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ONE HUNDRED FOURTEENTH CONGRESS

Congress of the United States
House of Representatives

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**MEMORANDUM**

**May 13, 2015**

**To: Subcommittee on Health Democratic Members and Staff**

**Fr: Committee on Energy and Commerce Democratic Staff**

**Re: Subcommittee on Health Markup of the discussion draft H.R. __, the "21st Century Cures Act" and of H.R. 1321, Microbead-Free Waters Act of 2015**

On Thursday, May 14, 2015, at 10:00 a.m. in room 2123 of the Rayburn House Office Building, the Subcommittee on Health will convene for a markup of the discussion draft H.R. __, the "21st Century Cures Act." The subcommittee will also markup H.R. 1321, the "Microbead-Free Waters Act of 2015."

Background information provided in the memoranda for previous legislative hearings on both bills in the 114th Congress are attached.

## I. BACKGROUND ON H.R. __, THE "21st CENTURY CURES ACT"

A bipartisan substitute amendment (AINS) to the "21st Century Cures Act", released this morning, will be offered at the markup by Chairman Fred Upton, Rep. Diana DeGette, Health Subcommittee Chairman Joe Pitts, Ranking Member Frank Pallone, Jr., and Health Subcommittee Ranking Member Gene Green at the markup.

The AINS reflects the result of continued bipartisan efforts by the Committee, working with the Administration and a diverse group of stakeholders, to improve the bipartisan discussion draft that was released by the Committee in April. It also addresses several concerns raised at the April 20, 2015 legislative hearing. Discussions around "21st Century Cures Act" will continue, however, many of the outstanding concerns raised at that hearing have been resolved.

Some key changes to the AINS include the following:

- In Title I, Section 1041 was dropped. That section had provided for funding of research by emerging scientists. However, such funding is now provided through the NIH Innovation Fund in Section 1002.

- Title I also includes new Section 1083, which encourages the inclusion of children and seniors in clinical research.

- In Title II, Section 2102 was added to facilitate communication by companies of scientific and medical information about their medical products.

- New Section 2151 provides six months additional exclusivity and patent term extension for drugs that get a second indication for a rare disease or condition.

- New Section 2152 reauthorizes the rare pediatric disease priority review voucher incentive program; and

- New Section 2181 requires FDA to publish a guidance clarifying the individual responsibilities of the relevant FDA Centers in oversight of combination products.

## II. TITLE I—DISCOVERY

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

*Section 1002. NIH Innovation Fund*

The previous draft contained a general framework that would direct funding into specific priorities, including precision medicine and research projects conducted by emerging scientists. This draft has further developed this provision.

The five-year, $10 billion NIH Innovation Fund (Fund) would be routed through the NIH Director. The provision establishes certain criteria for the Director to allocate funding among the national research institutes and centers (ICs). The NIH Director would be granted flexibility in the award types that can be funded under the NIH Innovation Fund. Those types include research awards tied to a specific research aim, research awards for innovative scientists, and research awards to small businesses. NIH would be required to issue a strategic plan for the Fund that explains how the Fund will support research priorities.

At least $500 million must be allocated to the Accelerating Advancement Program (Program). The Program would provide matching funds to ICs to fund innovative research projects that could not be funded within the current budget of an IC. Requiring this buy-in from ICs also creates the potential that funding from the Innovation Fund will be used to launch new scientific research endeavors that could extend beyond the initial award from the Program.

Other criteria include a restriction on the amount of funding that may be used to support NIH intramural research. This restriction ensures that the majority of the funding from the Fund will be used to support scientists at academic and research institutions across the country. The Fund would also have specific funding allocations for young scientists and high-risk, high-reward research. The Fund would be required to provide funding to support advancing research in biomarkers, precision medicine, infectious diseases, and antibiotics.

The Fund also includes an exemption from HHS transfer authority and the Public Health Service Act (PHSA) Evaluation Set Aside Program, or "TAP." Transfer authority provides flexibility for HHS to transfer money from one agency or office to another. The TAP allows HHS to use funding from PHSA programs to pay to evaluate health programs within HHS.

### B. Subtitle C—Supporting Young Emerging Scientists

*Funding Research by Emerging Scientists (Section 1041 in previous draft)*

This provision has been deleted from the current draft. This provision in no longer needed because there is funding allocated for young scientists under the new NIH Innovation Fund.

### C. Subtitle E—Promoting Pediatric Research through the NIH

*Section 1083. Appropriate Age Groupings in Clinical Research*

This provision has been added to ensure that children and elderly adults are not inappropriately excluded from clinical trials sponsored by NIH. This provision would require NIH to consult with pediatric and geriatric experts in order to develop guidelines on how age should affect inclusion or exclusion in clinical trials. NIH would also be required to report on its website biennially the number of children included in NIH-sponsored clinical trials.

