Optimizing Outcome Measures for Clin. Trials in Pre-Clinical Alzheimers Disease

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Principal Investigators

EDLAND, STEVEN DYAL

Institution

UNIVERSITY OF CALIFORNIA SAN DIEGO

Contact information of lead PI
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USA

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Optimizing Outcome Measures for Clin. Trials in Pre-Clinical Alzheimers Disease

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Research Abstract

ABSTRACT This grant will develop statistical methods for deriving optimal endpoints for clinical trials and longitudinal cohort studies of cognitive aging, mild cognitive impairment, and Alzheimer’s disease, and will publish new statistically efficient clinical trial outcome measures derived using these methods. Methods to be developed in this grant will substantially improve

the efficiency of clinical trials, reducing the cost and increasing the probability that effective treatments will be identified. The Specific Aims are: Specific Aim 1. To derive and apply methods for optimal calculation of instrument total scores. This is an extension of our earlier work using Item Response Theory to find the optimal scoring of items when calculating an instrument total score. Our earlier work trained the rescoring algorithm on cross-sectional data. The extension will be to train on longitudinal data. We have pilot data demonstrating a 25% reduction in required sample size for select instruments by the proposed method. Specific Aim 2. To derive and apply methods for optimal construction of composite scales. Composite scales combining cognitive and functional measures promise to dramatically improve the efficiency of clinical trials of mild cognitive impairment and is an area of active research. We have derived an optimal formula and can demonstrate superior performance relative to current methods with real data and simulations. Specific Aim 3. To demonstrate de novo outcome measure development by applying the methods developed in Aims 1 and 2 to a different but related progressive disease, frontotemporal dementia (FTD). This exercise is intended to demonstrate the generalizability of our methods to other disease areas. Moreover, software developed in performance of this grant will be posted as the methods are published and will be applicable to instrument development for any chronic disease for which quantitative traits are used as endpoints for clinical trials. This grant is entirely motivated by the observation that clinical trials of chronic progressive disease are prohibitively expensive. In Alzheimer’s disease research this has limited our ability to test new treatments and find a cure for the disease. For less common diseases such as FTD and progressive supranuclear palsy the need for more efficient endpoints is even more pressing, as the availability of study subjects for clinical trials further limits our ability to test treatments. Every subject enrolled in a clinical trial is a precious resource. This grant is intended to advance methods to optimally utilize all information obtained from subjects enrolled in clinical trials and increase the probability that effective treatments are identified.

Further information available at:

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