Alzheon Presents New Findings in APOE4/4 Homozygous Patients with Mild Alzheimer's Disease, Further Supporting Precision Medicine Approach and Planned Confirmatory Clinical Trial with ALZ-801

Presentation at the Alzheimer's Association International Conference Details Patient Responder Analyses on Key Clinical Outcomes

FRAMINGHAM, Mass., July 23, 2018 – Alzheon, Inc., a clinical-stage biopharmaceutical company focused on developing new medicines for patients suffering from Alzheimer's disease (AD) and other neurological and psychiatric disorders, today announced the upcoming presentation of data at the Alzheimer's Association International Conference (AAIC) in Chicago on Wednesday, July 25th, 2018.

The presentation at the AAIC highlights new responder analyses that focus on APOE4/4 homozygous patients with Mild AD at baseline, a genetically-defined population that has previously shown the largest clinical efficacy signals in the North American Phase 3 study of oral tramiprosate.[1,2] Tramiprosate is the active agent in ALZ-801, Alzheon's Phase 3-ready drug candidate that is being developed as a potential disease modifying treatment for Alzheimer's disease.

Large clinical benefit on both cognition and function have been observed in APOE4/4 AD patients with Mild disease in the tramiprosate North American study. In patients with Mild AD (MMSE 20 and above) treated with the high dose of tramiprosate, 150 mg twice daily, the responder analyses showed:

- Significantly more patients remained cognitively stable over 78 weeks of treatment, based on measures of Alzheimer's Disease Assessment Scale-Cognitive Subscale (ADAS-cog): drug 57% vs. 20% for placebo;
- Significantly more patients had minimal or no decline in function over 78 weeks of treatment, based on measures of Disability Assessment for Dementia (DAD): drug 46% vs. 18% for placebo.

"Responder rates in APOE4/4 patients with Mild AD support meaningful efficacy of tramiprosate in this population, and will help us design our planned confirmatory studies with ALZ-801," said Susan Abushakra, MD, Chief Medical Officer of Alzheon. "We continue to build on the body of clinical evidence to support the development of ALZ-801, and we are enthusiastic to initiate the pivotal program with ALZ-801 for Alzheimer's patients in need of effective treatment."

These clinical data support the Alzheon's precision medicine approach with ALZ-801. The analyses further expand on Alzheon's previous findings from the tramiprosate Phase 3 studies that showed the APOE4 gene dose effect, with the largest clinical benefit in AD patients with two APOE4 alleles, i.e. APOE4/4 homozygotes.[1,2] This clinical profile is consistent with the recently elucidated molecular mechanism of action, where tramiprosate inhibits beta amyloid (Aβ) monomer aggregation and formation of soluble toxic Aβ oligomers.[3] These data are also consistent with the positive clinical data from completed studies with anti-amyloid antibodies aducanumab and BAN-2401 targeting soluble toxic Aβ oligomers and proto-fibrils.

"This latest data presentation at AAIC strengthens the confidence in our pioneering precision medicine approach for ALZ-801 in the genetically-defined population of APOE4/4 homozygous patients with Mild or Early Alzheimer's disease," said Martin Tolar, MD, PhD, Founder, President and CEO of Alzheon. "Targeting soluble amyloid aggregates is the only therapeutic approach to date that has shown a disease modifying effect in Alzheimer's patients. We believe that our oral medicine may provide a meaningful benefit to patients in need, and will continue our targeted clinical approach to advance ALZ-801 as a breakthrough medicine for the patients who will most benefit from the treatment."

About ALZ-801

ALZ-801 is a novel, oral anti-amyloid drug candidate that is an optimized prodrug of tramiprosate, which has shown promising results in analyses of clinical data and therapeutic mechanism of action. ALZ-801 received Fast Track designation by the U.S. Food and Drug Administration (FDA) in October 2017. The clinical data for ALZ-801[4] and its active agent, tramiprosate, suggest long-term clinical efficacy in AD patients with the APOE4 genotype, along with a favorable safety profile.[1,2] ALZ-801 acts through a novel molecular mechanism of action blocking the formation of toxic amyloid oligomers[3] associated with the development and progression of AD. The initial Phase 3 program for ALZ-801 will focus on patients with the homozygous APOE4/4 genotype at the Mild stage of AD, with the potential for future expansion to additional Alzheimer's populations.

About Apolipoprotein

Apolipoprotein E, or APOE, is a gene that provides a predictive window into an individual's Alzheimer's disease prognosis. In the brain, apolipoprotein E helps shuttle cholesterol to neurons to support their normal function. There are three forms, or alleles, of the APOE gene, called ε2, ε3 and ε4. The ε4 allele has been found to correlate with high risk and earlier onset of Alzheimer's disease. It is estimated that up to 65% of all AD patients in the U.S. are carriers of at least one APOE4 allele, and that 10-15% of the AD population, or approximately 560,000 individuals in the U.S., are APOE4/4 homozygotes. APOE4 carriers – and more considerably APOE4/4 homozygotes – show faster rates of cognitive decline, at pre-symptomatic, early and dementia stages of the disease. In addition, APOE4 carriers, in comparison to non-carriers, show a higher and faster accumulation of amyloid pathology, including soluble amyloid oligomers.

About Alzheon

Alzheon, Inc. is committed to developing innovative medicines by directly addressing the underlying pathology of devastating neurodegenerative disorders. Our lead Alzheimer's clinical candidate, ALZ-801, is a Phase 3-ready, first-in-class, small molecule oral inhibitor of beta amyloid aggregation and neurotoxicity–hallmarks of Alzheimer's disease. ALZ-801 is a novel prodrug that builds on the safety and efficacy profile of the active compound tramiprosate, which has been evaluated in clinical trials involving over 2,000 Alzheimer's patients. Our clinical expertise and technology platform are focused on developing drug candidates using a Precision Medicine approach based on individual genetic and biological information to advance therapies with the greatest impact for patients.

Alzheon Publications:

[1] Abushakra et al. *Journal of Prevention of Alzheimer's Disease*, 2016
[2] Abushakra et al. *Journal of Prevention of Alzheimer's Disease*, 2017
[3] Kocis et al. *CNS Drugs*, 2017
[4] Hey et al. Clinical Pharmacokinetics, 2018

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