# Alzheon Raises $100 Million Series E Financing Round to Advance Development and Commercialization of Oral Tablet ALZ-801/Valiltramiprosate for Treatment of Alzheimer's Disease

*Proceeds Will Support Completion of Pivotal APOLLOE4 Phase 3 Study Evaluating ALZ-801/Valiltramiprosate and Regulatory Filings for Patients with Early Alzheimer's Disease in 2024*

*ALZ-801 Tablet Inhibits Formation of Soluble Toxic Amyloid Aggregates and Acts Upstream from All Late-Stage Amyloid Targeting Treatments*

*Supports Product Manufacturing and Preparations for Commercial Launch of Oral ALZ-801 as Potentially First Oral Disease Modifying Therapy in Alzheimer's*

FRAMINGHAM, Mass., June 12, 2024 — Alzheon, Inc., a clinical-stage biopharmaceutical company developing a broad portfolio of product candidates and diagnostic assays for patients suffering from Alzheimer's disease (AD) and other neurodegenerative disorders, today announced that it has raised $100 million in Series E financing, led by Alerce Medical Technology Partners. Alzheon previously raised $50 million through a Series D financing round in 2022.

"Alzheon has experienced tremendous progress in the past year and the promise of our novel oral Alzheimer's treatment, ALZ-801, has attracted prominent institutional and private investors. Our ability to raise $150 million over the last two financing rounds in the current climate speaks volumes about the prospects of our innovative science and technology," said Martin Tolar, MD, PhD, Founder, President, and CEO of Alzheon. "We are at the dawn of a new era in the treatment of Alzheimer's disease, and our novel therapeutic approach has an opportunity to transform the standard of care and improve access to treatment for all Alzheimer's patients. Our well-differentiated drug candidate with a favorable safety profile, showing no increased risk of vasogenic brain edema in more than 3,000 AD patients, is positioned to potentially become the first oral disease modifying therapy for the treatment of Alzheimer's disease."

ALZ-801 (valiltramiprosate) is a first-in-class, investigational oral disease-modifying therapy in Phase 3 development for the treatment of early Alzheimer's disease. In mechanism-of-action

studies, ALZ-801 fully blocked the formation of neurotoxic soluble beta amyloid oligomers at the Phase 3 clinical dose. ALZ-801 has shown potential for robust clinical efficacy in the highest-risk AD population – patients with two copies of the apolipoprotein ε4 allele (APOE4/4 homozygotes), and favorable safety with no increased risk of brain vasogenic edema. This population is the focus of Alzheon's pivotal Phase 3 APOLLOE4 trial, which is fully enrolled and will be completed in 2024.

"At Alerce, we focus on investing in companies in the late stages of development that are developing lifesaving drugs or devices with the potential to benefit millions of patient lives," said Muneer Satter, Founder and Managing Partner of Alerce Medical Technology Partners. "Alzheon's ALZ-801 provides an innovative precision-medicine solution in an emerging Alzheimer's pipeline with a path to potential approval in 2025. We are incredibly excited to support the Alzheon team in their latest round of financing."

ALZ-801 is currently being evaluated in two clinical trials in Early AD subjects. A two-year, 84-patient, Phase 2 biomarker study comprising APOE4 carriers, including 31 APOE4/4 homozygotes was completed in the second half of 2023. This Phase 2 biomarker trial is currently completing a fourth-year extension following positive results from the core portion of the study. The pivotal APOLLOE4 Phase 3 study of APOE4/4 homozygotes, which screened over 6,000 patients and enrolled 325 subjects, will complete and read out in the third quarter of 2024. Alzheon is planning for NDA submission in 2024.

"This latest fundraising ensures that we will have sufficient capital to complete our pivotal Phase 3 program and prepare commercialization of oral ALZ-801/valiltramiprosate with runway well into 2026," said Ken Mace, Chief Financial Officer of Alzheon. "ALZ-801 has the potential to disrupt the Alzheimer's treatment paradigm by slowing the progression of this relentless and debilitating disease, and the results from our pivotal APOLLOE4 Phase 3 trial will set the stage for the potential NDA filing this year, followed by the U.S. commercial launch in 2025."

