Alzheon Advances Industry-Leading Portfolio of Oral Anti-Amyloid Aggregation Inhibitors with First Subject Dosed in Phase 1 of ALZ-507, Highlighting Potential for Once-Daily Administration, Improved Safety and Efficacy, and APOE4 Corrector Mechanism

ALZ-507 Designed as Well-Differentiated Oral Candidate Targeting Soluble Amyloid Oligomer Pathology, with Additional Mechanism of Action as APOE4 Corrector

ALZ-507 IND Program Demonstrated Favorable Nonclinical Safety and Pharmacokinetic Profile that Supports Once-Daily Dosing

ALZ-507 Broadens Alzheon's Proprietary Precision Medicine Therapeutic Pipeline Targeting Disease Modification in Alzheimer's Disease

FRAMINGHAM, Mass., April 7, 2026 — Alzheon, Inc., a clinical-stage biopharmaceutical company developing a broad portfolio of investigational therapies and diagnostic assays for patients with Alzheimer's disease (AD) and other neurodegenerative disorders, today announced the dosing of the first cohort of human volunteer subjects in a Phase 1 single and multiple ascending dose clinical trial of ALZ-507, a novel investigational oral drug candidate for the treatment of Alzheimer's disease.

Martin Tolar, MD, PhD, Founder, President, and CEO of Alzheon, commented: "Dosing the first subject with ALZ-507 marks years of scientific progress at Alzheon as we deepen our understanding of Alzheimer's disease biology. This well-differentiated next-generation Alzheimer's drug candidate also functions as an APOE4 corrector, strengthening its anti-oligomer properties. ALZ-507 is a major step forward for Alzheon's precision medicine platform aimed at the prevention of formation of neurotoxic soluble amyloid oligomers. With Alzheimer's disease still representing a critical global unmet medical need, we are committed to work toward bringing to patients novel, safe, and accessible therapies that have the potential to slow disease progression."

ALZ-507 is an orally administered small molecule developed to target upstream amyloid aggregation and inhibit the formation of neurotoxic soluble amyloid oligomers, which are recognized as a key trigger and driver of Alzheimer's disease pathology. Additionally, ALZ-507

features an APOE4 corrector mechanism of action, potentially augmenting its primary anti-oligomer pharmacological effects. Designed as a next-generation oral treatment for Alzheimer's disease, ALZ-507 may offer the advantages of once-daily dosing and improved gastrointestinal tolerability. The Phase 1 study, employing an oral capsule formulation, will assess the safety, tolerability, and pharmacokinetics in healthy volunteer participants.

"The initiation of the ALZ-507 Phase 1 study with our novel oral product candidate demonstrates the capability of our development platform and our methodical approach to progressing high-quality, disease-modifying oral therapies with optimized pharmaceutical properties," stated John Hey, PhD, Chief Scientific Officer at Alzheon. "Building on our experience with upstream inhibition of amyloid aggregation, ALZ-507 further expands our portfolio of oral treatments designed to target the main causes of neurodegeneration. Preclinical studies have shown evidence of a positive safety and pharmacokinetic profile, as well as an enhanced efficacy profile, making ALZ-507 a valuable addition to Alzheon's portfolio of potential disease-modifying treatments for Alzheimer's disease."

The Phase 1 clinical trial will assess the safety, tolerability, and pharmacokinetics of both single and multiple ascending doses of ALZ-507. Findings from this investigation are anticipated to guide dose selection and inform formulation strategy for subsequent Phase 2 studies involving patients diagnosed with Alzheimer's disease (AD), Down syndrome–associated AD, and cerebral amyloid angiopathy (CAA).

