Transposon to Present Results from Phase 2 Study of TPN-101 for the Treatment of C9orf72-Related ALS/FTD at the 2024 Annual NEALS Meeting

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

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

SAN DIEGO, California, October 9, 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) have been accepted for both oral and poster presentation at the 2024 Annual Northeastern Amyotrophic Lateral Sclerosis Consortium (NEALS) Meeting. The hybrid meeting will take place October 21-24, 2024, in Clearwater, Florida, and virtually.

"Given the effects of TPN-101 on key biomarkers of neurodegeneration and neuroinflammation, including NfL and IL-6, and clinical outcome measures of disease progression and respiratory function in patients with *C9orf72*-related ALS, we are very pleased these data were accepted for presentation at NEALS," said Andrew Satlin, M.D., Chief Medical Officer at Transposon. "Based on these promising results, we are advancing TPN-101 into a Phase 3 registration study for the treatment of *C9orf72*-related ALS, while also continuing to develop TPN-101 for other neurodegenerative diseases."

Oral 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.
Session:	NEALS Affiliated Trial Session
Date and time:	Tuesday, October 22, 2024, at 11:15 am EDT
Location:	Opal Ballroom

The poster will also be on view during Poster Session 2 on Wednesday, October 23, 2024, from 4:00 to 6:00 pm EDT.

Title:	A Phase 2A Study of TPN-101, A Nucleoside Reverse Transcriptase Inhibitor, in Patients with C9ORF72-Related ALS/FTD
Session:	Poster Session 2
Date and time:	Wednesday, October 23, 2024, from 4:00 to 6:00 pm EDT

Location: Sea Salon & Sand Salon

For more information, please visit the 2024 Annual NEALS Meeting 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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