Alliance
FOR AGING RESEARCH

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May 18, 2015

The Honorable Fred Upton
Chairman
U.S. House of Representatives
Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
U.S. House of Representatives
2368 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Representative DeGette,

The Alliance for Aging Research, www.agingresearch.org, is the leading non-profit organization dedicated to accelerating the pace of scientific discoveries and their application to improve the experience of aging and health. On behalf of the Alliance, we applaud your work to arrive at a bill that, if fully funded, could improve various aspects of the biomedical research and regulatory approval process. We are encouraged by recent reports of your commitment to ensuring our federal agencies have the resources they need to carry out the increased responsibilities included in the 21st Century Cures Act. We urge you to include full funding authorizations for both the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) in the bill that will be considered by the full House Energy and Commerce Committee later this week.

We are pleased to support the most recent version of the 21 Century Cures Act approved by the House Energy and Commerce Subcommittee on Health. We would like to take this opportunity to acknowledge several provisions in the bill and to share our thoughts on how this important initiative can better serve patients in need of new treatments and medical technologies.

TITLE I- DISCOVERY

Subtitle A- National Institutes of Health Funding

The Alliance for Aging Research commends the committee for reauthorizing the NIH and for including an additional $10 billion in funding over five years. As you are aware, NIH sponsors researchers in every single state, and about 80 percent of its budget goes to fund 300,000 researchers around the world. The agency also supports training programs to increase the country's research capacity, employs about 6,000 scientists at its own labs and runs the world's largest hospital completely dedicated to clinical research. Since the doubling of NIH's base budget between fiscal years 1998-2003, the agency's funding has stagnated at around $30 billion

and is losing ground to inflation, particularly to the high rate of medical inflation. The deliberative process you have undertaken to arrive at the Innovation Fund has helped rectify this problem.

The mandatory funding the Innovation Fund provides, coupled with meaningful year over year growth in the Institutes' baseline budget, will make a profoundly positive difference in the discovery landscape. This will push the boundaries of knowledge wider to reveal new targets for the development and delivery of transformative drugs, biologics and medical devices. The Alliance urges you to continue to champion the NIH Innovation Fund.

Subtitle B—National Institutes of Health Planning and Administration

Section 1021 would require the Director of the NIH to develop a 5-year "biomedical research strategic investment plan" to make funding allocation decisions. We support the committee's interest in identifying strategic focus areas that consider "the return on investment to the United States public."

We would encourage the committee to extend further in its language and to ask the NIH to specifically consider costs to public healthcare programs (i.e. Medicare and Medicaid) as part of its return on investment and subsequent prioritization for research investment in specific conditions. For example, the costs of care for Alzheimer's disease are enormous—in 2015 Alzheimer's disease and other dementias will cost the nation $226 billion, with Medicare and Medicaid paying 68 percent of the costs. Without a treatment, costs are projected to increase to more than $1.1 trillion in 2050. Yet, federal funding for Alzheimer's disease and related dementias lags behind investment in other major disease areas by a factor of two to six.

Economic burden on public healthcare programs is not currently considered as part of the existing strategic planning process at NIH. An April 2014 GAO study found that the five selected ICs—awarding the largest amount of research funding—that it reviewed did so considering similar factors and using various priority-setting approaches. In priority setting, IC officials reported taking into consideration scientific needs and opportunities, gaps in funded research, the burden of disease in a population, and public health need, such as an emerging public health threat like influenza that needs to be addressed.

Section 1022 would create a five-year term for each institute and center director at the NIH (up from four years in the original bill draft). The Alliance opposes this provision and supports its removal. Currently, the directorships of NIH Institutes and Centers (i.e., other than that of the National Cancer Institute, which is appointed by the President under the 1971 National Cancer Act) are filled by the NIH Director. These directorships 1) do not require a Presidential appointment or Congressional approval and 2) do not have terms for their appointment. We believe that the introduction of terms will distract directors with campaigning and will encourage jockeying among colleagues who should be spending their time managing research programs. The NIH Director is already allowed to hire and fire, and IC Directors positions should not be further politicized.

