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

June 15, 2012

The Honorable Tom Harkin
Chairman
Committee on Health, Education Labor and Pensions
United States Senate
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Michael Enzi
Ranking Member
Committee on Health, Education, Labor and Pensions
United States Senate
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Fred Upton
Chairman
Committee on Energy & Commerce
United States Congress
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Henry Waxman
Ranking Member
Committee on Energy & Commerce
United States Congress
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairmen Harkin & Upton and Ranking Members Enzi & Waxman:

As you and your colleagues finalize the Food and Drug Administration (FDA) user fee legislation, we are writing to thank you for your effective work to reach bipartisan consensus on a bill that will ensure a modern approach to the review of new products and allow for more timely patient access. Accelerate Cure/Treatments for Alzheimer's Disease (ACT-AD) is a coalition of more than 50 not-for-profit groups. Since 2010, ACT-AD has been represented at various stages of the negotiations to reauthorize the FDA user fee programs and we are pleased with the inclusion of a number of provisions to help spur the development of therapies for Alzheimer's disease.

Enhancements that were incorporated into the PDUFA V agreement that we support include- accelerating drug development through greater focus on regulatory science; supporting the development of innovative clinical trial designs; re-evaluating how the FDA assesses benefits and risks of therapies and communicates benefit-risk information; and ensuring that Risk Evaluation and Mitigation Strategies (REMS) do not serve as a barrier to patients in need of treatments.

We also believe several other provisions included in H.R. 5651, the *Food and Drug Administration Reform Act of 2012* and S. 3187, the *Food and Drug Administration Safety & Innovation Act of 2012* warrant our continued support.

"Breakthrough Therapy" Designation

Currently what constitutes success to the FDA in slowing or preventing the progression of Alzheimer's disease is unclear, and safety barriers imposed by the Agency in the pre-market space for the approval of new treatments for a broad patient population are high. The "Breakthrough" designation supported in the House and Senate provides the FDA with a new approach to development programs for very promising therapies. Their approach could include the use of a variety of innovative clinical trial designs that are flexible enough to shorten or combine traditional phases of drug development for patients who are highly likely to respond to a treatment and establish specific strategies for accumulating further evidence on the breakthrough drug after it is approved.

Accelerated Patient Access

Since 2006, much of ACT-AD's work has focused on how to more efficiently design and conduct clinical trials. Topics ranging from identifying patients at the earliest stage of disease through biomarkers; demonstrating a meaningful treatment response; and using novel endpoints that lead to successful drug approval in Alzheimer's have been covered during meetings hosted by ACT-AD. Our conversations with the FDA on these topics have been tremendously productive, but the need for such discussions stemmed from ambiguity in how the FDA would assert its authority to apply expedited approval mechanisms, such as Accelerated Approval, in the area of Alzheimer's disease. The user fee legislation codifies that the Agency should encourage the use of innovative methods for designing trials and directs the FDA to utilize an expedited approval pathway for a wider range of diseases when it is appropriate. There is a great need to shorten the development and approval times for Alzheimer's treatments and the these provisions may provide a higher degree of certainty to those interested in developing better symptomatic treatments or truly disease-modifying therapies.

Reauthorization of the Critical Path Public-Private Partnerships

Public-private partnerships provide an opportunity to overcome many of the challenges associated with taking a basic scientific discovery through development and regulatory approval of a medical product. Partnerships between the private sector, regulatory and other government agencies, academic institutions, nonprofit organizations, and patient groups represent a new model offering innovation and efficiencies in drug development. Many public-private partnerships exist around Alzheimer's disease and have helped to advance barriers to research and drug development. Extending FDA's participation in these public-private partnerships is critical.

The pending user fee legislation will speed the approval of much-needed therapies and cures for patients who are facing serious and life-threatening conditions like Alzheimer's disease. This important legislation will help FDA maintain high standards for approval while at the same time ensuring that the agency can help facilitate the delivery of novel therapies to patients in a more timely manner. We appreciate your leadership and we urge swift passage of the bill by Congress. If you have any questions or require additional information, please contact Cynthia Bens at cbens@agingresearch.org or (202) 293-2856.

Sincerely,

Daniel Perry
Chairman