### D. Subtitle F—Advancement of NIH Research and Data Access

*Section 1102. Standardization of Data in Clinical Trial Registry Data Bank of Eligibility for Clinical Trials*

This section would require NIH to post clinical trial inclusion and exclusion criteria in a standardized format. Minor changes have been made to the current version of this provision that would give NIH flexibility in determining which inclusion and exclusion criteria to standardize.

### E. Subtitle G—Facilitating Collaborative Research

*Section 1121. Clinical Trial in Data System*

In the previous draft, Section 1121 would have created a permanent research sharing system for trials solely funded by the NIH. That system would require all data from research solely funded by NIH to be input into the system and shared upon the request of eligible parties. There remain questions about whether this system is the best method for ensuring that research data can be used beyond an individual research project. Because those questions remain, this draft would create a seven-year pilot research sharing system rather than a permanent system in order to determine the effectiveness of such a system. Before the end of the 6th year of the pilot, the GAO would be required to issue a report to Congress and the Secretary of Health and Human Services evaluating the effectiveness of the pilot. At the end of the pilot, the Secretary would decide whether to extend the pilot, make the pilot permanent, or terminate the pilot.

*Section 1122. National Neurological Diseases Surveillance System*

This section now includes an authorization of appropriations of $5 million.

*Section 1123. Data on Natural History of Diseases*

This section now includes an authorization of appropriations of $5 million.

### F. Subtitle H—Council for 21st Century Cures

*Section 1141. Council for 21st Century Cures*

This section now includes an authorization of appropriations of $10 million.

## III. TITLE II—DEVELOPMENT

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

*Section 2021. Biomarkers, surrogate endpoints, and other drug development tools*

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 would clarify and encourage collaboration and engagement between FDA and biomedical research consortia, entities, and other individuals with experience in the development and qualification of biomarkers and other drug development tools, including during the development of the taxonomy and guidance documents. Further, the AINS would outline how FDA should prioritize the qualification of these tools, and would authorize $10 million a year for fiscal years (FY) 2016 through 2020 to assist FDA in these efforts.

### 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.

The following areas that the guidance may address include: evidence needed to support the use of biomarkers that identify subsets of patients as likely responders to therapies in order to streamline clinical trials; recommendations for study designs that will help demonstrate the validity of a biomarker; how benefit-risk assessments may be impacted by the use of biomarkers in identifying patient population subsets; development of companion diagnostics; and considerations for developing biomarkers that would help in prescribing decisions for a drug or

biological product and when such information should be included in FDA-approved product labeling. FDA is required to issue guidance within 18 months of enactment.

This revised section reflects the desire of FDA to have greater flexibility in issuing guidance relating to precision medicine, and the need for the agency to be able to adapt as innovation and development related to precision medicine continues to evolve.

### C. SUBTITLE D – Modern Trial Design and Evidence Development

*Section 2062. Utilizing Evidence from Clinical Experience*

Section 2062, which 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. The revised language would allow FDA an additional time to establish a draft framework, for implementation, and to publish and finalize guidance for industry on utilization of the program.

This section would also require FDA identify and execute pilot demonstrations to extend the use of the Sentinel System surveillance infrastructure in support of the collection of clinical evidence. Discussions continue regarding how best to extend Sentinel system for such use, while maintaining privacy of patients.

*Section 2063. Streamlined Data Review Program*

Section 2063 would require FDA to establish a streamlined data review program that allow for the submission of clinical data summaries to support the approval or licensure of specified new indications of drugs and biologics if certain qualifying criteria are met. Discussions around this section are continuing and are centered around whether full data sets should be submitted at the time the qualified data summaries are submitted. Submission of the full data set at the time of the summary would allow the agency access to the data if needed.

### D. SUBTITLE E – Expanding Patient Access

*Section 2082. Expanded Access Policy*

Section 2082 of the AINS would require certain sponsors to make publicly available their policy regarding expanded access, including the contact information, process, and criteria for such requests, as well as the length of time the sponsor anticipates will be needed to acknowledge the request. The revised language would allow sponsors to post a general policy applicable to all of the sponsor's investigational drugs, and would specify the process by which a sponsor revise such a policy.

### E. 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 ensure safeguards continue to remain to prevent the promotion of the product for uses that have not been approved by FDA as being safe and effective.

It is important to note that payors and formulary committees are a sophisticated and skeptical audience who are likely to evaluate critically all such information they receive from manufacturers. Discussions around this section are continuing.

*Section 2102. Facilitating Responsible Communication of Scientific and Medical Development*

Section 2102 would require FDA to issue draft guidance, within 18 months of enactment, on facilitating the dissemination of responsible, truthful, and non-misleading scientific and medical information not included on the drug label. Discussions around this section are continuing.

