



## Pain Therapeutics, Inc.

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## **Pain Therapeutics Announces FDA Has Cleared an Investigational New Drug (IND) Application for PTI-125**

**– Clinical Study Initiation Is Expected Shortly, with Funding by NIH –**

**AUSTIN, Texas – July 31, 2017** – Pain Therapeutics, Inc. (Nasdaq: PTIE), a biopharmaceutical company, is pleased to announce that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for PTI-125, a novel drug candidate to treat Alzheimer’s Disease. As a result, clinical testing with PTI-125 will begin shortly, with funding provided by a \$1.7 million research grant from the National Institutes of Health (NIH).

PTI-125 will initially be tested in a Phase I ‘first-in-human’ study to observe measures of safety and pharmacokinetics and to inform dosing for future studies. The design of this Phase I study was based on FDA feedback, clinical and scientific rationale and observations from previously conducted preclinical and in vitro studies. Study results are expected by year-end 2017.

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The Company's Phase I study with PTI-125 will be funded by a \$1.7 million grant from the NIH.

The NIH's *National Institute on Aging* awarded this research grant to Pain Therapeutics following a competitive, peer-reviewed evaluation of PTI-125 on scientific merit and clinical promise by a panel of academic, clinical and industry experts in Alzheimer's Disease and other neurological disorders. 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.

### **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. There is no approved drug therapy to reverse, or even halt, the course of AD. PTI-125 is an oral, 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.

Pain Therapeutics owns worldwide commercial rights to PTI-125 and related technology.

### **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](http://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 initiate a clinical study with PTI-125. 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 our ability to predict the actual effects of PTI-125 on people before it is studied in humans; our ability to demonstrate the safety, efficacy or potential health benefits of PTI-125 in humans; unexpected adverse side effect; and our ability to determine which patient, or subpopulation of patients, may benefit from treatment. 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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