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2019 Part the Cloud: Translational Research Funding for Alzheimer's Disease (PTC)

Bosutinib effects on safety, biomarkers and clinical outcomes in DLB

This Phase Ib clinical trial will determine if a FDA-approved leukemia drug is safe in people with Dementia with Lewy Bodies, a type of dementia similar to Alzheimer's.

Background

Dementia with Lewy bodies (DLB) is a common form of dementia, characterized by specific brain changes, including the presence of Lewy bodies and tau neurofibrillary tangles. Lewy bodies are chiefly comprised of alpha-synuclein, a protein that loses its normal shape and clumps together, similar to the beta-amyloid protein that clumps together and forms plaques, a hallmark of Alzheimer's. When the tau protein gets misfolded and forms masses or aggregates, it gives rise to tau tangles- another hallmark of Alzheimer's.

Past studies in genetically engineered Alzheimer's and DLB-like mouse models by Dr. Charbel Moussa's team have demonstrated that a FDA approved cancer drug called bosutinib can remove toxic proteins that accumulate in the brain and improve cognitive symptoms.

Research Plan

Building on their prior work, Dr. Charbel Moussa and colleagues will perform a phase Ib clinical trial to evaluate the safety and tolerability of bosutinib, in people with DLB. For the study, the researchers plan to enroll 30 people with DLB and will evaluate the overall safety of this drug in this population. In addition, Dr. Moussa will measure the effects of daily bosutinib at a low dosage on harmful protein deposits and clinical symptoms associated with DLB and Alzheimer's.

Impact

This clinical trial represents an important step to determine if an FDA-approved leukemia drug could be repurposed to tackle cognitive disease. If successful, the results of this work could lead to future large-scale clinical trials of bosutinib for Alzheimer's disease, DLB and other dementias to evaluate potential efficacy.

PI

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- Scientific and Clinical Research Director of the Translational Neurotherapeutics program at Georgetown University

STUDY

- CADRO category: Translational Research & Clinical Interventions
- Dr. Moussa has been the recipient of the Association's New Investigator Research Grant in the past.