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CSF1R inhibitor EI1071 for modulating microglia-associated neuroinflammation

This Phase 1 trial will examine safety and tolerability of a chemical compound that may reduce brain inflammation in Alzheimer's.

Background

Microglia are the primary immune cells of the brain and serve to help maintain healthy nerve cells in the brain. Individuals with Alzheimer's typically experience increased brain inflammation caused by an uncharacteristically active immune system, including increased activity of microglia. This increased activity may lead to nerve cell damage.

Studies show that a protein called Colony-Stimulating Factor 1 Receptor (CSF1R) is important for the growth and survival of microglia. Preliminary experiments by Dr. Hung-Kai Chen's team and by other research groups showed that blocking CSF1R using chemical compounds reduced microglia in genetically engineered Alzheimer's-like mouse models. Moreover, they also observed that the compound reduced brain inflammation and other brain changes associated with Alzheimer's in these mice.

Research Plan

Researchers have been studying ways to reduce brain inflammation during Alzheimer's to help preserve nerve cell function and communication. Building on their preliminary findings, Dr. Chen and colleagues will conduct a first in human, Phase I clinical trial to evaluate the safety and tolerability of their chemical compound in cognitively unimpaired adults. Participants in the study will receive either the drug or a placebo (not the actual drug but an inactive substance that has no risk for the participant). The researchers will monitor participants closely, to test the safety of the drug and any potential adverse side effects. Dr. Chen and colleagues will periodically collect blood samples from the participants.

The researchers will analyze collected blood samples to evaluate the impact of their chemical compound in terms of biological markers (biomarkers) associated with inflammation as well as to monitor the level of their compound in these blood samples. The knowledge gained

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STUDY

- CADRO category: Translational Research & Clinical Interventions

from this study will be used to guide dosage selection of their chemical compound for future clinical studies in individuals with Alzheimer's.

Impact

If successful, the study results may give rise to larger clinical trials and may help reduce brain inflammation in Alzheimer's and other brain diseases thought to be associated with inflammation such as Parkinson's, amyotrophic lateral sclerosis and multiple sclerosis.

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