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

August 26, 2013

The Honorable Patty Murray
Chair
Senate Budget Committee
624 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Jeff Sessions
Ranking Member
Senate Budget Committee
624 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Chairwoman Murray and Ranking Member Session,

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 Senate for introducing S. 1413, *the FDA User Fee Protection 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 the 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in these programs the FDA became a more active partner in the development process for Alzheimer's disease by issuing draft guidance for industry further clarifying requirements for testing early-stage Alzheimer's treatments, expressing the conditions under which they would consider the use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 national and international meetings with patient advocates, the scientific community, and industry focused on improving AD clinical trials.

User fees are intended to 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 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 success 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 Evaluation and Mitigation 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 any of these new initiatives without collecting their full fees this year. While a noble pursuit there have been sacrifices made to move forward with them 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 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 representatives can stay, and how many conference can be attended each year. These conferences provide the best opportunities for FDA reviewers to learn about current and emerging areas of science and help to guide those involved in therapy development to incorporate the science most effectively in clinical programs. Without access to the same scientific information, reviewers are not able to operate from the same knowledge base as those in the private sector. Many reviewers are also 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.

Finally, the breakthrough therapy designation that was established under PDUFA V was much more successful than either the Agency 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 process through the typical review process at FDA.

Approving *the FDA User Fee Protection Act* to exempt FDA user fees from the sequester will begin to restore the agency's ability to meet the needs of all of the 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 expressed above. 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. Senator Mark Pryor (D-AR)
Senator Roy Blunt (R-MO)
Senator Daniel Coats (R-IN)
Senator Al Franken (D-MN)
Senator Jerry Moran (R-KS)