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

Early Indicators in CU Alzheimer's Drug Trial Show "Unexpected" Positive Signs

The Rocky Mountain Alzheimer's Disease Center (RMADC) at the University of Colorado Anschutz Medical Campus reported at AAIC17 unexpected positive results related to thinking, memory, and everyday living for a drug being tested for Alzheimer's disease. The drug known as Leukine® or GM-CSF is commonly used in bone marrow transplant patients to stimulate white blood cell production, which can generate a particular cell type responsible for cleaning up debris and fighting infections (i.e. macrophages). This ongoing clinical trial is testing whether stimulating white blood cell production causes any negative side effects. They are also testing whether Leukine® removes the amyloid plaques, a hallmark of Alzheimer's, and possibly helps the brain repair itself.

An interim report was presented at AAIC17 using preliminary data on the study of Leukine®, led by Drs. Huntington Potter, Jonathan Woodcock, and Tim Boyd. Because the original intent was to report on the safety of Leukine®, the scientists were not expecting to see noticeable study results on improving thinking, memory, or the ability to perform normal everyday activities. "We say 'unexpected' because this phase of the clinical trial is primarily testing the drug for safety, so the improvement in cognition scores was a bonus," said Dr. Potter, director of the RMADC and member of the Linda Crnic Institute for Down Syndrome. Generally, studies at this early stage are not designed to see changes in cognition or daily living activities, so improvements may suggest the effects are greater than anticipated and certainly warrant further studies.

This report also stated there were no indications of negative side effects that could be associated with the drug, such as small hemorrhages in the brain created by removing amyloid - something observed in other clinical trials using different drugs to remove amyloid. "This is a short safety trial, lasting just under three weeks for each participant, and we weren't expecting to see improvements in cognitive testing results in that short of time," said Dr. Boyd, who has been working with Dr. Potter on the Alzheimer's treatment theory involving Leukine®. "For us to see these kinds of improvements gives us a lot of hope."

The researchers will complete the safety phase of this current clinical trial this summer. "The positive safety results in this interim report," Dr. Potter said, "justify moving forward with the longer study, which will be funded, in part, by a recent \$1 million grant from the Alzheimer's Association's "Part the Cloud" initiative." The next part of this Phase 2 trial, participants will be studied for a longer period, around 18 months, to better evaluate whether Leukine® makes a measureable difference in thinking, memory, and biomarkers, such as amyloid plaques.

"We have a long way to go," said Dr. Potter, "but these early preliminary results with Leukine® are encouraging enough to show that we should continue. Without the help of the Part The Cloud funding, we would not be able to carry out the clinical trial that is essential for determining the potential GM-CSF/Leukine as a treatment for Alzheimer's disease."