Alzheimer's patients' cognition improves with Sargramostim (GM-CSF), new study shows

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A new study suggests that Sargramostim, a medication often used to boost white blood cells after cancer treatments, is also effective in

treating and improving memory in people with mild-to-moderate Alzheimer's disease. This medication comprises of a natural human protein produced by recombinant DNA technology (yeast-derived rhu GM-CSF/Leukine).

The study, from the University of Colorado Alzheimer's and Cognition Center at the University of Colorado Anschutz Medical Campus (CU Anschutz), presents evidence from their clinical trial that shows that Sargramostim may have both disease-modifying and cognition-enhancing activities in Alzheimer's disease patients. It was published online today by *Alzheimer's & Dementia: Translational Research and Clinical Interventions*, an open access journal of the Alzheimer's Association.

"The goal of the clinical trial was to examine the impact of a natural human protein called granulocyte-macrophage colony stimulating factor (GM-CSF) on people living with Alzheimer's disease. We tested GM-CSF because people with rheumatoid arthritis tend not to get Alzheimer's disease and we had previously found this protein, which is increased in the blood of people with rheumatoid arthritis, reduced amyloid deposition in Alzheimer's mice and returned their poor memory to normal after a few weeks of treatment. Thus, naturally increased levels of GM-CSF in people with rheumatoid arthritis may be one reason that they are protected from Alzheimer's disease," said Huntington Potter, Ph.D., director of the CU Alzheimer's and Cognition Center, who together with Jonathan Woodcock, Timothy Boyd and collaborators carried out the new trial.

"Human GM-CSF is the active compound in the known human drug Sargramostim, and we are the first to study its effect on people with Alzheimer's disease."

GM-CSF/Sargramostim is used to stimulate the bone marrow to make

more white blood cells of a particular kind called macrophages and granulocytes, as well as progenitor cells that repair blood vessels. These white blood cells circulate throughout the body and remove cells, bacteria and amyloid deposits that aren't supposed to be there, as well as promoting repair to damaged blood vessels and to the brain.

The researchers carried out a randomized, double-blind, placebo-controlled phase II trial to test the safety and efficacy of Sargramostim treatment in participants with mild-moderate Alzheimer's disease. Study participants who met eligibility criteria were randomized to receive injections of either Sargramostim (20 participants took a standard FDA dosage 250 mcg/m^2/day subcutaneous injection for five days a week for three weeks) or placebo (20 participants took saline for five days a week for three weeks). The majority of the participants from the study were recruited and treated at CU Anschutz with a few from the University of South Florida.

The CU Anschutz researchers then conducted and studied multiple neurological, neuropsychological, cell, cytokine, Alzheimer's pathology biomarkers and neuroimaging assessments.

They found that short-term Sargramostim treatment increased innate and other immune cells, modulated cytokine measures, and was safe and well-tolerated by participants. They also found cognition memory improved by almost two points in the 30 point Mini-Mental State Exam. Measures of blood biomarkers of Alzheimer's disease—brain amyloid, tangles, and neurodegeneration—all improved toward normal.

"These results suggest that short-term Sargramostim treatment leads to innate immune system activation, cognition and memory improvement, and partial normalization of blood measures of amyloid and tau pathology and neuronal damage in participants with mild-to-moderate Alzheimer's disease," said Potter.

"This surprising finding that stimulating the innate immune system and modulating inflammation may be a new treatment approach and induced us to start a larger trial of Sargramostim in Alzheimer's disease with more participants treated over a longer time."

More information: Huntington Potter et al. Safety and efficacy of sargramostim (GM-CSF) in the treatment of Alzheimer's disease, *Alzheimer's & Dementia: Translational Research & Clinical Interventions* (2021). DOI: 10.1002/trc2.12158

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