### F. Subtitle G – Antibiotic Drug Development

*Section 2121. Approval of Certain Drugs for Use in a Limited Population of Patients*

Section 2121 will facilitate the development of important new antibiotics that might not otherwise have been developed. It provides a process by which developers can work with FDA on the requirements it will need to meet to make use of the limited population pathway, and creates a labeling mechanism to ensure that providers will understand that the antibiotic approved through this mechanism was found to be safe and effective only for a limited population.

### G. 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. Meetings Between CDC and Vaccine Developers*

These sections formalize the process under which the Advisory Committee on Immunization Practices (ACIP) makes vaccination scheduling recommendations to CDC, the process by which CDC reviews those recommendations and takes actions, and the process for meetings between CDC and vaccine developers.

### H. Subtitle I—Orphan Product Extensions Now/Incentives for Certain Products for Limited Populations

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

This new section would provide six months exclusivity and patent extension to a drug already on the market if it gets approval for a new indication to treat a rare disease. It is modelled on the Best Pharmaceuticals for Children Act, which has successfully incentivized the testing of drugs for pediatric indications by granting such drugs the same structure of six month extension of exclusivity and patent term.

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

This new section reauthorizes the Rare Pediatric Disease Priority Review Voucher Incentive Program (PRV Program) until 2022. The PRV program was created under section 908 of the Food and Drug Administration Safety and Innovation Act (FDASIA) in 2012. Under the program, a drug company that receives approval of an eligible rare pediatric disease product application can be granted a voucher that can then be sold to another company without limitation. The voucher can be redeemed by the holder for a drug application not otherwise eligible for a priority review. FDA is required to review the holder's drug application through the priority review process rather than the standard review process.

## I. Subtitle J—Domestic Manufacturing and Export Efficiencies

*Section 2161. Grants for Studying the Process of Continuous Drug Manufacturing*

This section now specifies that there will be $5,000,000 appropriated for each of fiscal years 2016 through 2020 to carry out this section.

*Section 2162. Re-Exportation Among Members of the European Economic Area*

This section remains in brackets as we continue to vet the language.

## J. Subtitle K—Enhancing Combination Products Review

*Section 2181. Enhancing Combination Products Review*

This new section requires FDA to issue guidance, within 18 months of the law's enactment, describing the responsibilities of each FDA center regarding its review of combination products, and to review and update the guidance periodically.

## K. Subtitle M – Medical Device Regulatory Process Improvements

*Section 2221. Third-Party Quality System Assessment*

This section, which previously was only in the discussion draft as a placeholder, requires the Secretary to establish a program under which accredited persons can assess whether a medical device company's quality system can reasonably assure the safety and effectiveness of specified devices.

*Section 2225. Easing Regulatory Burdon with Respect to Certain Class I and Class II Devices*

This remains a placeholder, but we expect to have agreed language by markup.

### L. Subtitle N–Sensible Oversight for Technology Which Advances Regulatory Efficiency

*Section 2241. Health SOFTWARE*

This section provides the clarity to health software makers by distinguishing between the kinds of software subject to FDA oversight and the kinds not under FDA oversight, while ensuring that FDA retains proper authority over software posing risks to patients.

*Section 2261. Protection of Human Subjects in Research*

This section remains under discussion. The Office of Human Research Protections (OHRP) and FDA are working to come up with language that maintains human subject protections while eliminating all unnecessary redundancies between their complementary oversight regimes.

## IV. Title III—DELIVERY

*Section 3001. Interoperability*

Since 2009, the federal government has invested more than $28 billion through the HITECH Act and subsequent legislation to accelerate the development and adoption of health information technology (health IT). The HHS Office of the National Coordinator (ONC) is responsible for the administration's Health Information Technology efforts. ONC has helped guide our health system into wide use of electronic health records (EHR), but the next, necessary step is EHR interoperability. Though 94% of hospitals and 78% of doctors' offices now use certified EHRs, it is imperative that these EHRs are interoperable—that is, that they can exchange information with each other in a meaningful way.

Barriers persist in getting widespread and effective *sharing* of this electronic health information. Most of the health care system has adopted health IT, but these health IT systems are not interoperable. ONC is exploring ways to achieve nationwide interoperability of health technology, but ONC recently put out a report identifying barriers. This section would require new, stronger standards for interoperability in order for products to be certified for meaningful use. It would also institute penalties for those vendors, health information system providers, and providers that are knowingly blocking interoperability. The language in the bill is still under discussion. The Committee has agreed to continue working in a bipartisan manner with the Administration on solutions to remove these barriers and achieve nationwide interoperability.

