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FOR AGING RESEARCH

BIOCOM and Congressional Life Science Caucus

Precision Medicine Briefing
Capitol Hill
June 22, 2016

Cynthia A. Bens, Vice President of Public Policy

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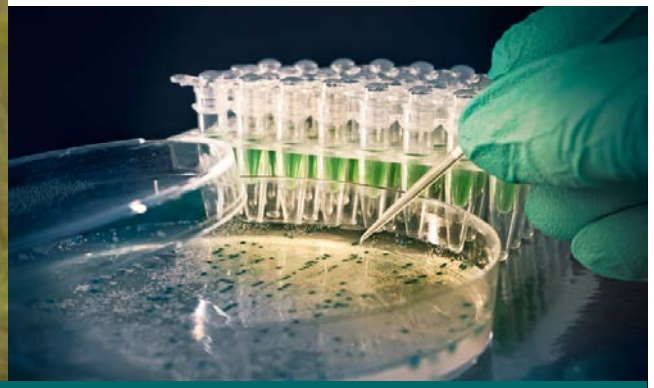

WHO WE ARE

The Alliance for Aging Research is the leading non-profit organization dedicated to accelerating the pace of scientific discoveries and their application in order to vastly improve the universal human experience of aging and health.

www.agingresearch.org

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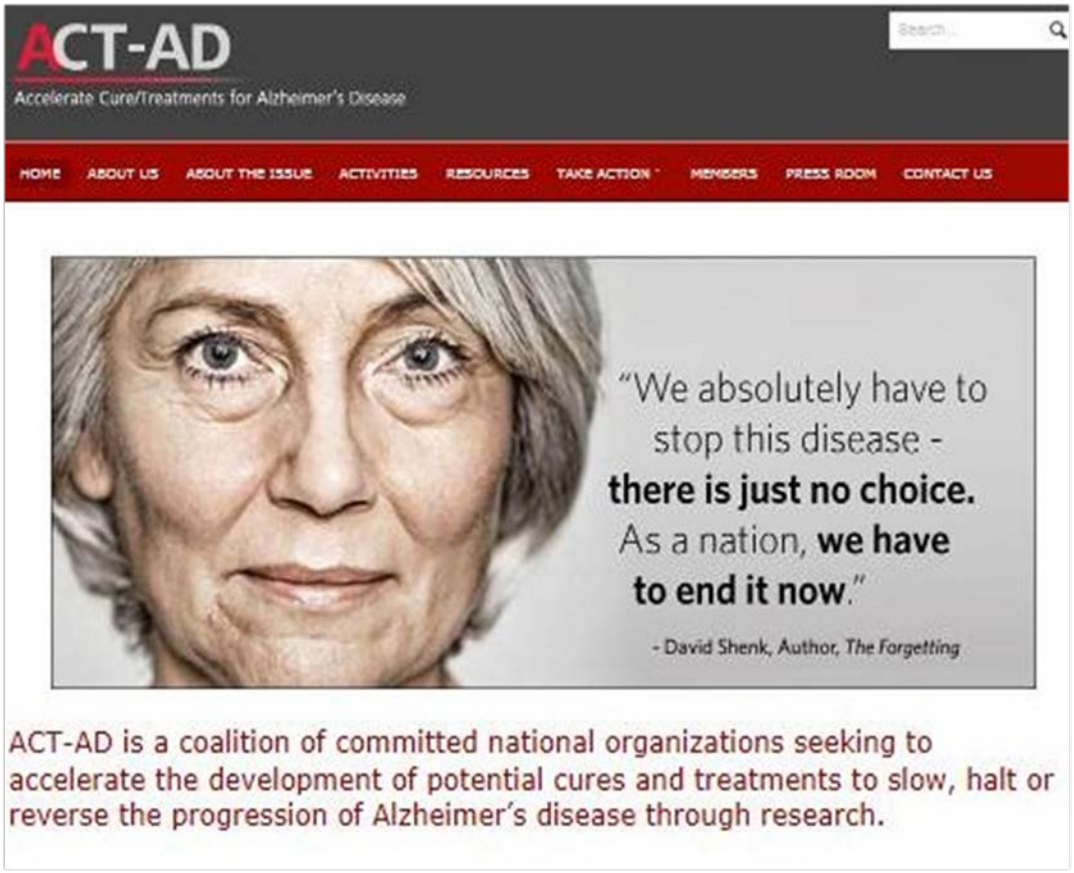
Alzheimer's Disease