About ALZ-801

Valiltramiprosate/ALZ-801 is an investigational oral agent currently in Phase 3 development as a potential first-in-class, disease-modifying treatment for Alzheimer's disease.[3-7,9,12] Valiltramiprosate is designed to inhibit the formation of neurotoxic soluble beta amyloid oligomers that contribute to cognitive decline in individuals with AD.[4-8,10,15] Preclinical mechanism-of-action studies have demonstrated that ALZ-801 can completely block the formation of these neurotoxic oligomers at the dosage used in Phase 3 clinical trials.[3,9,12,14] Valiltramiprosate employs an enveloping molecular mechanism of action intended to prevent the aggregation of soluble amyloid oligomers in the human brain, [14] which are associated with the onset and progression of cognitive impairment in AD patients.[3,4,7,9,10] In recognition of its therapeutic promise, valiltramiprosate received Fast Track designation from the U.S. Food and Drug Administration in 2017 for the treatment of Alzheimer's disease.

Clinical trial data indicate that valiltramiprosate exhibits strong clinical efficacy at the MCI stage, and a favorable safety profile, with no observed increase in the risk of brain vasogenic edema.[1-10,13,15] The initial Phase 3 program for valiltramiprosate targets Early AD patients who are homozygous for the apolipoprotein ε4 allele (APOE4/4), with plans to expand future research to include AD treatment and prevention in individuals carrying one copy of the APOE4 gene.[3-10]

Valiltramiprosate APOLLOE4 Phase 3 Trial

An Efficacy and Safety Study of Valiltramiprosate in APOE4/4 Early Alzheimer's Disease Subjects (NCT04770220): This trial was designed to evaluate the efficacy, safety, biomarker and imaging

effects of 265 mg twice daily oral dose of valiltramiprosate in Early AD subjects with two copies of the apolipoprotein ε4 allele (APOE4/4 homozygotes), who constitute approximately 15% of Alzheimer's patients. This double-blind, randomized trial compared oral valiltramiprosate to placebo treatment over 78 weeks. The APOLLOE4 trial was supported by a grant from the National Institute on Aging to Alzheon, with Susan Abushakra as the principal investigator.

Valiltramiprosate APOLLOE4 Long Term Extension Trial (Phase 3 LTE)

A long-term extension of the trial, APOLLOE4-LTE, evaluated valiltramiprosate in subjects who complete the core APOLLOE4 study for an additional 104 weeks of treatment for a total of 182 weeks or 3.5 years over the core and LTE study. This LTE study ended in January 2026 (NCT06304883).

Valiltramiprosate Phase 2 Biomarker Trial

Biomarker Effects of Valiltramiprosate in APOE4 Carriers with Early Alzheimer's Disease (NCT04693520): This trial was designed to evaluate the effects of 265 mg twice daily oral dose of valiltramiprosate on biomarkers of AD pathology in subjects with Early AD, who have either the APOE4/4 or APOE3/4 genotype and constitute 65-70% of Alzheimer's patients. The primary outcome was the change from baseline in plasma p-tau$_{181}$. The trial also included evaluation of clinical efficacy, safety, tolerability, and pharmacokinetic profile of valiltramiprosate over 104 weeks of treatment. A completed long-term extension of the trial evaluated the same dose of valiltramiprosate for an additional 104 weeks of treatment for a total of 208 weeks.[3,7,8]

About Alzheon

Alzheon, Inc. is a clinical-stage biopharmaceutical company dedicated to advancing a diverse portfolio of product candidates and diagnostic assays for individuals affected by Alzheimer's disease and other neurodegenerative disorders. The company is focused on innovating therapeutic solutions that directly target the underlying pathology of neurodegeneration. Its lead Alzheimer's clinical candidate, valiltramiprosate/ALZ-801, is a first-in-class oral agent currently in Phase 3 clinical development as a potentially disease-modifying treatment for Alzheimer's disease. Valiltramiprosate is an orally administered small molecule shown in preclinical studies to completely inhibit the formation of neurotoxic soluble amyloid oligomers. Its well-differentiated follow-on candidate, ALZ-507, is a once daily oral therapy designed to inhibit the formation of amyloid oligomers while also correcting the high risk APOE4 gene. Leveraging clinical expertise and a robust technology platform, Alzheon pursues drug discovery and development using a precision medicine approach that incorporates individual genetic and biomarker profiles, aiming to advance therapies with meaningful benefits for patients.

