

Policy Forum

Advancing Alzheimer's disease drug review as a national priority

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Abstract

The aging of the baby boom generation continues to put more Americans at increased risk for Alzheimer's disease (AD). The need for meaningful treatment options to fight the destruction caused by AD has never been greater. This article highlights the pivotal role that the U.S. Food and Drug Administration will play in making the review of emerging AD therapies a national priority and ways that various stakeholders are engaging with regulators to meet the challenges posed by the growing AD epidemic.

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1. Overview

The aging of the baby boom generation continues to put more Americans at increased risk for Alzheimer's disease (AD), with 5.2 million people affected today and an estimated 615,000 new cases each year by 2030 [1–3]. This represents one of the greatest public health crises of our time and threatens the nation on many fronts: robbing Americans of quality of life and productivity, overburdening families with caregiving responsibilities, and devastating our healthcare system. The need for meaningful treatment options to fight the destruction of AD has never been greater, and that need continues to grow more urgent each day.

Today, nearly two dozen compounds that might slow, stop, reverse, or even prevent AD are in late-stage development [4]. This new generation of therapies designed to modify the disease might represent significant advances over currently available therapies, which only temporarily relieve some symptoms. However, the effective review of disease-modifying therapies will require significant AD expertise within the U.S. Food and Drug Administration (FDA) and standards of evaluation that better reflect the nature of these novel therapies.

The FDA is in a pivotal position to meet the challenges posed by the growing AD epidemic and has already begun to reexamine its approach to promising new therapies. This

represents an important opportunity for the FDA to demonstrate the same urgency and responsiveness to patients, caregivers, families, physicians, and the nation as a whole that it showed in the face of similar public health crises like human immunodeficiency virus/acquired immune deficiency syndrome and cancer. Improving the drug review process for AD will require continued collaboration between the FDA, industry, government, and other AD stakeholders.

2. The Accelerate Cure/Treatments for Alzheimer's Disease coalition

Established in 2006, Accelerate Cure/Treatments for Alzheimer's Disease (ACT-AD) is a coalition of national organizations committed to bringing interventional therapies to patients, providers, and families in the next decade by making the review of promising AD therapies a top national priority. This commitment has guided ACT-AD's efforts to collaborate with the FDA on specific reform, including three projects that were initiated by the agency in direct response to the coalition's advocacy.

First, FDA Intra-Agency Neurology Working Group, an internal agency working group, brings together FDA experts involved in the regulation of drugs, biologics, and medical devices, with the goal of providing more consistent review. Second, the FDA's Patient Consultant Program was expanded at the urging of ACT-AD to be applied to AD and assure the participation of patients and caregivers in the FDA's review of new treatments. Third, the first in a series

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of AD Ally/FDA Workshops has been coordinated by ACT-AD, and co-hosted by the Alzheimer's Association and the Alzheimer's Study Group, to focus on specific clinical issues involved in regulatory review of AD. The initial workshop was held on March 13, 2008 and brought together leading scientists, regulators, drug developers, and AD advocates to further the dialogue on clinical meaningfulness in AD and to open the door to future discussions with the FDA on AD therapies. The workshop provided clarification of current FDA standards for reviewing AD therapies, recognition on the part of the scientific community that these standards need to be reassessed to evaluate potential disease-modifying therapies, and confirmation by Dr Russell Katz, Director of the FDA's Division of Neurology Products, that the agency would be willing to accept a fair amount of risk with new AD treatment because of the severity of the disease. In addition to Dr. Katz, presenters at the workshop included Sid Gilman, MD, Department of Neurology at the University of Michigan; David Knopman, MD, Department of Neurology at the Mayo Clinic; Jeffrey Cummings, MD, Director of the AD Disease Center at the University of California, Los Angeles; and Howard Fillit, MD, Executive Director of the AD Drug Discovery Foundation.

3. Meaningful therapies for AD patients now

The progress made to date by the FDA is promising, but the urgent threat posed by AD to aging Americans, their families, and the nation's healthcare infrastructure overall makes continued change in AD drug review a national priority.

ACT-AD will continue to collaborate with the FDA to engage key AD allies, including the Alzheimer's Association and the Alzheimer's Study Group, and the FDA in a scientific discussion about the disease, the critical need for new treatments, and regulatory issues involved in the development and review of potential AD therapies. Furthermore, to help ensure that the FDA has the resources to accelerate the review of meaningful AD treatments and to pursue other important initiatives, ACT-AD will encourage its organizational members to actively support increased federal appropriations for the agency.

Toward the goal of making the review of AD therapies a national priority, the coalition will continue to engage in the following activities:

- AD Ally/FDA Workshops: The coalition will work with the agency and AD experts to convene additional sessions to address specific issues related to clinical development in AD and ongoing drug review. Academic and clinical experts in AD have suggested topics for future workshops, including the use of biomarkers in clinical trials, standards for disease modification, and a follow-up session on clinical meaningfulness.
- FDA Intra-Agency Neurology Working Group: Although the AD advocacy community awaits a mission statement from this working group, ACT-AD will work with group leaders in their ongoing efforts to modernize agency-wide review of neurology therapies and to stress the need for a proactive response from the agency in addressing challenges posed by the coming wave of new AD therapies. ACT-AD would also encourage and support decisions made by FDA leadership to consolidate the review of AD therapies within a single division focused on AD.
- AD expertise at the agency: The coalition will collaborate with the FDA leadership to enhance AD expertise at the agency and to build direct AD experience among in-line reviewers at the Center for Biologics Evaluation and Research.
- FDA Patient Consultant Program: ACT-AD will continue to work with member organizations and the agency to identify AD patients and caregivers to participate in the FDA review of new therapies to treat and possibly prevent the disease.

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