

Alzheimer's Disease

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Urgency/Background

There is unprecedented urgency and unmet medical need in Alzheimer's disease (AD). AD is the sixth-leading cause of death in the United States and is the only leading cause of death without any means to effectively prevent, cure or even slow progression. More than 5 million Americans are living with Alzheimer's disease. Without the development of important medical breakthroughs, by 2050, the number of people with Alzheimer's disease could reach 13.8 million. The human suffering of both patients and caregivers, and the economic burden on the nation's health care system and government programs, is enormous. With the baby boomers reaching the age of elevated risk, we are running out of time. This is a crisis. We need to take action and do what we can to advance AD research... to discover the effective treatments that we want and need. Statistical urgency?

Proposal

The proposal is to form a Scientific Working Group (SWG) for the advancement of AD treatments led by statisticians from industry, academic and government sectors. The focus of this SWG will be research collaboration to develop analytical approaches in relevant areas of AD, with the primary goals of advancing the understanding of the disease and enabling the next generation of breakthrough treatments. The Working Group will initially focus on specific topics that are currently of great importance and interest to the AD research and medical product development communities. For example, as the AD pharmaceutical development field is moving away from symptomatic treatments and focusing more on disease modification, how can we do a better job of demonstrating these disease-modifying effects? Currently, there is limited research and publication on how to define and demonstrate AD disease modification. Concerted effort on this topic will potentially provide guidelines for future clinical trial design and regulatory evidence for decisions on new disease-modifying drugs. This will likely have wide-reaching impacts beyond Alzheimer's and will be potentially applicable to a number of other neurodegenerative diseases. Other examples of areas within AD that may have the potential to influence and improve AD research are listed below under "Topics." As AD is an evolving field of research and development, there will be many opportunities for statisticians to take leadership roles in developing novel trial designs and statistical methodologies to help with the search for effective treatments.

Topics

- Primary analysis/missing data/estimands in AD
- Endpoints for earlier AD/time to event
- Disease modification methods
- Adaptive design in AD
- Designing/conducting controlled trials after the first DM drug reaches the market
- Additional topics will be added as the SWG grows and evolves.

Goals

The AD Scientific Working Group has the following goals:

- Assume a statistical leadership role in advancing the AD field in the areas of research and drug development
- Form a broad-based AD Stats Community that will stay current, connected and collaborative

This AD SWG will strive to leverage statistical thinking and innovation -- in helping to lead, motivate and support the AD field in the development of novel clinical trial designs and analysis methodologies. To effectively accomplish this goal, the SWG intends to reach out and partner with other organisations that are interested in developing AD treatments. For example, the Alzheimer's Association Research Roundtable (AARR) is a forum for pre-competitive collaboration among medical representatives from industry and academia, as well as representatives from government and regulatory bodies. The SWG will serve to connect and build synergy with a broad base of AD research organisations, such as AARR. The SWG will also strive to stimulate and link the broader research and patient communities -- develop workshops, educational short courses, talks and sessions focusing on statistical concepts and methodologies for international medical conferences, such as the Alzheimer's Association International Conference (AAIC) and the Clinical Trials on Alzheimer's Disease (CTAD) annual meetings. Commitment.

There is no doubt that AD is one of the most significant health crises facing the United States and the World for decades to come. Many of us either have had loved ones who have passed away from the disease or are currently engaged in fighting courageous battles with this terrible illness. So the passion we all feel is real and personal. We must act now to find the next generation of treatments for AD ... treatments that will slow, and, hopefully, prevent the disease. As statisticians, we have the will and the analytical power/expertise to shape the future of AD treatment development by connecting/collaborating with one another to explore and develop innovative approaches for the quantitative decision-making needed to discover and develop these much-needed new medical products and treatments.