- Progressive, fatal form of dementia that causes memory loss, cognitive impairment, and behavioral problems
- Affects more than 5 million Americans
- Devastating to patients and their families, including the very high cost of care
- Number of people with the disease is expected to triple over the next three decades
- Finding effective treatments is an urgent public health goal



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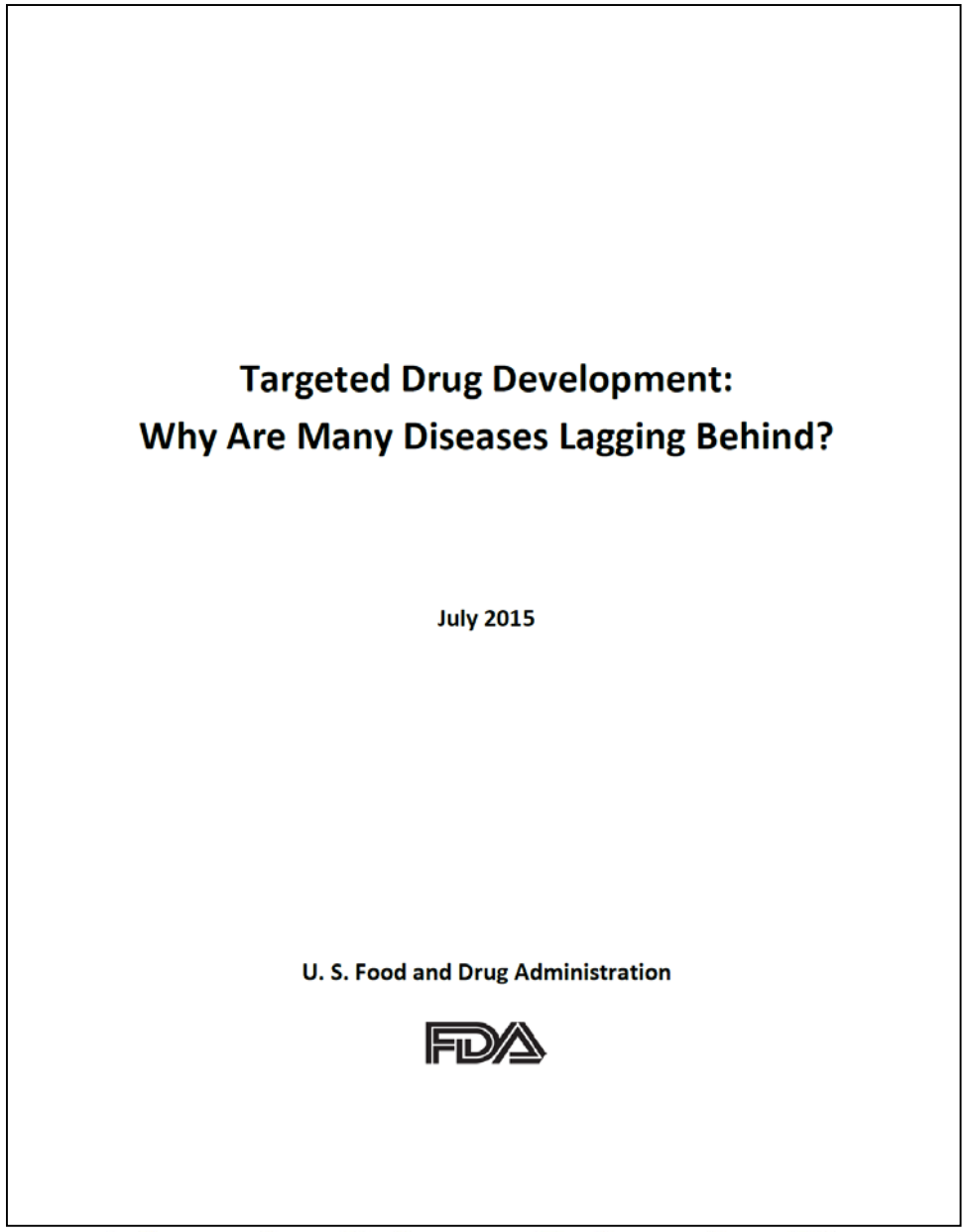


FDA's Assessment of Targeted Drug Development for AD

Scientific knowledge is in its infancy

Diagnostic biomarkers = No

Prognostic biomarkers=No

Theranostic biomarkers=No

Effective therapy is FDA priority=Yes!

