

# Treatment of ALS and other Neurological Diseases

Published date: Aug. 28, 2018

## Technology description

## 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.

### Key Publication:

[“ Haploinsufficiency leads to neurodegeneration in C9ORF72 ALS/FTD human induced motor neurons,” Nature Medicine, February 5, 2018.](#)

## Application area

Treatment for ALS and other neurological diseases

## Institution

[University of Southern California](#)

## 联系我们



叶先生

电话：021-65679356

手机：13414935137

邮箱：yeyingsheng@zf-ym.com