Alzheon Announces Phase 1 Results of ALZ-801, a First-in-Class Inhibitor of Amyloid Formation and Neurotoxicity for Alzheimer's Disease

Phase 1 data demonstrated superior properties for ALZ-801 as an optimized prodrug of tramiprosate

Clinical program for ALZ-801 supported by expanded post-hoc Phase 3 analyses with tramiprosate that showed cognition and function improvements in ApoE4-positive Alzheimer's disease patients

Data presented at the 12th International Conference on Alzheimer's and Parkinson's Diseases

LEXINGTON, Mass., March 20, 2015 – Alzheon, Inc., a clinical-stage biopharmaceutical company focused on brain health, memory and aging, today announced that the company presented Phase 1 clinical data for ALZ-801, a first-in-class small molecule inhibitor of amyloid formation and neurotoxicity for the treatment of Alzheimer's disease. As additional support to the clinical program for ALZ-801, Alzheon presented expanded post-hoc analyses of Phase 3 data with tramiprosate, the parent molecule of the optimized prodrug ALZ-801, which demonstrated sustained efficacy on cognition and function in a population of patients with Alzheimer's disease that are positive for the ε4 gene variant of apolipoprotein E (ApoE4), associated with increased risk of Alzheimer's disease. The Phase 1 results and Phase 3 post-hoc clinical analyses were presented at a poster session today at the 12th International Conference on Alzheimer's and Parkinson's Diseases in Nice, France.

"These findings on ALZ-801 and its parent molecule, tramiprosate, advance promising observations in the field of Alzheimer's disease," said Jeffrey Cummings, MD, ScD, Director of Cleveland Clinic Lou Ruvo Center for Brain Health and the Camille and Larry Ruvo Chair for Brain Health, co-author of the ALZ-801 poster and member of the Alzheon Scientific Advisory Board. "First, they build valuable new drug development insights based on the observed clinical effect with tramiprosate in ApoE4 population and second, they leverage the evidence that ApoE4 is an important risk factor for Alzheimer's disease."

In the Phase 1 single ascending dose study, ALZ-801 demonstrated superior therapeutic attributes over the parent molecule, tramiprosate, and in particular, reduced the inter-subject pharmacokinetic variability that hindered the efficacy signal of tramiprosate in the previous Phase 3 studies. The Phase 1 study included 67 healthy elderly subjects, and ALZ-801 showed an extended half-life of 14.9 hours, demonstrating extended bioavailability to allow efficient once-daily dosing. Further, ALZ-801 showed improved gastrointestinal tolerability as measured by reduced incidence of nausea and vomiting, as well as the decreased inter-subject pharmacokinetic variability, compared to the parent molecule, tramiprosate.

In addition, Alzheon conducted expanded post-hoc analyses and presented results today from the North American Phase 3 trial of tramiprosate, the parent molecule of ALZ-801, which analyzed 599 Alzheimer's disease patients from the Phase 3 trial with heterozygous or homozygous ApoE4 genotype. The analyses showed that homozygous ApoE4-positive Alzheimer's disease patients who received twice-daily administration of 150 mg tramiprosate demonstrated a greater than 4-point improvement in ADAS-cog (Alzheimer's Disease Assessment Scale-cognitive subscale) at 78 weeks, compared to the placebo group. In all-comers ApoE4-positive patients, which included both homozygous and heterozygous ApoE4-positive patients, tramiprosate showed sustained improvement in ADAS-cog of up to 2 points over 78 weeks.

"These data leverage the extensive safety and clinical efficacy dataset with an established drug molecule, tramiprosate, and extend it to bring new clinical advancements for the prodrug ALZ-801 with the potential to offer a novel medicine for Alzheimer's disease where new treatments are desperately needed," said Miia Kivipelto, MD, PhD, Director of Research and Education at the Geriatric Clinic and Head of the Unit of Clinical Trials at Karolinska University Hospital in Stockholm, co-author of the ALZ-801 poster and member of the Alzheon Scientific Advisory Board.

Based on the positive results and findings from these clinical studies related to ALZ-801, Alzheon has accelerated the clinical development program of ALZ-801 to confirm the dose regimen for a planned pivotal Phase 2/3 study in a defined population of ApoE4-positive patients with Alzheimer's disease.

"With ALZ-801, Alzheon has an exciting new molecule with compelling clinical rationale as a late-stage drug candidate for Alzheimer's disease," said John Hey, PhD, Chief Scientific Officer of Alzheon. "We are highly encouraged by the Phase 1 data which met our criteria for demonstrating optimized drug properties of ALZ-801 over tramiprosate, as well as strong therapeutic profile. Additionally, our extensive analyses of the previous Phase 3 dataset of tramiprosate further clarify and focus our clinical plan as we advance ALZ-801 toward a Phase 2/3 pivotal study later this year."

About Alzheon

Alzheon, Inc., a clinical-stage biotechnology company focused on brain health, memory and aging, develops innovative treatments for Alzheimer's disease and other neurological and psychiatric disorders. The company pursues product candidates for which there is evidence of both target engagement and clinical safety and efficacy, and where new insights and development strategies can be applied. Alzheon's lead product candidate, ALZ-801, is a small molecule inhibitor of neurotoxicity and amyloid aggregation with potential to be a first-in-class therapy for a genetically defined population of patients with or at high risk for Alzheimer's disease. For more information, please visit www.alzheon.com.

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