USC Stevens Center for Innovation

USC University of Southern California

Treatment of ALS and other Neurological Diseases

USC Case #2015-285

Market Opportunity:

Amyotrophic lateral sclerosis (ALS) is a rare neurological disease that affects approximately 30,000 people in the United States, with roughly 5,000 new cases that get diagnosed each year. Symptoms for ALS include fasciculation, respiratory problems, muscles weakness, and cramps. There is currently no cure for ALS and treatments presently available only help alleviate symptoms. Due to the high mortality rates of ALS patients, there is a need to develop new treatment options.

USC Solution:

USC researchers have identified a method of treating ALS by restoring the levels of the C9ORF72 protein. Additional studies have shown that inhibiting the PIKFYVE kinase with inhibitors prevent the degeneration of neurons derived from ALS and frontotemporal dementia patients and rescues neurodegeneration in cell models of sporadic ALS. These results demonstrate how stem cell and reprogramming-based approaches can help to identify new therapeutic targets to treat ALS and other neurological diseases.

Value Proposition

- Novel method for treating neurological diseases
- Restoring C9ORF72 protein expression or replacing its function with small molecules rescues neuronal survival

Keywords:

Amyotrophic lateral sclerosis, AML, Lou Gehrig's disease, frontotemporal dementia, C9ORF72, endosomal trafficking

Applications

- Treatment for ALS and other neurological diseases

Stage of Development

- Tested in human induced motor neurons and cell models
- Available for non-exclusive and exclusive license

Intellectual Property

Status:

Patent application published - US20180161335A1

Key Publication:

"Haploinsufficiency leads to neurodegeneration in C9ORF72 ALS/FTD human induced motor neurons," *Nature Medicine*, February 5, 2018.

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