Accelerating Medicines Partnership: Alzheimer’s Disease

The National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), 10 biopharmaceutical companies, and multiple non-profit organizations launched an unprecedented new public-private partnership in February 2014. Managed through the Foundation for the NIH (FNIH), the Accelerating Medicines Partnership (AMP) brings high-level government, industry, and non-profit organization partners together to identify and validate the most promising biological targets for therapeutics. The partners have designed a bold, milestone-driven research plan to tackle this challenge for Alzheimer’s disease, as well as for type 2 diabetes and the autoimmune disorders of rheumatoid arthritis and systemic lupus erythematosus (lupus). Importantly, the AMP data and analyses will be made publicly available to the broad biomedical community. This fact sheet addresses the AMP research plan for Alzheimer’s disease.

Alzheimer’s Disease

Dementia, of which Alzheimer’s disease is the most common form, is estimated to affect 36 million people worldwide. This number is expected to rise to 115 million by 2050 unless an effective therapeutic is developed. The financial toll of dementia is already staggering: in the U.S. alone, the costs of caring for people over 70 with dementia were estimated to be as high as $215 billion in 2010. AD is characterized by the presence of two signature brain lesions: plaque deposits between nerve cells composed of fragments of the protein, amyloid beta (Aβ), and neurofibrillary tangles (NFT) composed of aggregated tau proteins in the interior of cells.

Need for New Therapies

The evidence linking Aβ plaque accumulation as the cause of AD has resulted in the development of therapies by many biopharmaceutical companies. However, none to date has demonstrated clinical efficacy in patient trials. These failures may reflect problems with specific molecules and/or trial design rather than the underlying hypothesis. There is a pressing need for improved tools to support target validation in patients prior to Phase III clinical trials and to identify new targets that provide alternative approaches to targeting the disease process.

Additionally, it is critical to identify reliable biomarkers that are predictive of clinical response to therapeutic intervention.

AMP Approach

Human genetic studies have been critical in developing our understanding of AD and have recently provided new targets for drug development, such as brain inflammation, immune function, and...
cellular trafficking, in addition to more traditional targets such as beta-amyloid. The lack of data from clinical trials in humans to support target validation requires new approaches. Capitalizing on the collective expertise and resources from public-private partnerships may address this gap. This project seeks to a) identify markers of the disease (biomarkers) that can predict clinical outcomes by incorporating selected biomarkers into three NIH-funded clinical trials, which include industry support, designed to delay or prevent disease onset; b) conduct a large-scale analysis of human AD patient brain tissue samples to validate biological targets previously shown to play key roles in disease progression and, more significantly, to increase our understanding of the molecular pathways involved in the disease to identify new potential therapeutic targets. While past studies have demonstrated the promise of several AD biomarkers, this project aims to establish an expanded set of biomarkers that can be embedded in therapeutic trials as well as identify new biological targets for drug development.

**Governance**
The AD arm of the AMP initiative is managed by an AD steering committee (SC), comprising and non-profit organizations. The SC operates under the direction of the overall AMP Executive

**Budget: 5 years ($129.5 Million Total Project Funding)**

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<th>($Millions)</th>
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<th>Total Industry</th>
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