



Pain Therapeutics, Inc.

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Pain Therapeutics Announces Successful Phase I Clinical Study for PTI-125

- PTI-125 Is Well-Tolerated in First-in-human Clinical Study -

AUSTIN, TX – October 24, 2017 – Pain Therapeutics, Inc. (Nasdaq: PTIE) announced today the completion of a Phase I clinical study for PTI-125, a new experimental drug therapy to treat Alzheimer’s Disease. This study investigated for the first time the safety, dosing and pharmacokinetic profile of PTI-125 in healthy human volunteers.

Summary of Key Findings

- PTI-125 was safe and well-tolerated at all doses studied
- PTI-125 demonstrated favorable pharmacokinetics for further drug development

"The clinical data are encouraging," said Remi Barbier, President & CEO of Pain Therapeutics. "Given the absence of dose-limiting effects in healthy adults, an excellent non-clinical safety database, a strong scientific rationale, and multiple peer-reviewed publications and research grant awards, we are eager to move this drug program to the next level of development."

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About the Phase I Study

PTI-125 was evaluated in 24 healthy human volunteers in a single-site in the U.S. for safety, tolerability and pharmacokinetics. Study subjects were administered a single oral dose of 50, 100 or 200 mg of PTI-125. The drug was well-tolerated in all subjects. Importantly, PTI-125 showed no treatment-related adverse effects and no dose-limiting safety findings. Pharmacokinetic measurements showed PTI-125, a small molecule, was rapidly absorbed. Dose-proportionality outcomes were observed over the entire dose range of 50 to 200 mg. Company scientists plan to present full results of this study at the 10th Annual International Conference on Clinical Trials on Alzheimer's Disease (CTAD), in Boston, MA, on November 1-4th.

Pain Therapeutics conducted this study with support from a \$1.7 million research grant award from the *National Institute on Aging*, part of the National Institutes of Health.

About Alzheimer's Disease and PTI-125

Alzheimer's disease (AD) is a progressive brain disorder that slowly destroys memory and thinking skills, and eventually the ability to carry out the simplest tasks. Damage to the brain starts a decade or more before problems appear. During this early stage of disease, people seem to be symptom-free, but toxic changes are taking place in the brain. Eventually, brain damage becomes widespread and affected people are often unable to care for themselves. Currently, Alzheimer's cannot be detected until symptoms appear. Over five million Americans live with Alzheimer's, a number that will increase significantly in the coming years.

PTI-125 is a small molecule drug candidate that was designed in-house and characterized by outside collaborators. PTI-125 has been shown to significantly improve AD neuropathologies in mouse models of the disease and in post-mortem brain tissue from AD patients, including receptor dysfunctions, neuroinflammation, tau hyperphosphorylation, insulin resistance and plaques and tangles that are hallmarks of AD.

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PTI-125 works by binding to filamin A (FLNA), a protein critical to beta amyloid's toxicity. Beta amyloid exerts multiple toxic effects, eventually causing the plaques and tangles found in the brains of people with Alzheimer's. By binding to FLNA, PTI-125 prevents and even reverses amyloid-related Alzheimer's damage. Building on this science, we also have under development a blood-based diagnostic for AD. Support for our diagnostic program is being funded by a \$1.8 million research grant award from the National Institutes of Health.

The underlying science for PTI-125 has been published in *Journal of Neuroscience*, *Neurobiology of Aging*, *Journal of Biological Chemistry*, *PLOS-One* and other peer-reviewed scientific journals.

Pain Therapeutics owns all worldwide development and commercial rights to PTI-125 and related technology, without royalty or other obligations to third-parties.

About Pain Therapeutics, Inc.

Pain Therapeutics, Inc. is a clinical-stage biopharmaceutical company that develops novel drugs. The FDA has not yet established the safety or efficacy of any of our drug candidates. For more information, please visit www.paintrials.com.

Note Regarding Forward-Looking Statements: *This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995 (the "Act"). Pain Therapeutics disclaims any intent or obligation to update these forward-looking statements, and claims the protection of the Safe Harbor for forward-looking statements contained in the Act. Examples of such statements include, but are not limited to, statements regarding the safety or effectiveness of PTI-125 and the Company's plan to develop PTI-125 for Alzheimer's Disease. Such statements are based on management's current expectations, but actual results may differ materially due to various factors. Such statements involve risks and uncertainties, including, but not limited to, those risks and uncertainties relating to the ability to demonstrate the safety, efficacy or potential health benefits of PTI-125. For further information regarding these and other risks related to our business, investors should consult our filings with the U.S. Securities and Exchange Commission.*

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