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

August 26, 2013

The Honorable Paul Ryan
Chair
House Budget Committee
207 Cannon House Office Building
Washington, D.C. 20515

The Honorable Chris Van Hollen
Ranking Member
House Budget Committee
207 Cannon House Office Building
Washington, D.C. 20515

Dear Chairman Ryan and Ranking Member Van Hollen,

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. Our mission is to support efforts to expedite the development, review, and approval of transformational therapies for Alzheimer's disease (AD). ACT-AD thanks your colleagues in the House for introducing H.R. 2725, *the Food and Drug Administration Safety Over Sequestration Act,* and on behalf of the coalition, I would like to urge prompt consideration of this legislation by the committee.

As you are well aware, approximately 5.2 million Americans currently live with Alzheimer's disease. By the middle of this century that number is expected to increase to 7.1 million. Without more meaningful treatment options that allow for improved quality of life for those with the disease or interventions that halt, delay or reverse progression of AD in its earliest stages, the human and economic burdens associated with this disease will continue to advance at unsustainable rates. In recent years several late-phase therapeutic development programs for Alzheimer's disease were discontinued due to marginal or negative results. In response to challenges faced in these programs the FDA became a more active partner in the development process for Alzheimer's disease by issuing draft guidance for industry further clarifying requirements for testing early-stage Alzheimer's treatments, expressing the conditions under which they would consider the use of Accelerated Approval for an AD treatment, and routinely participating in meetings and conferences with patient advocates, the scientific community, and industry focused on improving AD clinical trials.

User fees directly fund drug, device and biologic review activities so that new interventions can be made available to patients in a timely and predictable manner. ACT-AD was represented at various stages of the most recent round of negotiations to reauthorize FDA's user fee programs beginning in 2010 and we were pleased to see the inclusion of a number of provisions that could be paid for with user fees to help spur the successful development of therapies for diseases like Alzheimer's disease. Enhancements that were incorporated into the PDUFA V and MDUFA III agreements that we supported include-accelerating drug and device development through greater focus on regulatory science; supporting the development of innovative clinical trial designs involving biomarkers and reliable patient reported outcome tools; re-evaluating how the FDA assesses benefits and risks of interventions; and ensuring that Risk

Strategies (REMS) do not serve as a barrier to patients in need of treatments. The FDA has been trying to maintain their direct review activities and pursue many of these new initiatives without collecting their full fees this year. While a noble pursuit, there have been sacrifices made to move forward with new initiatives relying on budget authority (based on a FY 2011 funding level) and in the complete absence of funds in some cases. Such sacrifices will not bode well for the agency in the future.

Due to budget constraints, FDA has not been able to pay out performance awards this year for employees who meet or exceed their goals. This elimination of awards is on top of a pay freeze that has been in effect for two years. FDA travel to scientific conferences has been dramatically limited in the number of representatives that can be sent, if they can be sent at all, how long FDA representatives can stay, and how many conferences can be attended each year. These conferences provide the best opportunities for FDA reviewers to learn about current and emerging areas of science. Without access to the same scientific information reviewers are not able to operate from the same knowledge base as those in the private sector and it impacts their ability to do their jobs. Many reviewers are medical professionals. Recently these reviewers have had to use their own personal leave and finance their own continuing medical education (CME) to be able to stay current with the practice of medicine creating an added level of hardship. Finally, the breakthrough therapy designation that was established under PDUFA V was much more successful than either the FDA or outside stakeholder anticipated. The agency has received an influx of request to consider this pathway for new therapies and they have granted more than five times the number of breakthrough designation than was expected. The review of breakthrough products is much more time consuming than a typical review because there is more frequent interaction between the agency and a drug sponsor and there is a greater need for adapting as trials for these products progress. Without the expected user fees to support the additional of staff under PDUFA V and MDUFA III, the success of this designation could have unintended consequences for those therapies that must proceed through the standard review process at FDA.

Approving *the Food and Drug Administration Safety Over Sequestration Act,* to exempt FDA user fees from the sequester will begin to restore the agency's ability to meet the needs of all stakeholders it serves, particularly those patients who live with Alzheimer's disease and are in need of new treatment. Thank you for your leadership in the Senate and for your careful consideration of our views. If we can be of assistance as the committee takes action on this important bill, please contact Cynthia Bens on the coalition staff at cbens@agingresearch.org or (202) 293-2856.

Sincerely,

Daniel Perry
Chairman

CC. Rep. Leonard Lance (R-NJ)
Rep. Anna Eshoo (D-CA)
Rep. Doris Matsui (D-CA)
Rep. Mike Rogers (R-MI)