

## **Alzheon to Make Presentations on ALZ-801 and its Anti-Oligomer Mechanism at the 10th Clinical Trials on Alzheimer's Disease Congress**

### *Clinical Data and Research Support Advancement of ALZ-801 to Phase 3 Study as Oral Precision Medicine to Treat Alzheimer's Disease*

FRAMINGHAM, Mass., October 12, 2017 – [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 that the company will be making three presentations at the 10th Annual Clinical Trials on Alzheimer's Disease (CTAD) congress to be held on November 1-4, 2017 in Boston.

These presentations highlight Alzheon's lead drug candidate, [ALZ-801](#), an orally-administered, anti-amyloid inhibitor that blocks the formation of toxic amyloid oligomers associated with the development and progression of AD. The results presented at CTAD further support the pivotal Phase 3 development of ALZ-801 as a [Precision Medicine approach](#) to treat Alzheimer's patients with apolipoprotein  $\epsilon$ 4 (APOE4) genotype, with future expansion to additional Alzheimer's populations.

- An oral presentation at CTAD will discuss the central role of toxic amyloid oligomers in AD, highlighting Alzheon's discovery of the novel mechanism of action of tramiprosate, the active agent in optimized prodrug ALZ-801 that blocks formation of toxic beta amyloid (A $\beta$ ) oligomers. Recent findings also clinically translate the molecular mechanism of action of tramiprosate to clinical effects in Alzheimer's disease patients.<sup>1,2,3</sup>
- The company's Precision Medicine clinical program for ALZ-801 is further supported by clinical data in a poster presentation at CTAD that analyzed efficacy and safety in AD patients with two APOE4 alleles (APOE4/4 homozygotes), who after completion of the North American Phase 3 trial continued into an Extension Study. Those patients who received tramiprosate showed persistent efficacy, compared to subjects who had a delayed start on active drug, i.e., initial placebo-treated subjects. These clinical benefits in APOE4/4 AD patients are consistent with the key role A $\beta$  oligomers play in AD pathogenesis, and the recently discovered molecular mechanism of tramiprosate, where tramiprosate inhibits A $\beta$  monomer aggregation and formation of toxic A $\beta$  oligomers at clinically relevant doses.<sup>1</sup>
- In a poster presentation at CTAD, Alzheon will describe results from clinical studies of ALZ-801, an optimized prodrug of tramiprosate. The clinical studies demonstrated the improved tolerability and pharmacokinetics of ALZ-801 in comparison to oral tramiprosate, as well as establishing a dose that achieves equivalent target drug exposure. The completed ALZ-801 Phase 1 bridging program supports the pivotal Phase 3 clinical program for development of ALZ-801 as an oral, anti-oligomer inhibitor for targeting A $\beta$  pathology in the treatment of Alzheimer's disease.

The details for the presentations are as follows:

### **Oral Presentation (OC6)**

**Title:** *Amyloid Beta Oligomers in Alzheimer's Disease: A Missing Piece of the Alzheimer's Puzzle*

**Date:** Thursday, November 2, 2017

**Time:** 9:45 am ET

**Presenter:** Jeffrey Cummings, MD, Director, Cleveland Clinic Lou Ruvo Center for Brain Health in Las Vegas and Professor of Neurology at Cleveland Clinic

**Other Authors:** Sandrine Andrieu, MD, MPH, University of Toulouse, France; Philip Scheltens MD, PhD, VU University Medical Center, Amsterdam, Netherlands; Kaj Blennow, MD, PhD, The Sahlgrenska Academy at University of Gothenburg, Molndal, Sweden; Petr Kocis PhD, Alzheon, Inc.; John A. Hey PhD, Alzheon, Inc.; Aidan Power, MD, Alzheon, Inc.; Martin Tolar, MD, PhD, Alzheon, Inc.; Susan Abushakra, MD, Alzheon, Inc.

### **Poster Presentation (P29)**

**Title:** *Sustained Clinical Effects of Tramiprosate in APOE4/4 Homozygous Patients with Alzheimer's Disease Over 130 Weeks: Results of Phase 3 Extension Study*

**Poster Session:** Clinical Trials: Results

**Date:** Wednesday, November 1 and Thursday, November 2

**Presenter:** Susan Abushakra, MD, Chief Medical Officer, Alzheon, Inc.

**Other Authors:** Anton Porsteinsson, MD, University of Rochester; Carl Sadowsky, MD, Palm Beach Neurology, Florida; Bruno Vellas, MD, University of Toulouse, France; Serge Gauthier, MD, McGill University, Montreal, Canada; Aidan Power, MD, Alzheon, Inc.; Larry Shen, PhD, Pharmapace, Inc., San Diego; Peter Wang, PhD, Pharmapace, Inc., San Diego; John Hey, PhD, Alzheon, Inc.; Martin Tolar, MD, PhD, Alzheon, Inc.

### **Poster Presentation (P65)**

**Title:** *Beta Amyloid Anti-Oligomer Action of ALZ-801 and Clinical Dose Translation Analyses Support Confirmatory Phase 3 Program in Alzheimer's Disease*

**Poster Session:** Clinical Trials: Biomarkers

**Date:** Friday, November 3 and Saturday, November 4

**Presenter:** John A. Hey, PhD, Chief Scientific Officer, Alzheon, Inc.

**Other Authors:** Petr Kocis, PhD, Alzheon, Inc.; Susan Abushakra, MD, Alzheon, Inc.; Jeremy Yu, MD, PhD, Alzheon, Inc.; Aidan Power, MD, Alzheon, Inc.; Kaj Blennow, MD, University of Gothenburg, Molndal, Sweden; Martin Tolar, MD, PhD, Alzheon, Inc.

### **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 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 is 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.

<sup>1</sup> [Kocis et al. \*CNS Drugs\*, 2017](#)

<sup>2</sup> [Abushakra et al. \*J Prev Alz Dis\*, 2016](#)

<sup>3</sup> [Abushakra et al. \*J Prev Alz Dis\*, 2017](#)

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