Transposon Receives US FDA Fast Track Designation for TPN-101 for Progressive Supranuclear Palsy

Designation Supports Accelerated Drug Development for PSP

TPN-101 is the First PSP Treatment to Reduce NfL and IL-6 Levels, Key Biomarkers of Neurodegeneration and Neuroinflammation in PSP

SAN DIEGO, California, May 21, 2024 – Transposon Therapeutics, a biotechnology company developing a platform of novel, orally administered therapies for the treatment of neurodegenerative and aging-related diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to TPN-101 for progressive supranuclear palsy (PSP).

"Fast Track designation for TPN-101 is an important acknowledgement by the FDA of the critical need to find an effective treatment for PSP, a rare and devastating neurological disorder with no approved treatment options," said Dennis Podlesak, Chairman and Chief Executive Officer of Transposon. "We look forward to working collaboratively with the FDA to advance the development of TPN-101 as rapidly as possible for the treatment of PSP and other neurodegenerative diseases including ALS and Alzheimer's disease."

The designation of TPN-101 as a Fast Track product for PSP is supported by data from a Phase 2, randomized, double-blind, placebo-controlled study of TPN-101 in patients with PSP. In that study, TPN-101 was the first treatment for PSP to reduce levels of neurofilament light chain (NfL), a key biomarker of neurodegeneration in tauopathies such as PSP and Alzheimer's disease. TPN-101 also showed dose-related reductions in interleukin 6 (IL-6) cytokine levels, a biomarker of neuroinflammation that is elevated in PSP and correlates with disease progression and severity. Participants treated with TPN-101 for the entire 48-week trial duration showed a stabilization of their clinical symptoms as measured by the PSP Rating Scale (PSPRS) between weeks 24 and 48. Further information on the study can be accessed at ClinicalTrials.gov.

About Fast Track Designation

The FDA grants Fast Track designation to facilitate the development and expedite the review of medicines to treat serious conditions and fill an unmet medical need. Fast Track status allows for enhanced communication and collaboration between the FDA and drug developers, potentially speeding up the delivery of life-saving treatments to patients.

About PSP

PSP is a rare neurodegenerative disorder that causes slowing of movement, loss of balance leading to falls, impaired eye movements, and disturbances in cognition and behavior. The disease typically affects people in their mid- to late-60s, and the mean survival for individuals

with PSP is 6 to 7 years. There are currently no treatments capable of delaying the progression of the disease.

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 progressive supranuclear palsy, amyotrophic lateral sclerosis, frontotemporal dementia, and 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.

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.

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