In Section 1022, we appreciate the changes made to the R-series grant review process and support the inclusion of this language in the final bill.

Subtitle C-Supporting Young Emerging Scientists

As in previous comments on 21st Century Cures Act, the Alliance supports mechanisms that encourage careers in medical research. We appreciate the changes made to this bill from previous drafts that adjust loan repayment terms for young emerging scientists as opposed to redirecting funds from the Public Health Service Evaluation Set Aside, also known as the "evaluation tap."

TITLE II-DEVELOPMENT

Subtitle A- Patient-Focused Drug Development

The Alliance agrees that there is a need to develop and use patient experience data to improve the drug development process and to enhance structured risk-benefit assessments. We appreciate that the current bill requires a more scientific and systematic approach to gathering patient experience data. Thank you for defining what individuals and groups are intended to collect this data. The definition clarifies what was meant by an "entity" in the previous draft bill. We support patients, caregivers, patient advocacy groups, and members of the scientific and medical research communities being acknowledged as equal agents capable of conducting this type of research.

We still feel that the loosely structured FDA Patient-Focused Drug Development meetings, established during the fifth reauthorization of the Prescription Drug User Fee Act, have resulted in valuable resources on anecdotal experience that help inform new endpoint development, outcome measure selection in clinical trials, and benefit-risk decision making by regulators. To better contextualize the entirety of patient's experiences with a disease, the Alliance believes that information on anecdotal experience should still be permitted, either in conjunction with or without the patient experience data framework established by Section 2001. It may be worth refining this section to state that the proposed structured framework is intended to compliment information gathered through unstructured interactions with patients, their caregivers and patient advocacy groups.

Subtitle B-Qualification and Use of Drug Development Tools

The Alliance supports the authorization of $10 million annually, from fiscal year 2016-2020, to support the qualification and use of drug development tools. As you know, FDA established a process several years ago through which drug development tools like biomarkers, outcome assessments and other endpoints could be qualified for a specific use and then incorporated into clinical trials. Section 2041 intends to build on the existing qualification process. Through this process a company, group of companies or other organization could opt to work with regulators in a collaborative fashion to reduce the cost of developing these tools individually and produce a tool that once qualified became publicly available. This process has been slow to result in qualified tools due in part to the slow pace of science and a lack of resources available at the

FDA. Section 2014 intends to enhance the existing process for qualification and alleviate one of the two main factors for the resulting delay in qualification.

We would like to note that even with an enhanced qualification process in place, there is still the ability for a company, group of companies or other organization to talk directly with the FDA's medical product review divisions on the use of unqualified biomarkers and unqualified endpoints in specific clinical trials. FDA frequently approves the use of unqualified biomarkers and endpoints in trials and unqualified tools served as the basis of many drug approvals. The FDA's Office of New Drugs, medical product review divisions, and the Study Endpoints and Labeling Division should retain the flexibility to decide on the appropriate use of unqualified drug development tools for the purposes of expediting clinical trials.

Subtitle D-Modern Trial Design and Evidence Development

Should funding authorization accompany Section 2061, the Alliance supports the proposed FDA public meeting on broader application of Bayesian statistics and adaptive trial designs. Such a meeting including diverse stakeholders will help to foster a dialogue on the importance of more modern clinical trial infrastructure and uncover possible limitations to incorporating these methods in clinical trials for specific diseases. This would also provide a venue for discussing opportunities for additional research on how best to pursue future directions for adaptive clinical trials. We understand the desire for final guidance in this area; however FDA should have the option to operate under draft guidance, particularly if there is a lack of consensus on the best path forward following the public meeting. We support the call for FDA to update its draft guidance but we suggest removing the requirement to finalize guidance within 18 months.

