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Phase 2 Trial of Rapamycin for Alzheimer’s Disease

This Phase 2 clinical trial will evaluate whether an immune suppressing drug can be repurposed to tackle Alzheimer’s.

Background
Aging is the biggest risk factor for Alzheimer’s. Recent work has shown that there may be overlap between the biological pathways involved in aging and Alzheimer’s. As a result, researchers are studying novel therapeutic targets that could address both advanced aging and Alzheimer’s.

Rapamycin is a drug that targets a biological pathway called mTOR (mammalian Target of Rapamycin), which impacts a variety of cell functions including cell growth, metabolism (process by which cells convert nutrients to energy) and cell death amongst others. It is currently approved by the FDA (Food and Drug Administration) for individuals who have received organ transplants. It suppresses the immune system in these individuals and prevents them from rejecting transplants. However, the biological pathway through which Rapamycin acts on the immune system is not yet entirely understood. Preliminary studies by Dr. Sudha Seshadri’s team indicate that rapamycin may decrease the accumulation of beta-amyloid plaques and tau tangles, the two hallmark brain changes observed in Alzheimer’s and that the drug may improve cognitive deficits.

Research Plan
Building on their Phase 1 study, Dr. Seshadri and colleagues will conduct a Phase II clinical trial to test the safety and tolerability of rapamycin in 40 older adults with early-stage Alzheimer’s or mild cognitive impairment (a condition with subtle memory loss that may precede dementia, including Alzheimer’s dementia). Participants in the study will receive either a daily dose of the drug or a placebo (not the actual drug but an inactive substance that has no benefits and also no risk for the participant) for six months. The researchers will perform brain scans, cognitive tests and collect blood and cerebrospinal fluid
samples (a biological fluid found in the brain and spinal cord). The researchers will study changes in cognition as well as biological markers (biomarkers) associated with Alzheimer’s in order to evaluate the impact of rapamycin on brain changes in these participants.

Impact
This study will test whether Rapamycin might be repurposed to reduce brain changes seen in individuals with mild cognitive impairment and Alzheimer’s.

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