Transposon to Present Results from Phase 2 Study of TPN-101 for the Treatment of C9orf72-Related ALS/FTD at the 7th Annual ALS ONE Research Symposium

Treatment with TPN-101 showed clinical benefits on key clinical outcome measures, including the Revised ALS Functional Rating Scale (ALSFRS-R) and Slow Vital Capacity (SVC)

TPN-101 also had lowering effects on key biomarkers of neurodegeneration and neuroinflammation, including neurofilament light chain (NfL) and interleukin 6 (IL-6)

SAN DIEGO, California, November 6, 2024 – Transposon Therapeutics, a biotechnology company developing a platform of novel, orally administered therapies for the treatment of neurodegenerative and aging-related diseases, including Alzheimer's disease, today announced that results from its Phase 2 study of TPN-101 in patients with amyotrophic lateral sclerosis (ALS) and/or frontotemporal dementia (FTD) related to hexanucleotide repeat expansion in the *C9orf72* gene (*C9orf72*-related ALS/FTD) will be presented at the 7th Annual ALS ONE Research Symposium. The symposium will take place virtually on November 13-15, 2024.

Presentation details

Title:	A Phase 2A study of TPN-101, a nucleoside reverse transcriptase inhibitor, in patients with C9ORF72-related ALS/FTD
Presenter:	Andrew Satlin, M.D.
Date and time:	Thursday, November 14, 2024, at 2:00 pm EST

For more information, please visit the ALS ONE Research Symposium website.

About the Phase 2 Study in C9orf72-related ALS/FTD

The Phase 2 study in patients with *C9orf72*-related ALS/FTD was a multi-center, randomized, double-blind, placebo-controlled parallel-group, two-arm study with an open-label treatment period. Participants (n=42) were randomized 3:2 to receive daily doses of 400 mg of TPN-101 or placebo. The study included a six-week screening period, a 24-week double-blind treatment period, a 24-week open-label treatment period, and a follow-up visit four weeks post-treatment. Further information on the study can be accessed at ClinicalTrials.gov.

About TPN-101

TPN-101 specifically inhibits the LINE-1 reverse transcriptase that promotes LINE-1 replication. LINE-1 elements are a class of retrotransposable elements that in humans are uniquely capable of replicating and moving to new locations within the genome. When this process becomes dysregulated, LINE-1 reverse transcriptase drives overproduction of LINE-1 cDNA, triggering innate immune responses that contribute to neurodegenerative, neuroinflammatory, and aging-related disease pathology.

About ALS and FTD

ALS is a neurodegenerative disease characterized by progressive muscle weakness, and loss of ability to speak, eat, move or breathe. FTD is a progressive frontal/temporal cortex disease associated with behavior and personality changes, emotional problems, and difficulty walking, communicating, or working. A *C9orf72* hexanucleotide repeat mutation accounts for 10-15% of both disorders. With onset commonly in middle age or earlier, patients with ALS have a mean survival of two to three years. Patients with FTD have a mean survival of nine years.

About Transposon

Transposon Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing a platform of novel therapies for the treatment of neurodegenerative and aging-related diseases, including Alzheimer's disease. The company's lead clinical compound, TPN-101, is first-in-class to address LINE-1 reverse transcriptase for treating neurodegenerative and autoimmune diseases. The company also has a discovery platform supporting a deep pipeline of novel therapies to address additional indications.

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