Thank you for the changes made to the bill from previous drafts on the issue of utilizing evidence from clinical experience to support regulatory decisions pre- and post- approval. We were pleased to see that Section 2062 now calls for the development of a draft framework identifying available sources of clinical experience data, gaps in current data collection activities, current standards and methodologies for clinical experience data collection, and opportunities for the development of pilot programs. We support this moderate approach and believe that it will allow FDA to play a constructive role in ensuring that a future program incorporating real world, clinical experience evidence is well-designed.

Subtitle G-Antibiotic Drug Development

We support sections 2121-2123 for the purposes of addressing the growing crisis of antibiotic resistance in this country. We feel that these sections provide a pathway for needed antibacterial and antifungal drugs to be approved by the FDA for use in limited populations of patients who are vulnerable and unresponsive to other treatments. We know that because older adults are most likely to contract resistant infections, they will benefit from these provisions. We appreciate the added requirements in the bill for monitoring the use of these products and the increased emphasis on making information publicly available regarding trends in resistance and ensuring appropriate stewardship. Safeguards like these can reduce inappropriate off-label use.

Subtitle L-Priority Review for Breakthrough Devices

We believe timely access to cutting-edge medical devices is as important to the health and independence of the older population as access to new drugs and biologics. We continue to support Section 2201 which would establish a priority review process for medical devices. We continue to support the establishment of this process because. FDA's Center for Devices and Radiological Health (CDRH) has already taken steps to create the structure for a voluntary program that would expedite access for pre-market approval of devices intended for life-threatening illnesses and for areas of unmet need. We are encouraged that the proposed priority review process in Section 2201 builds upon the work done by CDRH to increase flexibility in device approvals and enhance the level of communication with the developers of medical devices. Since the amount of resources necessary to conduct expedited reviews of new products will increase, we believe that additional authorization of funding is necessary and should be included in the final 21st Century Cures Act.

Subtitle P-Improving Scientific Expertise and Outreach at FDA

We are gratified that you and your colleagues agree that for the FDA to be effective it must be populated with highly capable staff that is constantly up to date on new scientific knowledge and developments. The Alliance fully supports inclusion of Section 2281 that addresses FDA's ability to hire and retain qualified scientific and technical experts. This section will allow the FDA to more quickly recruit professionals in the field of engineering, bioinformatics and other emerging fields so that they are able to keep pace with innovation in the private sector. It will also ensure that FDA can provide competitive wages for employees with highly specialized skills. We appreciate that Section 2281 now includes language promoting FDA participation in and sponsorship of scientific conferences and meetings.

TITLE III-DELIVERY

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

The Alliance opposes the inclusion of Section 3141 in the final version of the bill. We do not believe Section 3141 provides a balanced approach toward addressing the problem of prescription drug abuse in the United States.

Around 100 million Americans live with persistent pain - more Americans than those who are affected by diabetes, heart disease, and cancer combined. Surgery is a common cause of persistent pain. According to the National Hospital Discharge Survey, adults age 65 and older are 2.6 times more likely to have surgery than those ages 45-64. The Centers for Disease Control and Prevention found that around half of adults age 65 and older have been diagnosed with arthritis, another common cause of persistent pain. Section 3141 of this bill has the potential to inappropriately label legitimate pain suffers as at-risk of abuse and limit their access to needed pain relief by restricting where they can fill their prescriptions. This section should not be included in a bill aimed at providing need treatments and technologies to patients in need.

Chairman Upton and Congresswoman DeGette, thank you for your leadership on behalf of patients. We look forward to continuing our support for your efforts as the 21st Century Cures Act advances. If you have any questions or would like additional information, please do not hesitate to contact us at (202) 293-2856 or via email (speschin@agingresearch.org and cbens@agingresearch.org).

Sincerely,

Susan Peschin, MHS
President & CEO

Cynthia Bens
Vice President, Public Policy