energy and Commerce Bipartisan Telemedicine Member Working Group has been working to find a solution that has plagued Congress and our health system for decades: how to adopt new

technologies into our delivery system in ways that promote greater quality care and fiscal integrity. Section 3021 supports the efforts of the working group by requiring specific actions of government bodies identified as critical to developing a long-term solution to this problem.

Subtitle C – Encouraging Continuing Medical Education for Physicians

- **Section 3041 – Exemption from Manufacturer Transparency Reporting Certain Transfers Used for Education Purposes**: This section would exempt certain transfers of value to physicians from reporting requirements that have hindered physician participation in important continuing medical education activities.

Subtitle D – Disposable Medical Technologies

- **Section 3061. Treatment of Certain Items and Devices**: Today, seniors who receive their care in a home setting are not able to access certain services afforded others because of the nature of the DME payment system. The committee believes that this needs to change and is currently working with the administration on a solution.

Subtitle E – Local Coverage Decision Reforms

- **Section 3081. Improvements in the Medicare Low Coverage Determination (LCD) Process**: The LCD process is an important means by which seniors can access treatments that would otherwise not be covered by Medicare due to the length of time it takes for the national process to conclude its work. However, improvements are needed. Section 3061 would increase transparency around the LCD process.

Subtitle F – Medicare Pharmaceutical and Technology Ombudsman

- **Section 3101. Medicare Pharmaceutical and Technology Ombudsman**: This section would create a new technology ombudsman within Medicare to address problems relating to coverage of new and life-saving technologies.

Subtitle G – Medicare Site-of-Service Price Transparency

- **Section 3121. Medicare Site-of-Service Price Transparency**: The Medicare benefit currently pays varying rates for the same services depending on where they are delivered. Seniors' out of pocket costs can be higher or lower for a given procedure based upon where the service is provided. This section would give seniors the ability to shop among various sites of service for certain services so that they can identify the most cost-effective treatment.

Subtitle H – Medicare Part D Patient Safety and Drug Abuse Prevention

- **Section 3141. Programs to Prevent Prescription Drug Abuse Under Medicare Parts C and D**: This section would allow prescription drug plans in Part D to develop a safe

prescribing and dispensing program for beneficiaries that are prescribed a high volume of controlled substances.

III. STAFF CONTACTS

If you have any questions regarding this markup, please contact Karen Christian of the Committee staff at (202) 225-2927.