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Vaccine Targets Alzheimer's Disease-like Characteristics in People with Down Syndrome

Clinical trial will investigate safety and tolerability of immunotherapy drug to block amyloid buildup in brain

In the first clinical trial of its type, researchers at University of California, San Diego School of Medicine and the Alzheimer's Disease Cooperative Study (ADCS), in collaboration with AC Immune, a biotechnology company based in Switzerland, will test the safety and tolerability of an immunotherapy vaccine that targets Alzheimer's disease-like characteristics in adults with Down syndrome.

Down syndrome (DS) is caused by an abnormality on chromosome 21, which, among other things, includes the gene regulating expression of amyloid-beta or A-beta, which is the main component of plaques that accumulate in the brain and are a hallmark of Alzheimer's disease. The likelihood of a person with Down syndrome also developing Alzheimer's disease is three to five times greater than a person without DS – and it will appear much earlier in life. Virtually all DS patients display beta-amyloid neuropathology by age 40.

"Persons with Down syndrome represent predictable cases of Alzheimer's disease," said William Mobley, MD, PhD, chair of the Department of Neurosciences in the UC San Diego School of Medicine and executive director of the Down Syndrome Center for Research and Treatment, which will conduct the trial in collaboration with AC Immune.

"This trial and vaccine offer a dual opportunity. First, it may be a way to modify progression of the disease through anti-amyloid intervention. Second, it should provide important insights about the efficacy and timing of such interventions when targeting sporadic Alzheimer's disease in the general population."

The ACI-24 vaccine is designed to induce antibodies against beta-amyloid, thus reducing its accumulation in the brain while not triggering a larger immune system response. Previous testing in a mouse model showed a robust antibody response and improvement in memory capacity.

“The human trial will focus on safety, tolerability and immunogenicity of the ACI-24 vaccine. Effects on cognitive function and biomarkers for Alzheimer’s disease will be secondary endpoints,” said Michael Rafii, MD, PhD, principal investigator of the clinical trial and assistant professor of neurosciences who studies the links between Down syndrome and Alzheimer’s disease.

Rafii, with co-authors Ahmad Salehi, MD, PhD, of Stanford University and Cristy Phillips, EdD, of Arkansas State University, recently published a textbook on the topic: Common Pathogenic Mechanisms between Down Syndrome and Alzheimer’s Disease: Steps toward Therapy.

The study will involve 24 adults with DS between the ages of 35 and 45. Participants will be treated for 12 months, with 12 months of follow-up.

Funding for the study is provided by the National Institutes of Health (grant 1R01AG047922-01) and a grant from the LuMind Research Down Syndrome Foundation, an international non-profit organization based in Massachusetts.

Trial organizers say it is the first test of an anti-amyloid immunotherapy treatment of Alzheimer’s disease in Down syndrome and the first public-private collaboration for a clinical trial in the field of Down syndrome research.

For more information about this trial, contact Holly Hainley at 858-246-1300.

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