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

May 6, 2013

Division of Dockets Management (HFA-305)
U.S. Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

RE: Docket No. FDA-2013-N-0196: Food and Drug Administration Prescription Drug User Fee Act V Benefit-Risk Plan

The coalition to Accelerate Cure/ Treatments for Alzheimer's Disease (ACT-AD), www.act-ad.org, 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 has been consistently represented in regular meetings between U.S. Food and Drug Administration (FDA), patient advocates, and consumer advocates since 2010 to advance patient-focused enhancements as part of legislation to reauthorize the prescription drug user fee program (PDUFA). We are pleased with the FDA's interest in being more inclusive of patient and caregivers views when approaching regulatory issues that matter most to patients by expanding existing avenues for receiving input (e.g. the patient representative program) and exploring new ways to solicit feedback from people touched by disease in an effort to better inform the Agency's decision-making process. The FDA's leadership is to be commended for committing to pursue their planned patient-focused drug development activities despite having user fees to support these activities sequestered under the Budget Control Act of 2011. This pledge conveys to people with diseases like Alzheimer's and their loved ones a belief that the Agency is serious about putting their interests at the forefront of drug development.

One of the activities that falls under the umbrella of the FDA's Patient-Focused Drug Development Initiative is the refinement and implementation of a more structured and transparent framework for FDA reviewers to utilize when assessing the potential benefits and risks of new treatments. Such a framework is crucial for providing more certainty to those developing products for disease like Alzheimer's disease where the consequences of the disease are severe and the unmet need for new treatments and cures is high. The structured risk-benefit framework can help sustain innovation in the CNS space where there are multiple points of uncertainty in the path to approval of a new treatment and odds are low that a treatment will ultimately make it to market.

The draft risk-benefit report released in February of 2013 describes in great detail how information from patients with multiple sclerosis (MS) on the benefits they received from a particular treatment (Tysabri) was instrumental in assisting the Agency in making a critical decision to allow that product to remain on the market despite significant health risks. While this experience is provided as a case study, the report does not describe how lessons learned from the Tysabri experience will ultimately be

translated into actions the Agency will take moving forward in making risk-benefit conversations more patient-focused. It also does not address at which points the Agency intends to engage patients in product-specific decisions on risk-benefit in the future. We strongly encourage that the final report include details on how early in the drug development and review processes patient views will now be included in conversations between the FDA's product review divisions and a drug/product sponsors, the amount of information the Agency intends to solicit from patients (e.g. broad via open public comment or narrow via the assembly of a representative sample of affected patients for their views), and the mechanisms it plans to use to garner patient and caregiver input (e.g. Federal Register Notice, face-to-face meetings, consumer-friendly web portal, etc.).

The draft report later references the role 20 disease-specific meetings will play in assisting the FDA in gaining a better understanding of the patient and caregiver experience with a particular disease, ultimately providing reviewers with more confidence in their decision-making ability. Throughout the PDUFA V negotiations FDA representatives have expressed that each center at FDA and each division within those centers were involved in choosing a disease/condition to be the focus of one of these 20 meetings. As has been stated by the patient advocacy community and was reflected in many of the 4,000 comments the FDA received in advance of selecting diseases to be the focus of these 20 meetings, the views of people suffering from ALL diseases with unmet medical need should be a priority under PDUFA V. We would like the Agency to include in the final report a section on the principles underlying these 20 meetings. Additional direction from FDA on aspects of the patient/caregiver experience that are most important to FDA reviewers can enable patient organizations like the ACT-AD and its members to stay engaged in FDA's patient-focused drug development activities and serve as more effective conduits between the review division, patients and their families. With more guidance, the Alzheimer's patient and caregiver communities could establish reliable communications tools to provide useful input to the Agency.

We understand that decision-making on the risks and benefits of new treatments is not always straightforward, particularly for Alzheimer's and other neurodegenerative diseases where the potential benefits of new treatments are likely to occur prior to the onset of manifest symptoms. However, we do believe that Alzheimer's patients and their caregivers can make significant contributions to assist FDA in the review process if a proper venue is made available to them. Thank you for considering these views. If you have any questions or require additional information prior to finalizing the report, please contact Cynthia Bens on the coalition staff at cbens@agingresearch.org or (202) 293-2856.

Sincerely,

Daniel Perry
Chairman