



Transposon Announces Interim Results from a Phase 2 Study of TPN-101 for the Treatment of Progressive Supranuclear Palsy to be Presented at the AD/PD™ 2024 International Conference on Alzheimer’s and Parkinson’s Diseases

24-Week data show TPN-101 is the first treatment for PSP to reduce NfL levels, a key biomarker of neurodegeneration in tauopathies such as PSP and Alzheimer’s disease

TPN-101 also showed a reduction in IL-6 cytokine levels, a biomarker of neuroinflammation that is elevated in PSP patients and correlates with disease severity

Once-daily oral dosing of TPN-101 was well-tolerated by patients with PSP

Transposon to report data from three Phase 2 clinical studies of TPN-101 in four disorders, including PSP, amyotrophic lateral sclerosis, frontotemporal dementia, and Aicardi-Goutières Syndrome, in Q1 2024

SAN DIEGO, California, November 14, 2023 – Transposon Therapeutics, a biotechnology company developing a platform of novel, orally administered therapies for the treatment of neurodegenerative and aging-related diseases, today announced its abstract of interim results from its Phase 2 study of TPN-101 for the treatment of progressive supranuclear palsy (PSP) has been accepted for poster presentation at the hybrid AD/PD™ 2024: 18th International Conference on Alzheimer’s and Parkinson’s Diseases. The meeting will take place online and in Lisbon, Portugal, from March 5-9, 2024.

The poster presentation will highlight results from the study’s predefined interim analysis, which examined the change from baseline to 24 weeks in biomarkers of neurodegeneration and neuroinflammation, including neurofilament light chain (NfL) and interleukin 6 (IL-6) levels in cerebrospinal fluid (CSF). In the treatment group receiving 400 mg of TPN-101 once daily for 24 weeks, TPN-101 showed an 18.4% reduction in NfL levels in CSF as compared to placebo. NfL is a biomarker of neurodegeneration that correlates with disease severity and progression in PSP. In the same 400 mg treatment group, TPN-101 also resulted in a 51.6% reduction in IL-6 levels in CSF as compared to placebo. IL-6 is a biomarker of neuroinflammation that is elevated in PSP patients and correlates with disease severity. Once-daily oral dosing of TPN-101 was well-tolerated at all dose levels.

“No previously tested drug has shown an effect on NfL levels in patients with PSP, a progressive neurological disorder with no approved treatment options to stop or even slow progression of the disease,” said Andrew Satlin, M.D., Chief Medical Officer at Transposon. “The lowering of CSF NfL levels seen in this interim analysis provides biomarker evidence of a treatment effect on neurodegeneration. We are excited about the potential of TPN-101 as a much-needed treatment option for patients with PSP. In addition, we believe these findings open the door to an entirely new therapeutic approach to treating Alzheimer’s and other neurodegenerative diseases.”

“The Transposon team continues to make excellent progress against our key development milestones and corporate priorities, and we look forward to presenting these interim results from our PSP program at the AD/PD 2024 International Conference on Alzheimer’s and Parkinson’s Diseases,” said Dennis Podlesak, Chairman and Chief Executive Officer of Transposon. “Beyond the promising reduction in NfL levels seen in our PSP study, we also look forward to reporting data from our three ongoing Phase 2 programs of TPN-101 in the first quarter of 2024 and, importantly, to expanding our development efforts for the compound into Alzheimer’s disease and other neurodegenerative and autoimmune disorders.”

Transposon will present results from the interim analysis in a poster presentation (abstract #433) at AD/PD 2024 entitled: “A Phase 2a Study of TPN-101, a Nucleoside Reverse Transcriptase Inhibitor, in Patients with Progressive Supranuclear Palsy.”

For more information, please visit the [AD/PD™ 2024 website](#).

About the Phase 2 study in PSP

The Phase 2 study in PSP is a multi-center, randomized, double-blind, placebo-controlled, parallel-group, 4-arm study with an open-label treatment phase in patients with PSP. Patients (N=42) were randomized to receive daily doses of 100 mg, 200 mg or 400 mg of TPN-101, or placebo. The study includes a 6-week screening period, a 24-week double-blind treatment period, a 24-week open label treatment period, and a follow-up visit 4 weeks post treatment. The predefined interim analysis was performed after all patients completed the 24-week double-blind portion of the study. The open-label extension of the study is ongoing. 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 DNA, triggering innate immune responses that contribute to neurodegenerative, autoimmune and aging-related disease pathology.

About PSP

PSP is a rare and fatal tauopathy with no approved treatment options. PSP mainly affects people in their mid- to late-60s and is characterized by early postural instability and falls, vertical gaze palsy, akinesia, rigidity, pseudobulbar palsy, and frontal dysfunction with cognitive and behavioral changes. The mean survival for individuals with PSP is 6 to 7 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.

Transposon will report data from three Phase 2 clinical studies of TPN-101 in four disorders, including progressive supranuclear palsy (PSP), amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), and Aicardi-Goutières syndrome (AGS), in Q1 2024. The company also has a discovery platform supporting a deep pipeline of novel therapies to address additional indications.

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