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2016 Part the Cloud Challenge for Immunity and Neuroinflammation Grant

Safety & Efficacy of GM-CSF/Leukine in Mild-to-Moderate Alzheimer's Disease

This Phase II study will determine if the FDA-approved drug, Leukine, is safe and can help slow or prevent the progression of Alzheimer's disease

Background

Most cases of arthritis are caused by normal wear and tear on joints. But a small percentage of cases are caused by abnormal activation of the immune system, and these cases are known as rheumatoid arthritis. There is some evidence to suggest that people with rheumatoid arthritis (RA) may not develop Alzheimer's at the same rates as individuals without RA. Studies have shown that this inverse correlation cannot be explained by the fact that people with RA use non-steroidal anti-inflammatory drugs (NSAIDs).

Huntington Potter, Ph.D., and colleagues have found that people with RA have unusually high levels of GM-CSF (granulocyte macrophage-colony stimulating factor) in their blood. GM-CSF is a hormone-like protein naturally found in the body that is important for the production and function of immune cells. In mice that have an Alzheimer's-like condition, treatment with GM-CSF inhibits the development of disease-related brain changes and memory problems. It is thought that GM-CSF may help modulate levels of inflammation in the brain or prevent the accumulation of beta-amyloid – both of these factors are associated with Alzheimer's disease.

Research Plan

Dr. Potter and colleagues are already conducting a small, short-term (15 days) Phase I clinical trial to assess the safety of a manufactured version of GM-CSF called Leukine® (sargramostim) in people who have mild to moderate Alzheimer's disease. They have observed no negative side effects and with the support of the Part the Cloud Challenge award propose a more extensive Phase II clinical trial to test the effects when the drug is administered for 24 weeks.

Dr. Potter's team plans to enroll 56 people with mild to moderate Alzheimer's disease and treat them with Leukine or a placebo. The participants will be monitored for side effects and the researchers will determine if Leukine can help slow or prevent disease-related brain changes and cognitive decline. Because Leukine is already FDA-approved to treat other conditions and significant safety data has been collected in

humans, it is allowed to be immediately tested in clinical trials for treating Alzheimer's disease.

Impact

This Phase II clinical trial will build on an existing clinical trial to provide additional safety information and greatly expand the potential to detect beneficial effects of Leukine treatment in people with Alzheimer's disease. If successful, this work could provide the foundation for testing Leukine in large-scale clinical trials to slow or prevent Alzheimer's disease progression.

Made possible through the generous funding from Part the Cloud, benefiting the Alzheimer's Association.