Transposon Receives Investment from the Alzheimer's Drug Discovery Foundation to Support Advancement of TPN-101 for the Treatment of Alzheimer's Disease

Building on the positive results of Phase 2 clinical trials in PSP and ALS, Transposon plans to initiate a Phase 2 clinical trial of TPN-101 for the treatment of Alzheimer's disease in Q4 2025

Concurrently, Transposon is advancing TPN-101 in the Phase 2/3 HEALEY ALS Platform trial

SAN DIEGO, CA, July 9, 2025 – Transposon Therapeutics, a biotechnology company focused on developing novel, orally administered therapies for the treatment of neurodegenerative and aging-related diseases, including amyotrophic lateral sclerosis (ALS), progressive supranuclear palsy (PSP), and Alzheimer's disease (AD), today announced that it has secured an investment from the Alzheimer's Drug Discovery Foundation (ADDF) to advance the development of TPN-101 for the treatment of AD. The company expects to initiate a Phase 2 clinical trial to study the effect of TPN-101 in patients with AD by the end of 2025.

"We are delighted to be partnering with the Alzheimer's Drug Discovery Foundation to rapidly advance the development of TPN-101 for the treatment of Alzheimer's disease," said Dennis Podlesak, Chairman and Chief Executive Officer of Transposon Therapeutics. "Based on the positive results of our recently completed Phase 2 studies of TPN-101 in both PSP and ALS, we believe TPN-101 has the potential to provide a critically needed treatment option for a wide range of neurodegenerative diseases, including Alzheimer's, ALS, and PSP."

In the Phase 2 clinical trial of TPN-101 in patients with PSP, TPN-101 was the first treatment to reduce CSF levels of neurofilament light chain (NfL) compared to placebo, 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. In the Phase 2 clinical trial of TPN-101 in patients with *C9orf72*-related ALS and/or frontotemporal dementia (FTD), treatment with TPN-101 also reduced levels of NfL and IL-6 in the ALS population and showed a slowing of ALS disease progression as measured by the Revised ALS Functional Rating Scale (ALSFRS-R).

"The ADDF has a long history of supporting and investing in approaches to combat the underlying pathology of aging including novel pathways like inflammation," said Howard Fillit, MD, Co-Founder and Chief Science Officer at the ADDF. "Transposon's advancement of TPN-101 to target neuroinflammation and the abnormally activated immune system is one of the many exciting new therapeutic approaches in development for Alzheimer's."

Concurrent with the Phase 2 clinical trial to study the effect of TPN-101 in patients with AD, Transposon is advancing TPN-101 in the Phase 2/3 HEALEY ALS Platform trial. Further information about the HEALEY ALS Platform Trial can be found at HEALEY ALS Platform Trial.

About the Alzheimer's Drug Discovery Foundation

Founded in 1998 by Leonard A. and Ronald S. Lauder, the Alzheimer's Drug Discovery Foundation is dedicated to rapidly accelerating the discovery of drugs to prevent, treat and cure Alzheimer's disease. The ADDF is the only public charity solely focused on funding the development of drugs for Alzheimer's, employing a venture philanthropy model to support research in academia and the biotech industry. The ADDF's leadership and contributions to the field have played a pivotal role in bringing the first Alzheimer's PET scan (Amyvid®) and blood test (PrecivityAD®) to market, as well as fueling the current robust and diverse drug pipeline. Through the generosity of its donors, the ADDF has awarded more than $370 million to fund 765 Alzheimer's drug discovery programs, biomarker programs and clinical trials in 21 countries. To learn more, please visit: **http://www.alzdiscovery.org/**.

About Transposon

Transposon Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on developing transformational therapies for the treatment of neurodegenerative and aging-related diseases, including PSP, ALS, and Alzheimer's disease. The company's lead clinical compound, TPN-101, is first-in-class to address LINE-1 reverse transcriptase for the treatment of 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 the overproduction of LINE-1 cDNA, triggering innate immune responses that contribute to the pathology of neurodegenerative, neuroinflammatory, and aging-related diseases.

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