*Section 3021. Telemedicine*

This section requires CMS to transmit information to Congress that will be crucial to making telemedicine policy, such as the populations of Medicare beneficiaries whose care would be most improved with telemedicine services, types of procedure codes and diagnoses that may be suitable for telemedicine services, and barriers that may prevent the expansion of telehealth

services. This section requires MedPAC to recommend Medicare coverage for certain telehealth services currently covered in Medicare Advantage. It also includes a sense of Congress, which will serve as a framework in future telemedicine policy actions. The Energy and Commerce Committee Bipartisan Telemedicine Member Working Group will continue to work toward a telemedicine policy after Cures, and the Committee supports these efforts.

*Section 3041. Exempting from Manufacturer Transparency Reporting Certain Transfers Used for Education Purposes*

The Physician Payments Sunshine Act requires medical product manufacturers to report any payments or other transfers of value made to physicians or teaching hospitals to CMS for publication. The purpose of this legislation was to shine "sunshine" in order to prevent conflicts of interest. There has been some concern that reporting of certain transfers of value discourages physicians from seeking continuing medical education. This section attempts to address this concern by exempting transfers of value used for education purposes.

*Section 3061. Treatment of Certain Items and Devices*

This section, on which discussions continue seeks to expand Medicare coverage for certain devices that represent improved technologies that would be covered if they were considered "durable medical equipment".

*Section 3081. Improvements in the Medicare Local Coverage Determination (LCD) Process*

Medicare covers certain treatments locally but not nationally. The local coverage determination (LCD) process is a way for these treatments to be covered earlier as LCDs require less time to process. This section would add more transparency and reporting to the LCD process.

*Section 3101. Medicare Pharmaceutical and Technology Ombudsman*

This section creates an ombudsman at CMS for pharmaceutical and device companies seeking Medicare coverage of their products.

*Section 3121. Medicare Site-of-Service Transparency*

Seniors out-of-pocket costs vary based upon the setting in which they receive care. This section requires the HHS Secretary to make a public, searchable website in which beneficiaries can compare their costs across settings.

*Section 3151. Establishing PDP Safety Program to Prevent Fraud and Abuse in Medicare Prescription Drug Plans*

As noted by MedPAC, GAO, OIG and others, there is growing concern about potential overuse and inappropriate prescribing of opioids among Medicare Part D beneficiaries. An evaluation performed by a Centers for Disease Control and Prevention expert panel found that patient review and restriction programs used in state Medicaid programs have generated savings and reduced narcotic prescriptions, abuse, and visits to multiple doctors and emergency rooms.

However, current law does not permit the use of such programs in Medicare Part D plans. Essentially, these provisions 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.

This section has been substantially revised, since the April 30, 2015 draft, to reflect stakeholder comments earlier this year on this issue through the Protecting the Integrity of Medicare Act of 2015 (PIMA). This section now mirrors PIMA language with respects to establishment of drug management programs, and definition of at-risk beneficiaries, and also includes all prior-agreed to language related to beneficiary notification, consultation with providers, and beneficiary protection of preferences. However, this section retains prior language permitting the use of drug management programs in Medicare Advantage as well as stand-alone prescription drug plans, and defines a frequently abused drug as those controlled substances that the Secretary determines to be frequently abused or diverted. Finally, this provision requests an HHS-OIG report on the effectiveness of Medicare Part D MEDICS, and a Sense of the Congress supporting the appropriate use of e-prescribing and other health information technology tools to implement this provision.

## V. BACKGROUND ON H.R. 1321, MICROBEAD-FREE WATERS ACT OF 2015

### A. Legislative Hearing

On May 1, 2015, the Subcommittee on Health held a hearing entitled *"Examining Microbeads in Cosmetic Products."* In recent years, a number of personal care products, most notably face washes and scrubs, have utilized microplastic particles, or microbeads, as exfoliants. While there is no evidence of negative health effects on users of these products, research has shown environmental impacts on water bodies from their increased use.

At the legislative hearing, testimony received was collectively supportive of the effort to phase out the use of plastic microbeads in personal care products. Several potential improvements to the bill were discussed at the hearing and are currently being negotiated by staff with the goal of finalizing changes prior to full committee markup of the bill. The bill in its current form does not impact the regulation of products classified by the FDA as over-the-counter (OTC) drug products, such as toothpaste and acne cream. General support for including regulation of OTC products, however, was expressed at the hearing.

Additional issues raised include refining the timeline for the phase out of manufacture and sale of products containing microbeads, as well as inclusion of a definition of synthetic plastic microbeads.

### B. Summary of the Bill

On March 6, 2015, Ranking Member Frank Pallone, Jr. and Chairman Fred Upton introduced H.R. 1321, the Microbead-Free Waters Act of 2015. The bill requires the Food and Drug Administration (FDA) to prohibit sale or distribution of cosmetics containing synthetic plastic microbeads beginning January 1, 2018. This outcome would be accomplished by adding

synthetic plastic microbeads to the list of adulterated cosmetics in Section 601 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 361).