

**Public Policy Division**  
1212 New York Ave NW  
Suite 800  
Washington, DC 20005

202.393.7737 **p**  
866.865.0270 **f**  
www.alz.org



May 12, 2015

The Honorable Fred Upton  
Chairman  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

The Honorable Frank Pallone  
Ranking Member  
House Energy and Commerce Committee  
2125 Rayburn House Office Building  
Washington, DC 20515

Re: 21st Century Cures Act

Dear Chairman Upton and Ranking Member Pallone:

The Alzheimer's Association appreciates the opportunity to comment on the latest draft of the 21st Century Cures Act and applauds you both for your leadership in furthering the 21st Century Cures Initiative. The Association also recognizes the many Representatives who have contributed to this bill and are grateful for the opportunity to provide feedback.

Founded in 1980, the Alzheimer's Association is the world's leading voluntary health organization in Alzheimer's care, support and research. Our mission is to eliminate Alzheimer's disease and other dementias through the advancement of research, and as the world's largest nonprofit funder of Alzheimer's research, the Association is committed to accelerating progress of new treatments, preventions and, ultimately, a cure. Through our funded projects and partnerships, we have been part of every major research advancement over the past 30 years.

No single organization can surmount a challenge as great as Alzheimer's. To help achieve our vision of a world without Alzheimer's, the Association partners with key government, industry and academic stakeholders in the global race to end Alzheimer's. We believe in the value of collaboration and work toward the day when we will have disease-modifying treatments, preventive strategies and gold-standard care for all people affected by Alzheimer's disease.

### **Promoting Patient and Caregiver Engagement in Drug Development**

The Association applauds the Committee for maintaining the provision Patient Focused Drug Development (PFDD; TITLE III: SUBTITLE A) in the bill. The Association agrees that it is crucial to include the patient perspective in such areas as risks and benefits, targeted endpoints, and meaningful outcomes, and thus supports the enhancement of the PFDD program. With a disease like Alzheimer's, it is important to also include the perspective of care partners as well as the individual with the disease. The Association looks forward to working with the Food and Drug Administration (FDA) through the public comment period and at the public workshop on this important topic.

### **Clinical Trial Modernization**

The National Plan calls for the National Institutes of Health (NIH) to identify ways to compress the time between target identification and release of pharmacological treatments. There is evidence that a single Institutional Review Board (IRB) for multi-site studies can lead to enhanced protections for patients through increased accountability, a decrease in conflicts of interest, and improved efficiency through a refocusing of resources. These benefits plus the acceleration of the pace of research is particularly important to individuals affected by Alzheimer's disease and other dementias.

The Alzheimer's research community overwhelmingly supports the concept of a centralized IRB (TITLE II: SUBTITLE N), as have participants in several expert think tank and strategy meetings, including the 2012 Alzheimer's Disease Research Summit and meetings of the Advisory Council on Alzheimer's Research, Care, and Services. Additionally, the Association supports the inclusion of modern trial design and evidence development (TITLE II: SUBTITLE D).

### **Data Sharing**

Establishing a 21st century data sharing framework for public research will help accelerate the development of new medical technologies and advance breakthroughs (TITLE I: SUBTITLE F). The Association has developed the Global Alzheimer's Association Interactive Network (GAAIN) to provide researchers around the globe with access to a vast repository of Alzheimer's research data. GAAIN is a global hub for AD research data that allows researchers to search across multiple data sources instantly and contact these data partners directly for data.

GAAIN aggregates information about our partners' data and shares with researchers without infringing upon data partner data sharing policies and regulations. Data partners always remain in control of their data. It is the first global big data initiative in Alzheimer's disease research and serves as a benchmark for computational research in other complex diseases. The Association supports efforts to facilitate data sharing and hopes the Committee will look to GAAIN as a successful example

### **Validation and Qualification of Biomarkers**

Identifying additional partnership opportunities with the private sector and facilitating collaborative efforts to enhance identification of risk factors and early biomarkers is a key action item in the National Plan to Address Alzheimer's Disease (National Plan). The surrogate endpoint qualification and utilization that was included in the discussion draft, (TITLE I: SUBTITLE B), not only would have established a predictable, transparent process for FDA's consideration and qualification of endpoints, but also allows FDA to use private-public partnerships to qualify other types of biomarkers.

The Association is disappointed that this section did not make it into the draft as this initiative mirrors efforts by the Association that have been called upon by the National Plan. Since 2005, the Association has partnered with the National Institute on Aging, the National Institute of Bioimaging and Bioengineering, the National Institute of Mental Health, the National Institute of Neurological Disorders and Stroke, the National Institute of Nursing Research and the National Institute on Drug Abuse on the Alzheimer's Disease Neuroimaging Initiative (ADNI.) ADNI seeks to find more sensitive and accurate methods to detect Alzheimer's disease at earlier stages and mark its progress through biomarkers. Partnerships like ADNI have made significant inroads into this complex disease and the Association supports these efforts by the Committee.

The Association appreciates the steadfast support of the Committee and the great endeavor in which they are engaged. We look forward to continuing to work with the Committee in order to address the Alzheimer's crisis and hope that the Association will be called upon for our expertise in this area. If you have any questions or need further information please contact Rachel Conant at [rconant@alz.org](mailto:rconant@alz.org) or 202-638-7121.

Sincerely,



Robert Egge  
Executive Vice President, Government Affairs