Avaliable at: http://www.fda.gov/aboutfda/reportsmanualsforms/reports/ucm454955.htm

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

8th Annual FDA/Alzheimer's Disease Allies Meeting

Assessing the Scientific Foundation for Alzheimer's Disease Therapeutic Development

Marriott Hotel & Conference Center
5701 Marinelli Road
Bethesda, MD 20852

September 16, 2015

Meeting Objectives

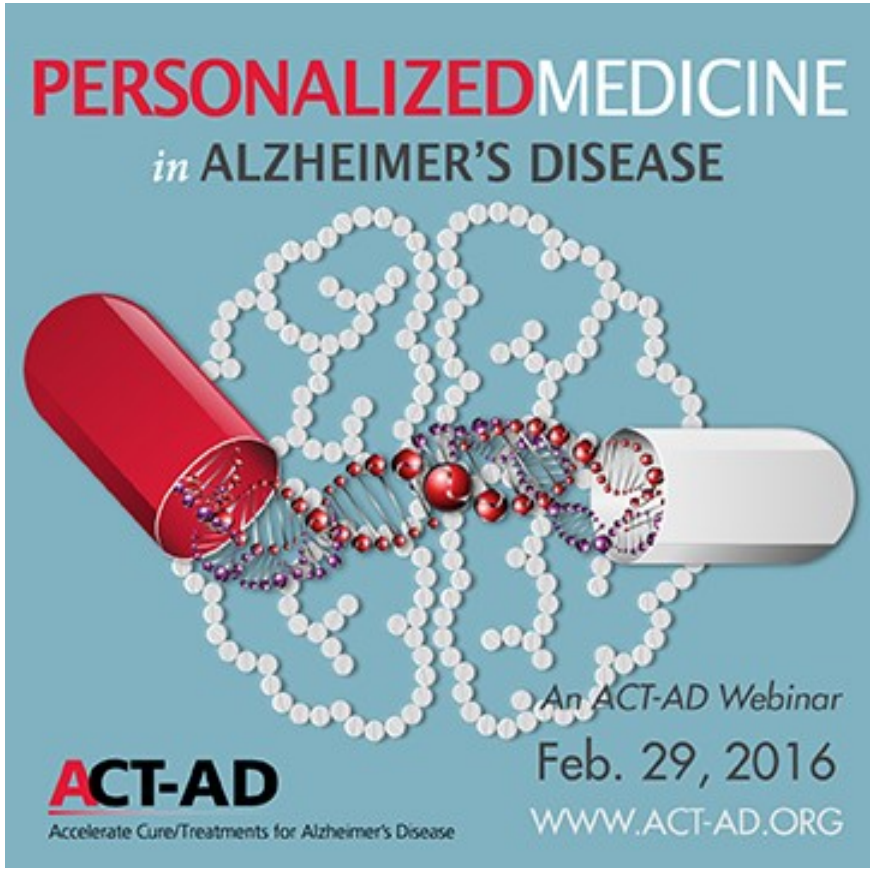
- Examine lessons from pioneering studies that incorporated Alzheimer's disease biomarkers and surrogate endpoints
- Explore how genetics and processes like neuroprotection, immunity, metabolism, and inflammation are changing the conceptualization of Alzheimer's disease
- Improve prospects for success by focusing on target validation, disease models, endpoint selection and effect size

Jason H. Karlawish, M.D.
Professor of Medicine, Division of Geriatrics
Penn Medicine

J. Michael Ryan, M.D.
Vice President, Neurodegeneration
Therapeutic Area Head
Novartis Pharmaceuticals Corp.

Cara Tenenbaum, J.D., MBA
Senior Policy Advisor, Office of the Center Director
Center for Devices and Radiological Health
U.S. Food and Drug Administration

Cynthia A. Bens
Vice President of Public Policy, Alliance for Aging Research
Executive Director, Accelerate Cure/Treatments for Alzheimer's Disease



Available at: http://act-ad.org/activities/webinar-on-personalized-medicine-and-alzheimers-disease/

Signs of Progress

- Current Phase III studies for disease-modifying treatments in early AD incorporated lessons from past trials regarding enrichment strategies and endpoints
- Prevention trials already underway testing the validity of patient's genetic profile in determining risk and accuracy of imaging biomarkers in demonstrating treatment efficacy
- Increased funding for research focused on validating biomarkers and interrogating alternative targets for drug development
- Regulators open to considering expedited approval pathways once evidence supports that a biomarker change is reasonably likely to predict clinical benefit

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Thank you for your attention.
Questions? Comments?

Cynthia Bens, Vice President of Public Policy
Email: cbens@agingresearch.org
Phone: 202-688-1230