ACT-AD
Accelerate Cure/Treatments for Alzheimer's Disease

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 coalition to Accelerate Cure/Treatments for Alzheimer's Disease (ACT-AD) is comprised of more than 50 national organizations representing patients, caregivers, researchers, health professionals, and other health advocates. For the past ten years we have supported efforts to expedite the development, review, and approval of transformational therapies for Alzheimer's disease (AD). On behalf of ACT-AD, thank you for the opportunity to provide feedback on the 21st Century Cures Act that was approved by the House Energy and Commerce Subcommittee on Health on May14, 2015. We applaud your work to arrive at a bill that if fully funded could improve various aspects of the biomedical research and regulatory approval processes.

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 and we urge you to include full funding authorizations for 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 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 II-DEVELOPMENT

Subtitle A- Patient-Focused Drug Development

We agree 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, we believe 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

ACT-AD 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, ACT-AD 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 drat 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 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. ACT-AD 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.

Chairman Upton and Congresswoman DeGette, thank you for your leadership on behalf of patients. Please feel free to contact me at (202) 293-2856 with any questions.

Sincerely,

Cynthia Bens
Executive Director