Alzheon Presents Positive Results of ALZ-801 Clinical Studies that Lead to Advancement to Pivotal Phase 3 Study in Alzheimer's Patients with APOE4/4 Homozygous Genotype

Pharmacokinetic and Safety Data from Phase 1b Studies with ALZ-801, an Optimized Prodrug of Tramiprosate, Presented at the Alzheimer's Association International Conference

FRAMINGHAM, Mass., July 25, 2016 – Alzheon Inc., today announced the results from two Phase 1b studies of ALZ-801, which provide the final clinical data necessary to progress ALZ-801 into the pivotal Phase 3 program in Alzheimer's disease (AD) patients who are homozygous for the ε4 allele of the apolipoprotein E gene (APOE4/4). ALZ-801 is an optimized prodrug of tramiprosate, the active agent in ALZ-801, and clinical development of ALZ-801 builds on the established safety and efficacy profile of tramiprosate from two prior Phase 3 trials in more than 2,000 AD patients.

The Phase 1b study results demonstrated favorable gastrointestinal tolerability, consistent and sustained plasma levels, and allowed selection of an ALZ-801 clinical dose that is bioequivalent to the tramiprosate dose that showed promising efficacy in prior Phase 3 trials. These data are being presented for the first time at the Alzheimer's Association International Conference (AAIC), held July 24-28 in Toronto, Canada. Alzheon is also making a late-breaking presentation with new analyses of the prior tramiprosate Phase 3 studies that include: 1) pooled safety analyses from 2,025 subjects across the two studies, 2) centralized assessment of brain MRI for occurrence of vasogenic edema or amyloid-related imaging abnormalities (ARIA), and 3) efficacy analyses in the apolipoprotein E4 (APOE4) subgroups, suggesting promising efficacy and favorable safety profile in Mild and Moderate AD patients with the APOE4/4 genotype.

"We now have all of the necessary clinical, toxicology and tablet data to start the pivotal Phase 3 program with ALZ-801, an oral, amyloid-targeting drug candidate for the treatment of patients with Mild to Moderate Alzheimer's disease," said Martin Tolar, MD, PhD, Founder, President and Chief Executive Officer of Alzheon. "Our body of ALZ-801 data and analyses suggest a well-differentiated and potentially transformative Alzheimer's medicine: an oral tablet that targets the underlying amyloid pathology in Alzheimer's, with compelling clinical efficacy in patients who are apolipoprotein E4 carriers. For the first approval for ALZ-801, we are preparing to initiate the ALZ-801 Phase 3 registration program in early 2017, focusing on the APOE4/4 homozygous patients that showed the most robust efficacy and represent a genetically-defined population with high unmet medical need."

Alzheon conducted the two Phase 1b clinical studies of ALZ-801 in healthy elderly volunteers: a single dose tablet bioequivalence study, and a multiple-ascending dose safety, tolerability and pharmacokinetic study. These studies showed that ALZ-801 demonstrates favorable pharmacokinetic (PK) properties, including steady target plasma levels with low inter-subject variability, as well as sustained plasma concentrations up to 24 hours. ALZ-801 showed an equivalent or improved PK profile compared to tramiprosate, including plasma exposures and dose proportionality, thereby allowing bioequivalence and bridging to the prior clinical data with tramiprosate. ALZ-801 also showed a favorable safety and tolerability profile, including improved gastrointestinal tolerability with lower incidence of nausea and vomiting compared to previous

studies with tramiprosate.

"We are extremely pleased with these clinical results for ALZ-801, which show that we have successfully optimized the pharmaceutical, pharmacokinetic and tolerability profile so that ALZ-801 is well positioned to advance into Phase 3 pivotal studies, and to potentially offer an important advance in AD treatment. In addition, we are excited to show that we have developed a simple, yet superior immediate-release tablet dose that can be used in the Phase 3 program and subsequently in broad commercial development," said John Hey, PhD, Chief Scientific Officer of Alzheon.

In a separate presentation, clinical data analyses from the prior Phase 3 trials of tramiprosate, the active compound in ALZ-801, are also being presented at AAIC. The APOE4 subgroup analyses of the tramiprosate Phase 3 studies showed clinically meaningful benefits on cognitive and functional endpoints in Mild and Moderate AD patients who are APOE4/4 homozygotes. The long-term safety profile was favorable in the Phase 3 studies, and was similar in the APOE4 carriers and non-carriers. AD patients who are APOE4/4 homozygotes have high prevalence and burden of cortical and vascular amyloid pathology, and are at high risk for occurrence of vasogenic edema with some amyloid targeted drug agents. There was no brain edema (ARIA-E) in the MRI subset of 426 patients in the Phase 3 studies treated with tramiprosate.

About Apolipoprotein E

Apolipoprotein E, or APOE, is a gene that provides a predictive window into an individual's Alzheimer's disease prognosis. APOE encodes for a protein called apolipoprotein E, which combines with fats to form lipoproteins that can be moved throughout the body. In the brain, apolipoprotein E helps shuttle cholesterol to neurons to support their normal function. There are three alleles, or forms, of the APOE gene, called ε2, ε3 and ε4. The ε4 allele has been found to correlate with high risk of developing Alzheimer's disease. People who inherit one copy of the ε4 allele, APOE4 heterozygotes, have an increased chance of developing the disease; those who inherit two copies of the allele, APOE4 homozygotes, are at even greater risk and tend to have more aggressive disease. The ε4 allele is significantly overrepresented in the Alzheimer's population compared to the general population: approximately 60 percent of Alzheimer's patients carry one or two copies of the ε4 allele compared to about 25 percent of the general population.

About Alzheon

Alzheon, Inc. is committed to developing innovative medicines for patients suffering from Alzheimer's disease and other neurological and psychiatric disorders. Our lead clinical candidate, ALZ-801, is a Phase 3-ready, first-in-class, small molecule oral inhibitor of amyloid aggregation and neurotoxicity – hallmarks of Alzheimer's disease. ALZ-801 is an optimized, novel prodrug that builds on the safety and efficacy profile of the active compound tramiprosate, which has been tested in clinical trials involving over 2,000 Alzheimer's patients. Our mission is to apply our clinical expertise and technology platform to develop novel therapeutics that make a difference in patients' lives by directly addressing the underlying pathology of devastating neurodegenerative disorders. For more information, please visit www.alzheon.com .

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