**About ALZ-801**

ALZ-801/valiltramiprosate is a potential first-in-class, investigational oral agent in Phase 3 development as a potentially disease modifying treatment for AD.[1,2,4,5] ALZ-801 is designed to block the formation of neurotoxic soluble beta amyloid oligomers implicated in cognitive decline in Alzheimer's patients.[1,2,4] In mechanism of action studies, ALZ-801 has fully inhibited the formation of neurotoxic soluble beta amyloid oligomers at the Phase 3 clinical dose.[6,7] ALZ-801 acts through a novel enveloping molecular mechanism of action to block formation of neurotoxic soluble amyloid oligomers in the human brain[8] associated with the onset and progression of cognitive decline in AD patients.[1,2,4] ALZ-801 received Fast Track designation from the U.S. Food and Drug Administration in 2017 for Alzheimer's disease. In clinical trials, ALZ-801 has shown potential for robust clinical efficacy and favorable safety results with no increased risk of brain vasogenic edema.[6–8,10] The initial Phase 3 program for ALZ-801 is focusing on Early AD patients with two copies of the apolipoprotein ε4 allele (APOE4/4 homozygotes), with potential future program expansion to AD treatment and prevention in patients carrying one copy of the APOE4 gene and noncarriers.[1–5]

**ALZ-801 Phase 2 Biomarker Trial**

Biomarker Effects of ALZ-801 in APOE4 Carriers With Early Alzheimer's Disease (NCT04693520): This ongoing trial was designed to evaluate the effects of 265 mg twice daily oral dose of ALZ-801 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 trial also included evaluation of clinical efficacy, safety, tolerability, and pharmacokinetic profile of ALZ-801 over 104 weeks of treatment. An ongoing long-term extension of the trial evaluates ALZ-801 for an additional 104 weeks of treatment for a total of 208 weeks.

**ALZ-801 APOLLOE4 Phase 3 Trial**

An Efficacy and Safety Study of ALZ-801 in APOE4/4 Early Alzheimer's Disease Subjects (NCT04770220): This ongoing trial is designed to evaluate the efficacy, safety, biomarker and imaging effects of 265 mg twice daily oral dose of ALZ-801 in Early AD subjects with two copies of the apolipoprotein ε4 allele (APOE4/4 homozygotes), who constitute approximately 15% of Alzheimer's patients. This is a double-blind, randomized trial comparing oral ALZ-801 to placebo treatment over 78 weeks. The APOLLOE4 trial is supported by a $51 million grant from the National Institute on Aging. An ongoing long-term extension of the trial evaluates ALZ-801 for an additional 52 weeks of treatment for a total of 130 weeks.

**About Alzheon**

Alzheon, Inc. is a clinical-stage biopharmaceutical company developing a broad portfolio of product candidates and diagnostic assays for patients suffering from Alzheimer's disease and other neurodegenerative disorders. We are committed to developing innovative medicines by directly addressing the underlying pathology of neurodegeneration. Our lead Alzheimer's clinical candidate, ALZ-801/valiltramiprosate, is a first-in-class oral agent in Phase 3 development as a potentially disease modifying treatment for AD. ALZ-801 is an oral small molecule that has been observed to fully block the formation of neurotoxic soluble amyloid oligomers in preclinical tests. Our clinical expertise and technology platform are focused on developing drug candidates and diagnostic assays using a precision medicine approach based on individual genetic and biomarker information to advance therapies with the greatest impact for patients.

**Alzheon Scientific Publications**


[1] 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, 2727.

[2] 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, 6355.

[3] 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: e12117.

[4] 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: 95.

[5] Tolar M, et al: *The Path Forward in Alzheimer's Disease Therapeutics: Reevaluating the Amyloid Cascade Hypothesis*, **Alzheimer's & Dementia**, 2019; 1-8.
[6] 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.
[7] 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.
[8] 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.
[9] Kocis P, et al: *Elucidating the Aβ42 Anti-Aggregation Mechanism of Action of Tramiprosate in Alzheimer's Disease: Integrating Molecular Analytical Methods, Pharmacokinetic and Clinical Data*, **CNS Drugs**, 2017; 31(6): 495-509.
[10] Abushakra S, et al: *Clinical Benefits of Tramiprosate in Alzheimer's Disease Are Associated with Higher Number of APOE4 Alleles: The "APOE4 Gene-Dose Effect,"* **Journal of Prevention of Alzheimer's Disease,** 2016; 3(4): 219-228.

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