

A Potential Drug Target for Amyotrophic Lateral Sclerosis (ALS)

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Technology

Amyotrophic Lateral Sclerosis (ALS) also named Lou Gehrig's disease, is a fatal neurodegenerative disease characterized by the progressive degeneration of cortical brain neurons, brainstem and upper and lower motor neurons. Within a short time, the disease leads to limb paralysis, compromise of speech and swallow, respiratory failure and death. The cause of ALS is unknown and to date there is no cure. Currently the only approved drugs for ALS treatment are riluzole (Rilutek) since 1995 and edaravone (Radicava) since 2017, both act to slow the progression of ALS symptoms and prolong survival, and both have shown only modest effects. Other off label treatments concentrates on slowing the progression of the symptoms, preventing unnecessary complications and trying to improve patient's quality of life. Therefore, there is a huge unmet need for ALS treatments using drugs with novel mechanism of action, which can alter disease progression.

Dr. Rachel Lichtenshtein's studies have shown over-expression of Fc γ RIIIA (CD16) on microglia cells and CD16-mediated motor neuron death mediated by Immunoglobulin G antibodies (IgGs) belonging to ALS patients. Therefore, blocking CD16 by Fc-like agonist in the CNS may reduce motor neuron degeneration and extend patient survival. Behavior tests of ALS mice administered by Fc-like agonist revealed extended survival by three weeks compared to survival of those injected by placebo. Molecular measurements after agonist administration showed homogenous distribution within microglia in the spinal cord and brain, better cleansing of cell debris, recovered motor neurons after 50 days from the cerebrospinal fluid injections and production of tissue-supportive cytokines. Therefore, we suggest a potential novel treatment for ALS patients, which can significantly reduce disease progression and increase physical abilities.

Advantages

- Novel treatment for ALS disease
- Therapy uses an Fc-like agonist from Fc γ RIIIA (CD16) as a novel modality to treat ALS.

Patent

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