

Inhibition of Protein Aggregation for Neurodegenerative Disease Therapeutics



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PHARMA

Neurology

SUMMARY

Neurodegenerative diseases represent a major and growing global health challenge.

In the case of Amyotrophic Lateral Sclerosis (ALS), aberrant protein aggregation disrupts essential cellular processes, leading to neuronal degeneration and dysfunction, severe disability, and ultimately death. Currently, no effective disease-modifying treatments are available.

The team is developing a novel therapeutic strategy for the treatment of neurodegenerative diseases, specifically targeting the root cause by preventing or reversing protein aggregation early in disease progression.

This approach is mechanistically targeted and has the potential to transform therapeutic options for ALS.

PROJECT GOALS

- Optimize candidates for stability, uptake and binding
- *In vitro* validation of mechanism
- Establish brain-delivery feasibility strategy
- Patent application

LONG-TERM GOALS

- *In vivo* proof of concept
- Development plan toward Investigational New Drug (IND)-enabling studies
- License IP to industry or create a startup