Alzheon Scientific Publications

[1]Abushakra S, et al: *Hippocampal Atrophy on Magnetic Resonance Imaging as a Surrogate Marker for Clinical Benefit and Neurodegeneration in Early Symptomatic Alzheimer's Disease: Synthesis of Evidence from Observational and Interventional Trials,* **CNS Drugs** 2026; 40, 199-214.

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[3]Pearson D, et al: *Polymorph Analysis of ALZ-801 (Valiltramiprosate), a Valine-Conjugated Oral Prodrug of Tramiprosate in Late-Stage Clinical Development for Alzheimer's Disease,* **Journal of Chemical Crystallography** 2025; 55, 206-215.
[4]Hey JA, et al: *Clinical Pharmacokinetics of Oral ALZ-801/Valiltramiprosate in a Two-Year Phase 2 Trial of APOE4 Carriers with Early Alzheimer's Disease,* **Clinical Pharmacokinetics** 2025; 64, 407-424.
[5]Aye S, et al: *Point of View: Challenges in Implementation of New Immunotherapies for Alzheimer's Disease,* **The Journal of Prevention of Alzheimer's Disease** 2025;12(1):100022.
[6]Abushakra S, et al: *APOLLOE4 Phase 3 Study of Oral ALZ-801/Valiltramiprosate in APOE ε4/ε4 Homozygotes with Early Alzheimer's Disease: Trial Design and Baseline Characteristics,* **Alzheimer's & Dementia** 2024; 10(3): e12498.
[7]Tolar M, et al: *The Single Toxin Origin of Alzheimer's Disease and Other Neurodegenerative Disorders Enables Targeted Approach to Treatment and Prevention,* **International Journal of Molecular Sciences** 2024; 25(5), 2727.
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[10]Tolar M, et al: *Neurotoxic Soluble Amyloid Oligomers Drive Alzheimer's Pathogenesis and Represent a Clinically Validated Target for Slowing Disease Progression*, **International Journal of Molecular Sciences** 2021; 22(12), 6355.
[11]Abushakra S, et al: *APOE ε4/ε4 Homozygotes with Early Alzheimer's Disease Show Accelerated Hippocampal Atrophy and Cortical Thinning that Correlates with Cognitive Decline*, **Alzheimer's & Dementia** 2020; 6(1): e12117.
[12]Tolar M, et al: *Aducanumab, Gantenerumab, BAN2401, and ALZ-801—the First Wave of Amyloid-Targeting Drugs for Alzheimer's Disease with Potential for Near Term Approval,* ***Alzheimer's Research & Therapy*** 2020; 12(1): 95.
[13]Tolar M, et al: *The Path Forward in Alzheimer's Disease Therapeutics: Reevaluating the Amyloid Cascade Hypothesis*, **Alzheimer's & Dementia** 2020; 16(11):1553-1560.
[14]Hey JA, et al: *Discovery and Identification of an Endogenous Metabolite of Tramiprosate and Its Prodrug ALZ-801 that Inhibits Beta Amyloid Oligomer Formation in the Human Brain*, **CNS Drugs** 2018; 32(9): 849-861.
[15]Hey JA, et al: *Clinical Pharmacokinetics and Safety of ALZ-801, a Novel Prodrug of Tramiprosate in Development for the Treatment of Alzheimer's Disease*, **Clinical Pharmacokinetics** 2018; 57(3): 315-333.
[16]Abushakra S, et al: *Clinical Effects of Tramiprosate in APOE4/4 Homozygous Patients with Mild Alzheimer's Disease Suggest Disease Modification Potential*, **Journal of Prevention of Alzheimer's Disease** 2017; 4(3): 149-156.
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